tools (See Section VI), there is significant demand from the patient, clinical, and public health communities for new, expanded data infrastructure and data access to support decision-making. Finally, investments in data infrastructure have the potential to generate significant additional investments in two ways. First, some of these investments could take the form of public-private partnerships. Second, data infrastructure is a tool that, once developed, will result in new research conducted and/or funded by entities such as biomedical research organizations, payers, foundations, and health care providers.

The Council received proposals on a number of potentially promising initiatives related to data infrastructure, including but not limited to:

- Building, expanding, and linking longitudinal administrative claims databases.
- Linking administrative data with EHR-based or registry data.
- Expanding high-impact patient registries, (e.g., collaborations with specialty organizations, SEER).
- Distributed data networks populated by EHRs in practice and provider settings.
- Expanding analysis of FDA and private sector data on drug and device trials and safety.

As the Office of the Secretary identifies specific opportunities in data infrastructure, the Council recommends that it consider most carefully those that:

- Expand access to existing resources, especially those currently managed by Federal agencies.
- Create scaled platforms by leveraging existing data and capabilities in the private sector.
- Capitalize on linkages between health IT investments and the potential for CER infrastructure to develop evidence to inform decision-making.
- Ensure that infrastructure is responsive to needs of patients, providers, and other decision-makers—and not driven by what is most feasible.

The Council appreciates the relationship and need for coordination between CER and health IT (e.g. through a distributed network of EHRs) investments. As the Secretary develops HHS's full portfolio of ARRA investments, it will be critical to consider both CER and health IT holistically, not as policy silos, recognizing that success in CER is largely dependent on success in health IT and vice versa.

With all data infrastructure investments, the government will need to ensure data security and privacy. Protecting security and privacy is key to maintaining the public's trust.

Secondary investments
Secondary investments include a core area of investment—Dissemination and Translation of CER—and two cross-cutting themes—Priority Populations and Types of Intervention.

Dissemination, translation and adoption of CER is about realizing the benefits that comparative effectiveness research has to offer both patients and providers. While the breadth and depth of the near-term impact depends on what types of pilot programs the OS supports, the lessons and tools for translation developed by those pilots will be relevant to all.

The lack of reliable success in disseminating findings from CER in ways that translate into better health outcomes highlights the uncertainty and difficulty of this enterprise. However, dissemination and translation is essential to improving outcomes for patients and the link between evidence production and how best to get this information to physicians and patients in a way they understand is critical to capitalizing on the CER investment. Despite important efforts by the Federal Government, especially AHRQ, NIH, VA and DoD, the majority of current funding goes to building evidence as opposed to ensuring that the existing evidence base is utilized in patient care and health systems management. This creates a unique role for OS ARRA funding. Investments in dissemination and translation programs also have the potential to generate additional investments, especially from providers, if private institutions elect to implement similar efforts or partner with the Federal Government on translation efforts.

There are a wide range of potential dissemination, translation and adoption programs that the OS could support, including:

- Investing in dissemination and translation of CER findings throughout the Federal delivery system.
- Dissemination and translation through partnerships with provider and/or patient organizations.
- Decision support and shared decision-making tools to provide information to clinicians and patients at the point of care.
- Developing standards for communication tools for patients and providers, (e.g., a patient-friendly simple scoring system).
- Partnering with an existing consumer media channel (e.g., Internet search engine or health information site) to expand patient access to existing CER data.
- Creating a National Patient Library with a primary focus on providing evidence to patients in easy-to-use and understandable formats.

The Council recommends that the Office of the Secretary consider the following in making investments in dissemination and translation:

- Investing in better understanding the most effective methods to disseminate and translate research findings to improve patient outcomes.
- Identifying opportunities both to develop tools for translation and to pilot implementation of these tools.
- Partnering with provider organizations in Federal agencies, as well as in states and the private sector.
- Accounting for potential surrogate decision-makers (e.g., families) and the context for decisions in patient-focused tools.
- Ensuring that programs address a specific need articulated by the implementing organization or the partner to ensure success and the sustainability of dissemination activities.
- Focusing on developing standards for communication.
- Increasing understanding of the most effective methods to disseminate findings to clinicians and patients to inform decision-making

From an operational perspective, investments in the cross-cutting themes are somewhat distinct from investments in the core areas. Whereas funding for a core area might go to a project or organization focused on a specific activity, funding for a cross-cutting theme requires multiple coordinated investments and activities to be successful. Investments in these themes could cover some or all of the four core activities: research, data infrastructure, human and scientific capital, and dissemination and translation. These investments could involve a coordinated investment across HHS or the Federal Government, or they could be focused in academic centers, integrated delivery system organizations, private industry, or other non-governmental entities. Collaborative efforts to inform and transform care will be essential to achieving meaningful impact across these cross-cutting themes.

Investments in specific populations, meanwhile, will help ensure that the benefits of CER are available to all. It can also focus CER efforts on populations with existing health disparities and worse outcomes. CER has the potential in some populations, such as racial and ethnic minorities, to fill critical gaps that, historically, efficacy research has left unaddressed.

The Council identified several populations for whom the Secretary should consider allocating CER funds:

- Racial and ethnic minorities
- Persons with disabilities
- Elderly
- Children
- Patients with multiple chronic conditions

Investment in specific types of interventions in a cross-cutting manner also presents a unique opportunity for the nation's health system. The Council has identified six specific interventions for the Secretary to consider that address large and varied populations, resulting in high potential impact, are areas of high clinical uncertainty, and are not being adequately addressed by other entities. They are:

- Medical and assistive devices (e.g., comparing rehabilitative devices).
- Procedures and surgery (e.g., evaluating surgical options or surgery versus medical management).
- Diagnostic Testing (e.g. comparing imaging modalities for evaluating certain types of cancer)
- Behavioral change (e.g., developing and assessing smoking cessation programs).
- Delivery system strategies (e.g., testing two different discharge process care models on readmission rates or testing two different medical home models on preventing hospital admissions and improving quality of life).
- Prevention (e.g., comparing two interventions to prevent or decrease obesity, comparing strategies for reaching populations that do not access the health care system with prevention efforts).

Furthermore, the Council recommends that the Office of the Secretary consider the following in making investments in the cross-cutting themes of priority populations and types of interventions:

- Focusing on immediate, specific patient needs that can generate results.
- Concentrating on areas with cross-cutting gaps in research, data infrastructure, scientific capital, and/or translation.
- Building on promising systems and practices already in place, both within the government and in the private sector, and measuring results when scaled up and disseminated.
- Strongly encouraging coordination across the government and with entities outside of the government.

Supporting investments

The Council recommends that the OS reserve some ARRA funding for Research, Human & Scientific Capital, and the Conditions cross-cutting theme. Because these investments and topics are the major foci of CER activities at NIH and AHRQ, both of which will likely utilize ARRA funds administered by those organizations for these purposes, they do not represent distinctive investment for OS funds. However, there will likely be targeted investments in these areas that could support other OS ARRA efforts, such as training new researchers in CER methods or addressing gaps not addressed elsewhere in the Federal Government.

In making these targeted investments, the Council recommends the Office of the Secretary consider:

- Focusing on areas that maximize the value of the Secretary's investments in other areas.
- Avoiding duplication of efforts with other agencies.

For all of the above investments, the Council recommends that the Office of the Secretary consider the portfolio of investments and where synergies exist to leverage one investment into multiple areas. For example, a data infrastructure investment that can also be used for a cross-cutting priority theme would be of higher value than an investment that has more limited applications. Doing so will help to ensure that the funds allocated to the Office of the Secretary for CER will have a significant positive impact on the quality of patient care in the near term, and lay the foundations for continued improvements going forward.

IX. LONGER-TERM OUTLOOK AND NEXT STEPS

Outlook

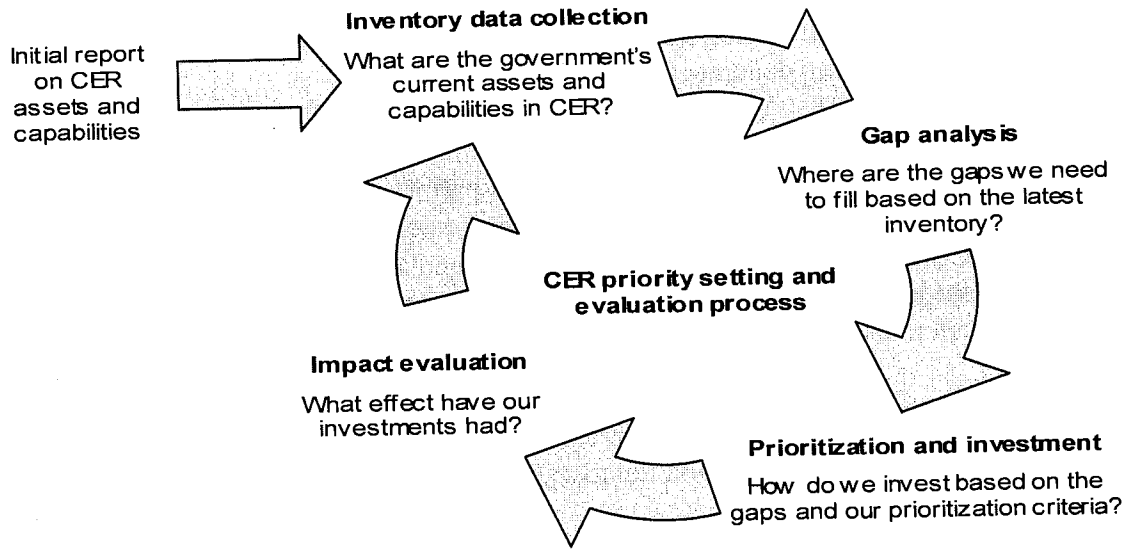
The CER investment strategy recommended in the previous section, if implemented successfully, has the potential to further a number of elements of the Council's vision for improved patient care. In the near term:

- CER dissemination and translation pilots in Federal health care delivery systems could help empower patients and their physicians to make better decisions now about their care and serve as models for expansion to private delivery systems. Moreover, a time limited investment can support establishment of a systematic strategy for translating the products of all relevant research to benefit patients served by Federal programs.
- An increased emphasis on CER for priority populations could ensure that all will benefit from comparative effectiveness research.
- Improved access for researchers to existing Federal data sources, and development and enhancement of distributed data networks and patient registries outside of the Federal Government, could jump-start a new wave of CER in the areas that matter most to patients.
- The inventory of Federal activities in CER will help reduce duplicative or uncoordinated investments among Federal agencies and help create transparency for patients.

It will be essential that a continuous cycle of CER priority-setting and evaluation of impact take place. The four critical steps in this cycle are inventory of CER and infrastructure; gap analysis; priority setting; and evaluation of impact. Figure 4 below depicts this process.

Figure 4

Continuous Evaluation of CER Inventory, Gaps, Priority Setting, and Impact



The strategic framework for comparative effectiveness research is intended to lay the groundwork for longer-term initiatives, such as innovative public-private partnerships to build data infrastructure and conduct CER. The goal of this investment is to generate some near-term results and momentum for the future. This strategy allows the government to facilitate the building of needed infrastructure, to expand access to existing infrastructure, and to demonstrate proof of concept for implementation efforts. These efforts are only a first step, however, to

achieving the vision of improved quality, safety, efficiency, equity, and patient satisfaction through improved medical decision-making and an optimized health care delivery system.

Indeed, a number of clear issues and challenges will remain for Federal CER efforts in the near term:

- **Listening and Engaging External Stakeholders.** While the Council has worked to create transparency and responsiveness in this process to date, it is critical that it continue to have a patient-centered focus going forward. In the future, the Federal Government may want to consider the options of listening and engaging stakeholders with representatives including, at minimum, patients, providers, payers, employers, and industry representatives, to guide CER or broader patient-centered outcome efforts.
- **Continued Coordination.** The Council laid the groundwork for coordination, but coordination will need to become embedded across the Federal Government. As the government makes investments in CER, there must be a mechanism in place to track and coordinate these investments and avoid duplication of efforts.
- **Building Scientific and Human Capital.** To maximize the potential benefit of investments in CER, the nation needs more researchers trained in the applicable research methods and further development of these methodologies. This presents both a short-term and a long-term challenge.
- **Maintaining Gains.** These investments represent only the beginning of CER efforts. New research findings will need to be disseminated and successful translation of evidence efforts should be expanded. New databases and data sets need to be maintained and kept current, and the catalog of Federal activities and data infrastructure in CER needs to become a living document.
- **Building Leverage.** The Federal Government is now a major funding source of CER, but the private sector still represents a majority of the investment in biomedical research. The government needs to find innovative ways of partnering with the private sector to leverage government investments and help private-sector investment better serve patients.
- **Keeping it Current.** There are no widely accepted and applied common standards or approaches for periodically re-evaluating CER to ensure that previous conclusions still hold. A system must be developed to ensure that the conclusions from CER remain valid over time.

These issues reflect both the fact that comparative effectiveness research remains in its infancy and that it must be seen as a continuous and iterative process that needs to constantly evolve based on the changing needs of the patient.

Next steps

There are a number of next steps leading to the Secretary's integrated strategy and spend plan for CER on July 30, 2009, and several requirements for the success in implementation of that strategy. Most importantly, it is critical to the success of CER and health care transformation that the plan is coordinated across the ARRA CER funding allocated to the Secretary, AHRQ, and NIH. Furthermore, the Secretary should develop the plan as part of HHS's broader portfolio of ARRA investments, not as a stand-alone program.

The following steps are needed to finalize the CER operational strategy prior to the July 30 deadline mandated by Congress:

- Integrate IOM and Council strategic recommendations and leverage the investments, resources, and capacity identified through the initial inventory effort.
- Provide more specific recommendations for a portfolio of initiatives for all of HHS' ARRA funds within the framework outlined in the report.
- Define metrics for evaluating success.
- Coordinate the submission of the CER ARRA spend plans to ensure that they cover the gaps in CER outlined in this report, and that the agencies' investments leverage the strengths of each agency and are complementary, not duplicative.
- Maintain transparency and engagement with the public.

The Federal Government will need to continue its work to coordinate CER investments and assure Americans that these resources are being invested wisely. ARRA required that the Council submit an annual report regarding its activities and recommendations concerning the infrastructure needs, organizational expenditures, and opportunities for better coordination of comparative effectiveness research by relevant Federal departments and agencies. The first annual report will likely be in June 2010.

Comparative effectiveness research is being considered as a key piece of health reform, and the Federal Government must demonstrate its capability to coordinate that investment, achieve impact, and measure the results. This report outlines the priorities and path forward. Now the Federal Government must make progress and deliver results for the American people.

APPENDICES

Appendix A. LISTENING SESSIONS AND PUBLIC COMMENT SUMMARY

Overview

In order to help guide the Council's thinking on the definition, framework, and priorities for comparative effectiveness research (CER), the Council held three listening sessions (April 14 in Washington, D.C.; May 13 in Chicago; and June 10 in Washington, D.C.) and solicited public comments through the hhs.gov/recovery Web site. The response was strong:

- 92 panelists testified
- Greater than 300 individuals and organizations submitted comments

A breakdown of the responses by stakeholder type reveals that over half of the comments came from three groups: health care associations; academia and think tanks; and consumer, community-based, and advocacy organizations. Additional comments were received from patients, providers, payers, hospital and health systems, pharmaceutical companies, foundations, public health entities, and private sector companies in the health care field. One U.S. Senator also submitted comments.

More important than the diversity of respondents is what individuals and organizations had to say. Respondents provided a wide range of opinions and offered recommendations on everything from stakeholder participation to how to prioritize investments in CER to specific areas of focus to knowledge transfer and dissemination.

Without question, the public input has been extremely valuable in informing the Council's deliberations; many of the major thematic threads that run through the public comments are reflected in the Council's strategic framework, focus, and recommendations for priorities for OS CER funds. Of particular value to the Council was the opportunity to engage with panelists at the listening sessions. This back-and-forth discussion enabled Council members to refine their ideas and solicit further feedback.

Before summarizing the key themes, it is useful to note that several respondents honed in directly on the reason why investments in comparative effectiveness research are important—CER matters. For example, one respondent talked about the value of and application of CER for everyone's health and health care. Another talked about how funding is crucial to reforming the practice of medicine to increase the quality, safety, cost benefits, and real world effectiveness of what providers bring to patients on a daily basis.

One theme that wove through many of the comments was the need for greater collaboration among Federal agencies, among organizations at the Federal, state and local levels, and between the public and private sectors. One respondent stated that because expertise on comparative effectiveness research resides in both public and private entities, every effort should be made to encourage public-private collaboration in the design, conduct, analysis, and reporting of CER. This discussion about collaboration dovetailed with the question of stakeholder input, including the need to ensure that patients have a defined and central role in the CER process.

Key Themes

Some respondents recommended targeted research topics; these ranged from testing the total effects of medication on the frail elderly, to testing the efficacy of a diet to treat children with seizures, to informing prescribing physicians on a wide range of what does and doesn't work well for patients with various complex conditions.

Most of the comments, however, fell into several broad categories—prioritizing the agenda, infrastructure development, research methodology and conduct, care delivery, knowledge transfer, cost, and health disparities and personalized medicine. A number of key themes and specific comments are summarized below.

Prioritizing the Agenda

A number of respondents tried to step back and look broadly at the question of how to prioritize the agenda for comparative effectiveness research and what criteria should guide decision-making in this arena. An overarching theme that echoed through many of these comments was the need to think big and look system-wide. One respondent stated that CER that is localized to a single disease may be less of a priority than questions that cross over diseases. Another talked about the need for CER to be undertaken for quality, efficiency, effectiveness, and other appropriate dimensions for health care delivery systems along the entire spectrum of systems integration, adding that the spectrum should include integrated delivery systems, multi-specialty group practices, single-specialty groups, "virtual" groups, and small medical practices.

A number of recommended areas of focus emerged. Many respondents talked about focusing on areas of major clinical significance and the greatest impact on health care delivery, including chronic conditions. One respondent specifically noted that CER on chronic diseases should focus on all relevant health care services, including medical and surgical procedures, diagnostics, and medical devices. Another respondent said that more attention is needed in the areas of post-acute and long-term care. Still others talked about the need for comparative effectiveness research on emergency care processes, and CER to evaluate regional differences in trauma care. A few people talked about studying the role of alternative treatments, including homeopathic treatments for chronic and acute disease states. Several respondents also talked about looking at conditions with the greatest impact on morbidity, and a few about doing research on conditions with the greatest impact on cost.

A few respondents discussed the need to ensure that the priorities of state and local jurisdictions be given consideration in evaluating various CER strategies. For example, one participant noted that many jurisdictions have on-going investigative agendas designed to improve program effectiveness that can be considerably amplified by Federal support, adding that such efforts would extend beyond purely clinical protocols to include the evaluation of public health, community-based, and behavioral strategies that may enhance the effectiveness of public programs.

One respondent suggested that significant resources be devoted to population-level interventions as well as patient-level effectiveness. Another respondent talked about the need for comparative effectiveness priority research areas to include critical cross-cutting research questions and cited

several examples (clinical decision-making, human-technology partnership, team coordination and continuity of care).

In addition, respondents talked about the need for Federal investments in CER to focus on health disparities and understudied sub-groups. Many of the respondents who addressed this topic talked about under-sampling of minorities in clinical trials and stressed the need for research that looks at the impact of various treatments on specific sub-groups, including women, minorities, people in rural communities, persons with disabilities, and children.

Infrastructure Development

A number of respondents honed in on the need to scale up the capacity to do comparative effectiveness research. As one respondent put it: "All healthcare reform proposals are predicated on the presumption that a robust and well-developed quality infrastructure exists. However, this is not uniformly the case."

Infrastructure capacity, as framed by the public comments, incorporates three components: human and scientific capital, organizational capacity, and data capacity.

Regarding human and scientific capital, respondents said that investments are greatly needed to enhance the skills, supply, and diversity of the research work force. One respondent pointed specifically to a dearth of researchers focused on mental health and substance abuse and treatment. Another respondent talked specifically about the need to increase the number of Hispanic health professional researchers, and suggested that HHS target Hispanic health professional, students, residents, and graduate students interested in serving in their communities.

Regarding organizational capacity, many of the comments focused on building capacity at the regional and local level. For example, one respondent talked about the role that health improvement collaboratives and chartered value exchanges can play in maintaining patient registries and other databases, and about using the information for performance reporting. A second respondent talked about the role that more community organizations could play in helping to address racial and ethnic health disparities were they to have the appropriate infrastructure and capacity.

The third critical subset of infrastructure development is data. A number of respondents talked about the need for both better data and access to data for comparative effectiveness research and decision-making. They urged the Council to access as much available data as possible, including clinical trials data, electronic health record systems, health care claims systems, administrative data, and Federal health data (including data from Medicare and Medicaid and that collected by the Veterans Health Administration). Respondents also talked about the need to invest in a coordinated effort to link public and private sector databases, as well as the need for standardized data available from the point of patient care.

Several respondents also talked about the value of registries, and the need to link data sets in order to provide valuable sources of data to examine appropriate use, effectiveness of care, cost of care, value-based health care, and other criteria. Another respondent stressed the need for

research that involves collaboration in different data environments and research that explores the use of different types of electronic health care data.

Research Methodology and Conduct

How should CER be undertaken? This is another theme that ran through many of the comments. Those who tackled this question addressed key issues that ranged from the enterprise level to guidance on study design. At the broader level, one respondent talked about the need for a broad Federal CER enterprise that spans treatment, prevention, promotion, and health-determinant interventions designed for both people and populations. Another respondent recommended adopting value of information principles and tools to prioritize CER investments on those studies where there is a greater likelihood that the research will lead to changes in practice. A third person spoke about the opportunity to fund research into "the science of CER" to build a foundation for this work.

Others talked about the scope of CER, noting that much of the research is conducted in single settings of care. One respondent, for example, noted that this poses a challenge for "generalizability," and suggested that many of the questions that remain unanswered relate to uncommon conditions or outcomes that have proven challenging to study. He recommended the use of multi-center research networks to address this issue.

Looking more closely at study design, one respondent noted that CER should continue to use a variety of study designs to generate evidence about the comparative effectiveness, comparative safety, and cost effectiveness of medical interventions. A second respondent talked about the limitations of randomized clinical trials, suggesting that the Council should also consider designs that are more common for evaluating comprehensive population-focused interventions, such as observational cross-sectional studies, quasi-experimental designs, and time series analyses. Another respondent stressed that clinical trial design and CER infrastructures must accommodate the goal of addressing health disparities. Another respondent pointed out that comparative effectiveness can at times be determined by assessing technology and using quantitative metrics rather than via an expensive and sometimes-lengthy clinical trial. A fourth respondent talked about the need to include utilization of laboratory services in order to effectively compare treatments and outcomes for major chronic disease cost drivers.

Several respondents also addressed the need for greater transparency throughout the process. They talked about the critical importance of transparency for reducing bias and rebuilding trust, and they recommended that researchers show results prior to adjustments as well as adjusted results. Respondents who tackled the issue of transparency also talked about the need to disclose in detail the methods and metrics used in any research. One respondent stressed that patients and providers need to know all the inputs that go into a research analysis so that they can weigh the costs, safety, and quality issues appropriately in each instance.

A corollary to transparency is addressing potential conflicts of interest. Respondents talked about the need to develop a strong and clear policy for conflicts of interest in both research and publishing, and suggested that funding decisions for CER should favor researchers and institutions that are focused on the public interest and do not have current conflicts. They also talked about the need for 100-percent disclosure and transparency at the outset of all conflicts by

individual researchers and institutions. One respondent specifically said that the ARRA expenditures on CER offer an opportunity to move to a platform where research funding is completely independent of other sources of funds in order to get to research that is independent, unbiased, untainted, and neither methodologically flawed nor influenced by industry.

Care Delivery

Several respondents pointed out that care delivery is critical, and that investments in CER are needed to look at how the health care delivery system should be organized and the best models for delivering care to patients. One respondent recommended that the Council invest in research that looks at optimal practice models for delivering patient care along with strategies for using information technology and clinical decision support tools to implement research findings into clinical practice. Another respondent suggested that CER is needed to look at the organization, design, and management of patient care. A third said that CER should focus on medical delivery systems and operations, resulting in information that can be leveraged to foster better clinical and cost outcomes.

Much of the discussion on care delivery was focused on people with one or more chronic conditions (e.g., diabetes). One respondent, for example, talked about the need for CER studies that compare current, more traditional models of chronic care delivery with team-based, patient-centers models that include patient education and self-care. Another respondent emphasized the need to focus research on the impact of non-medical services (e.g., providing housing) on cost-effective and clinical outcomes for chronically medically ill populations. A third person talked about CER around the role of support services (e.g., case management) in the health outcomes of people with HIV/AIDS; a fourth, about the need for CER on crisis residential services as an alternative to psychiatric hospitalization. Yet another respondent talked about the need to study the cost-effectiveness of community health worker interventions.

One respondent talked about the need to study care models that integrate primary and tertiary care. Another respondent suggested that there was a need for research into how to deliver care in a way that helps patients get the care they need, adhere to proposed treatment regimes, and prevent subsequent untoward effects of chronic diseases. Regarding adherence to treatment regimes, one respondent specifically noted that patient compliance is a seldom-accounted-for variable in CER, and he talked about the value of electronic verification devices to track compliance. Another respondent talked about the need to compare palliative care models to understand which processes of care and specific program interventions and models are the most effective.

One respondent noted that much of the literature on the impact of electronic medical records is anecdotal, and he expressed concern that people are considering the value of electronic health records without understanding the totality of what an effective system does for health care delivery. As a result, he urged that research be done to evaluate the comparative effectiveness of different types of EHR-mediated interventions. A second respondent likewise talked about the needs for research on how health information technology and EHR exchanges can be used to create more robust data sources and to help evaluate comparative effectiveness issues across a broader range of settings.

Knowledge Transfer

A number of respondents pointed out that all the data is meaningless if the information is not disseminated effectively. One respondent, for example, stressed that knowledge translation research must not be overlooked, while another respondent pointed out that both research and dissemination of research findings are essential to realizing the quality improvements and returns-on-investment that are integral to the success of comparative effectiveness research.

While respondents had different recommendations for how to approach knowledge transfer, there was a consensus that this work is critical. One respondent noted that the evidence base that is developed around clinical comparative effectiveness offers a substantial opportunity to improve value in health care if the information is disseminated and applied by physicians and patients. Others talked about the need to identify what approaches and incentives to dissemination and adoption are most effective (and under what circumstances), and when dissemination should target change at the organizational level, the community level, or the individual level. One respondent talked about cultural competence and health literacy research, and the need for both in order to change behaviors and improve lifestyles.

One respondent noted that while technology (including electronic health records) is one avenue for dissemination, other effective dissemination and translation techniques are also needed. She noted that while many strategies have been used to enhance the rate and extent of adoption of evidence-based best practices (including clinical guidelines, continuing education for health care professionals, patient education tools, and academic detailing), the approaches have not been well studied and the results are variable.

One respondent suggested that an independent body be established to disseminate comparative effectiveness research findings; others took the approach that everyone—including providers, payers, consumers, and employers—has a role to play in disseminating research results. Another respondent suggested creating a national citizens' advisory board to help HHS better understand the perspectives and values of the general public when designing and disseminating CER. Another respondent talked about the need not only to provide the evidence base for best disease prevention, health promotion, and/or clinical interventions, but also to look at how these findings can be implemented in "real-world, complex organizational settings."

Cost

Two distinct opinions about cost emerged: (1) that it should be a factor in comparative effectiveness research or (2) that it has no place in the discussion.

Those opposed to factoring cost into CER expressed concern that too often people put cost into a separate silo and make decisions without regard to efficacy, and they suggested that a focus on costs could lead to limiting access and benefits. For example, one respondent said that comparative effectiveness research should not be focused on looking for cheaper treatments, and it should not be the basis for coverage decisions. Another talked about the fear that CER results might impact physician reimbursement rates. Several respondents also expressed concern that CER could be used to restrict access to care, to deny coverage, or to reduce payments for interventions, thus undermining physician/patient decision-making and limiting patient access to treatment options.

On the flip side, other respondents felt equally strongly that cost was an integral component of informed decision-making. For example, one respondent said that information about costs enables understanding not only of the direct differences in terms of clinical outcomes but also of the value of interventions and whether they represent an efficient use of resources. Another respondent suggested that, if costs are not considered, the tradeoff in terms of lost health benefits would be too steep. Others stressed that a wide range of stakeholders—including employers, policymakers, and state and local public health departments—have said that they need cost information to make decisions.

Health Disparities and Personalized Medicine

Several respondents spoke about the related topics of the need to address health disparities within CER and support for the growth of personalized medicine. Inclusion of and attention to underrepresented sub-groups was spoken of as a means to address the problem of disparities in care. Others spoke of the importance of fostering the application of personalized medicine.

Respondents cited the need for more CER in the areas of preventive care, pediatric care and children's health, behavioral health interventions, addiction, mental disorders, and suicide prevention. One respondent pointed out that CER is needed to understand the cost and quality implications to the overall health system of continuing to under-treat conditions in systems that are siloed and distinct from mainstream health and health care. Another respondent specifically noted that the aim of personalized medicine and the mapping of the human genome is to achieve disease interventions much earlier (ideally at the point of preventing the disease from ever taking hold, he said).

One respondent stressed that CER must be mobilized to improve the health outcomes of various racial and ethnic minorities in order to close the gap that exists between the health status of some minority populations and other Americans. Others warned about relying on small, narrowly focused studies, suggesting that understanding and addressing health disparities requires a broader approach; conversely, respondents also cautioned against "one-size-fits-all" approaches that could decrease access to treatments. One respondent specifically talked about the need for research that examines health intervention outcomes across the lifespan, and for different minority and gender groups, in order to understand the effectiveness of interventions within and between population groups.

Several people talked about the need to design studies that appropriately include minority populations (see also *Prioritizing the Agenda*, above). For example, one respondent said that the design of studies must reflect the diversity of patient populations, including racial and ethnic diversity, and must communicate results in ways that reflect the differences in individual patient needs. Another respondent stressed that clinical trial design and CER infrastructures must accommodate the goal of addressing health disparities. There was also discussion more broadly about the need to build the infrastructure to address health disparities relating to people of color.

One respondent pointed to the dichotomy between studying populations and the promise of personalized medicine, asking: How can CER at a broad population level be balanced with the

goals and rapid scientific advancements in the area of personalized and stratified medicine in order to encourage the development of targeted therapies for sub-groups?

One respondent talking about personalized medicine recommended that CER studies include the evaluation of approaches to health care delivery and care management that foster the effective application of personalized medicine.

Appendix B: SUMMARY OF THE COUNCIL'S MEETINGS AND DELIBERATIONS

The following contains a summary of the Council's deliberations as they unfolded once the Council was officially convened.

April 10, 2009

The Council was presented with background information on comparative effectiveness research and briefed on CER activity at AHRQ, NIH, and VA. The Council also discussed the scope of their work and objectives.

Next, the Council began discussion of the components of the definition of CER and potential criteria for prioritization. The Council also discussed how CER and data infrastructure for CER might be categorized. Finally, the Council reviewed the timeline and discussed plan for listening sessions, including the first listening session on April 14, 2009.

April 22, 2009

The Council met to discuss what they had heard at the April 14 listening session. Members identified several key themes, including the need to outline a clear, well-delineated definition of comparative effectiveness research. They noted that participants had also talked about the need to prioritize methodology, and the fact that CER should be inclusive of all components of medical care.

Council members also noted that they had heard, loud and clear, that the Council's governance and processes must be transparent, and that the Council must incorporate input from all stakeholders to gain credibility and build trust.

Other themes that emerged from the listening session include the need to focus on patients and outcomes; the importance of incorporating diverse populations and multiple research methods; and the need for investments in infrastructure. Regarding the focus on patients and outcomes, Council members noted that participants had talked about the importance of considering patient input from the start and the fact that the results must be framed and disseminated in ways that are relevant to patients and providers. Regarding diverse populations, Council members observed that there was discussion about the need to include sub-groups with multiple chronic conditions, and the need more broadly to make CER relevant to sub-groups. Members also noted that participants had talked about the need to use a multitude of different research methodologies (not just randomized clinical trials), and to look at the Department of Veterans Affairs' experience using registries.

Regarding infrastructure, Council members observed that participants had stressed there was a need to expand, improve, and build on existing information and registries, and that perhaps this investment could lay the foundation for distributed data networks with the capability to answer many future CER questions. Members also noted that there had been discussion about the need to make data monitoring easier and more routine.

Finally, Council members talked about how they could tweak the listening session format to allow for a more robust conversation with participants.

May 1, 2009

The Council looked at the timetable for its work and the due dates for its key deliverable. The Report to Congress is due June 30, and the preliminary timetable builds in time for HHS and OMB clearance, comments, and suggested edits. The Council also briefly discussed the upcoming second listening session, slated for May 13 in Chicago.

Next, the Council briefly discussed the process for compiling the CER and data infrastructure inventories, and agreed that members would identify primary contacts in their division or agency who can work with the contractor to drive that process.

The Council's next goal was to arrive at consensus on a draft definition of comparative effectiveness research, prioritization criteria, and a categorization framework for CER. Once complete, the Council agreed to post the draft language on the hhs.gov/recovery Web site and to solicit public feedback.

To begin that work, the Council tackled the draft definition. There was considerable discussion about what the definition of CER should be. Members expressed the belief that the definition needed to be inclusive of the multiple stakeholders in the health care arena, including communities, and they also looked at what types of interventions should be called out. The Council ultimately came to consensus that they wanted a definition that was broad-based and inclusive, but that was not so detailed as to inadvertently narrow the scope of comparative effectiveness research.

The Council next turned its attention to the prioritization criteria. Before doing so, however, the Council first wrestled with the question of whether the criteria should be focused broadly or more narrowly targeted to provide guidance to the Office of the Secretary in allocating its Recovery Act funds. The Council generally felt that the criteria should be broad enough to allow the Council to make recommendations on overall funding and funding criteria.

Next, Council members discussed how to prioritize the CER criteria, including whether impact should be listed first, with feasibility and scientific merit second. One person spoke out about the need to keep the criterion on diverse populations and patient sub-groups within the top five. There was also discussion about whether knowledge gap was a criterion, or whether it should perhaps be wrapped into the criterion on impact.

The Council also looked at several potential frameworks for comparative effectiveness research, including categorization by type of CER investment, by patient sub-groups, by condition, and by type of intervention. The aim of developing a framework was to help categorize current CER activity and to identify gaps for potential future investments in CER. Council members also discussed CER centers, and agreed that Recovery Act funding could be used to support this work. One member suggested that the Council, at a future date, should discuss how to coordinate interest in CER centers across agencies.

Finally, the Council received a presentation on enhancing the inclusion of minority and other underserved populations in comparative effectiveness research. As a result, the Council agreed to establish a small workgroup co-led by NIH, AHRQ, the HHS Office of Minority Health, and the HHS Office on Disability. The workgroup will have two key tasks: (1) to develop recommendations for the inclusion of minority and other underrepresented populations in the expanded comparative effectiveness research agenda, and (2) to receive input from non-Federal groups on targeted actions.

May 8, 2009

The Council reviewed a revised definition of comparative effectiveness research and agreed to post the definition on the hhs.gov/recovery Web site on or about May 15.

Next, the Council resumed its discussion of the prioritization criteria. There was considerable discussion about whether "scientific rigor and validity" needed to be included in the threshold minimal criteria, with some members saying that it was implicit (and something already being done) and others expressing concerned about including a yes/no component to the threshold minimal criteria. The consensus of the Council was that scientific rigor and validity be included as part of a concept statement.

The Council then looked at a first draft outline of the Report to Congress. It included (1) Introduction, (2) Objectives, (3) Definition and Criteria, (4) Framework for CER, (5) Current CER and CER data infrastructure, (6) Recommendations for Priorities for OS CER Funds, and (7) Longer-term Vision and Opportunities.

Council members discussed a number of items that they believed needed to either be included or called out in the report, including concrete examples of what CER is and why it matters as well as a discussion about the full range of CER activities (and not just randomized clinical trials). There was also discussion about having a stand-alone section on high level priorities; the need to call out the roll of public/private partnerships; including a sub-section on the need for CER data to be synthesized and operationalized, along with some mechanisms for achieving this outcome; and the need to add language on sub-groups. Members also agreed to add a new section, Summary of the Listening Sessions, and to include a high-level Executive Summary.

Next, the Council began its discussion of CER priorities. To frame their discussion, members looked at four categories: primary research, dissemination of results, data infrastructure, and cross-cutting coordinated investments. One member asked, "What are the gaps that no one else can fill?" The Council agreed to continue its discussion at its next meeting.

May 22, 2009

The Council opened its meeting with a debrief from the May 13 listening session in Chicago. Members said they found the meeting both useful and exciting, and cited some themes they had heard that particularly struck them. These included the need to study chronic diseases (and to include sex, ethnicity, and race in the analysis); the idea of using theoretical models to assess how to approach a study (and to ensure the information is useable); the inclusion of mental health as a priority area; the importance of CER on pediatric populations; the importance of CER on prevention; and the need for training, and for starting to build the pipeline early.

The Council then briefly addressed next steps on the Report to Congress, including the fact that certain members would be assigned to draft specific sections of the report.

Next, the Council resumed its discussion of CER priorities where it had left off: looking at research, dissemination, data infrastructure, and cross-cutting investments. There was general consensus that OS funds should focus primarily on the latter three areas (as AHRQ and NIH are likely to make CER investments in research); there was also discussion about how to frame the priorities, including whether they should be framed around the type of CER investment or around types of diseases (e.g., people with multiple chronic illnesses, or people with disabilities and chronic illnesses). There was also specific discussion about the need to improve dissemination of research results—and a related topic, impacting practice. "If we just talk about dissemination," said one Council member, "we won't get anywhere. We need to look at the best methods for impacting practice."

There was also discussion around the question of how the Council should think about structuring its Report to Congress. At issue was whether the report should focus primarily on guidance to the HHS Secretary on how to allocate the $400 million in OS funds. In addition, the Council discussed the research time horizon, and whether ARRA monies could be used to fund projects that will have a time horizon longer than two years. One member suggested that one way to think about the question was to reframe it and ask, "Can we think about creating research centers that will be great resources into the future?"

Council members also stressed the need for the Council to address in its report the *process* for its deliberations and its recommendations, including making clear that CER investments are weighted to public health needs and responsive to the needs of decision makers. Council members suggested that some of the discussion about impacting practice might be linked to the discussion about data infrastructure investments.

May 29, 2009

The Council honed in on the details of the strategic framework for comparative effectiveness research, and the fact that it represents a comprehensive, coordinated approach to Federal investment in CER priorities that is intended to support immediate decisions for investments in CER priorities and to provide a comprehensive basis for longer-term CER investment decisions.

The Council discussed a framework that includes four major categories of activity (research, human and scientific capital, data and research infrastructure, and translation and adoption). The framework is designed to allow for investments within a single category or to cross-cut priority

themes. The Council agreed upon the categories. The Council's next step will be to determine the recommended mix among the major activities for OS funds.

The Council agreed to post on the hhs.gov/recovery Web site a copy of the broad framework diagram as well as a more detailed version to inform the public and to seek feedback on the strategic framework.

Next, the Council looked at some examples of the types of investments that might be made in the areas of infrastructure and translation and adoption. The idea of the discussion was to enable members to think about what types of projects might address gaps and further the CER enterprise.

The Council also looked briefly at an updated draft outline for the Report to Congress, and then members heard a presentation on three possible categories for investments in disability comparative effectiveness research.

June 5, 2009

The Council discussed the first draft of the Report to Congress. There was consensus that the Executive Summary needed to better frame the conversation around the value of CER to inform patients, clinicians, and other stakeholders. There was also discussion about setting out, early in the body of the report, why CER matters and how it matters to each stakeholder group. In addition, the Council agreed to add an additional appendix that contains a summary of its meetings and deliberations.

Next, the Council took up its recommendations for priorities. The discussion revolved around four key issues: the balance in spending priorities among the major activities versus cross-cutting themes; the distribution of spend priorities across the four major activities; what themes should be prioritized (and what the distribution of spend priorities should be across those themes); and whether the overall distribution makes sense vis-à-vis the prioritization criteria.

Regarding the distribution of spend priorities across the four major activities, Council members generally agreed that the majority of funding (e.g. 60 percent) should be spent on activities rather than themes. At the same time, there were lingering questions about the need to identify research gaps, implementation gaps, or both.

Regarding the distribution of spend priorities across the four major activities, the Council supported a breakdown that focuses the bulk of the funding in the areas of infrastructure (e.g. 60 percent) and translation (e.g. 20 percent). Members noted that there is a unique opportunity with ARRA funds to make significant investments in infrastructure.

Regarding potential priorities, members looked at draft lists of both priority populations and types of interventions. On the populations side, one Council member said that all of the proposed priority populations share in common that they have not traditionally been enrolled in clinical trials. There was also discussion about the need to include veterans as well as people with co-occurrence of mental health disorders along with physical comorbidities. On the interventions side, there was some discussion about the inclusion of delivery systems, and that

CER on delivery systems offers an opportunity to look at promising practices and how they might be scaled up and disseminated.

Finally, the Council was divided as to whether the bulk of OS funds should be used primarily for investments in populations or in interventions—or whether they should be equally important priorities.

June 12, 2009
The Council debriefed on what was heard in the third listening session. This generated enhancement to the common themes and some new information to be incorporated. The Council then revised the definition, threshold and prioritization criteria, and strategic framework based on the feedback from the session and the feedback received online. The Council then further discussed priority recommendations and the Report to Congress. The Council suggested edits for the Report prior to it going into clearance the next week.

Appendix C. PRELIMINARY DATA INFRASTRUCTURE AND CER BY CONDITION

The following is a preliminary inventory of examples of CER data infrastructure and CER by condition.

Person-Level Health Care Research Databases from First Inventory

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
US Federal							
Healthcare Cost and Utilization Project (HCUP)	AHRQ	Hospital information system	—	All	Y	N	Y
HIV Cost and Services Utilization Study (HCSUS)	AHRQ	Surevy & records abatsraction	2,864	HIV	Y	N	Y
AIDS Cost and Services Utilization Study (ACSUS)	AHRQ	Hospital information system	1,900	AIDS	Y	N	Y
National Vital Statistics	CDC	Surveillance program/registry data	—	All	n/a	N	N

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
National Vital Statistics—Natality	CDC	Surveillance program/registry data	4 million	All	n/a	N	Y
National Health Interview Survey	CDC	Survey	87,000	All	n/a	Y	Y
National Health and Nutrition Examination Survey	CDC	Survey	5,000	All	n/a	Y	Y
National Ambulatory Medical Care Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Hospital Ambulatory Medical Care Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Hospital Discharge Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Nursing Home Survey	CDC	Survey and records abstraction	13,507	All	N	Y	Y
National Home and Hospice Care Survey	CDC	Survey and records abstraction	9,416	All	N	Y	Y
Chronic Condition Data Warehouse	CMS	Administrative claims database, enrollment data, health assessment data, prescription drug event data	45 million	All	Y	Y	Y
Hospice Standard Analytical File (Hospice SAF)	CMS	Administrative claims database	—	All	Y	Y	?

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Medicaid Statistical Information System Personal Summary File (MSIS Personal Summary File)	CMS	Administrative claims database, EMR/EHR system	—	All	Y	Y	Y
National Claims History (NCH) 100% Nearline Fil	CMS	Administrative claims database	—	All	Y	Y	?
MEDPAR Claims Data	CMS	Administrative claims database	—	All	Y	Y	Y
MMA Part D Claims Data	CMS	Pharmacy claims database	25 million	All	Y	Y	Y
Sentinel System	FDA	Surveillance program/registry data	N/A	n/a	N	Y	N
SEER (Surveillance Epidemiology and End Results)	NCI	Surveillance program/registry data	11.4 million	Cancer	Y	N	Y
SEER-Medicare database	NCI, CMS	Administrative claims database, Surveillance program/registry data	3.3 million	Cancer	Y	Y	N
Cancer Research Network (CRN)	NCI, AHRQ	Administrative claims database, EMR/EHR system	—	Cancer	Y	Y	N
Computerized Patient Record System (CPRS)	VA	EMR/EHR system	4.2 million	All	Y	N	N
Diabetes Epidemi-ology Cohort	VA	Surveillance program/registry data	> 4,800	Diabetes	Y	Y	Y

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Hepatitis C Registry	VA	Surveillance program/registry data	>60 K	Hepatitis C	Y	N	Y
Immunological Case Registry	VA	Surveillance program/registry data	>15 K	HIV	Y	N	Y
Dementia Registry	VA	Surveillance program/registry data	>150 K	Dementia	Y	N	N
National Surgery Quality Improvement Program	VA	Surveillance program/registry data	>1 Million	All major surgery	Y	Y	Y
Scientific Registry of Transplant Recipients (SRTR)	HRSA	Transplant registry and outcomes data		Organ specific	Y	Y	Y
Pediatric Emergency Care Applied Research Network (PECARN) CDMCC*	HRSA	Emergency medical services for children	800,000 + patients	Emergency Services to Children	Y	Y	Y
AIDS Drug Assistance Program (ADAP)	HRSA	Care Program Registry Data	—	HIV/AIDS	Y	Y	N
US Private Sector							
National Oncologic PET Registry (NOPR)	Academy of Molecular Imaging	Intervention program data	>100,000	Cancer	Y	Y	?
Cerner Health Facts Database	Cerner	EMR/EHR system	—	All	Y	Y	Y
GE Centricity	GE	EMR/EHR system	10 million	All	Y	N	Y

* Central Data Management and Coordinating Center

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Ingenix Research Data Mart (RDM) Database	Ingenix	Administrative claims database	>39 million	All	Y	Y	Y
Premier Perspective Data Warehouse	Premier	Administrative claims database	—	All	Y	Y	Y
MarketScan Data Warehouse	Thomson-Reuters	EMR/EHR system	—	All	Y	N	N
International Databases							
General Practice Research Database (GPRD)	NHS (UK)	EMR/EHR system	> 3.6 million	All	Y	Y	Y
NHS Care Records Service (CRS)	NHS (UK)	EMR/EHR system	2 million	All	Y	N	Y
The Health Improvement Network (THIN)	INPS and EPIC (UK)	EMR/EHR system	—	All	Y	Y	Y

Priority Diseases/Conditions in CER

Priority Diseases/Conditions	AHRQ (n=178)	NIH (n=513)	DoD (n=26)	VHA (n=106)	Total (n=823)
Arthritis and non-traumatic joint disorders	6%	1%	0%	3%	**2%**
Cancer	10%	7%	23%	7%	**8%**
Cardiovascular disease, including stroke and hypertension	20%	10%	4%	23%	**13%**
Dementia, including Alzheimer's Disease	1%	1%	0%	3%	**1%**
Depression and other mental health disorders	8%	16%	8%	18%	**14%**
Developmental delays, attention-deficit hyperactivity disorder, and autism	4%	1%	0%	0%	**1%**
Diabetes mellitus	11%	11%	0%	8%	**10%**
Functional limitations and disability	8%	4%	15%	7%	**5%**

Infectious diseases including HIV/AIDS	3%	11%	0%	6%	**8%**
Obesity	1%	3%	0%	2%	**3%**
Peptic ulcer disease and dyspepsia	0%	0%	0%	0%	**0%**
Pregnancy, including preterm birth	1%	4%	0%	0%	**2%**
Pulmonary disease/asthma	5%	3%	0%	4%	**3%**
Substance abuse	2%	19%	0%	9%	**14%**
Other	20%	11%	50%	12%	**14%**

*Studies focusing on patients with more than one priority disease or condition are counted in applicable rows..
**NIH 2008 plus NIH multi-year sample.

Appendix D. COUNCIL LIST AND STAFF SUPPORT

1. Carolyn Clancy, MD	AHRQ
2. Peter Delaney, PhD, LCSW-C	SAMHSA
3. Ezekiel Emanuel, MD, PhD	OMB
4. Jesse Goodman, MD, MPH	FDA
5. Garth Graham, MD, MPH	Office of Minority Health
6. Anne Haddix, PhD	CDC
7. Deborah Hopson, PhD, RN	HRSA
8. David Hunt, MD	ONC
9. Michael Kilpatrick, MD	Dept of Defense
10. Joel Kupersmith, MD	Dept of VA
11. Michael Marge, Ed.D.	Office of Disability
12. Elizabeth Nabel, MD	NIH
13. James Scanlon, PhD	ASPE
14. Neera Tanden, JD	Office of the Secretary
15. Tom Valuck, MD, MHSA, JD	CMS

Executive Director: Patrick Conway, MD, MSc
Deputy Executive Director: Cecilia Rivera Casale, PhD

Alternates to the Council participating: Kelley Brix, Margaret Cary, Rosaly Correa-de-Araujo (replaced Michael Marge on Council June 12th), Elisabeth Handley, Lynn Hudson, Michael Millman

Contributors to Council and Report: Kate Goodrich, Lauren Hunt, John Poelman, Daria Steigman, Caroline Taplin, Jordan VanLare.

Appendix E. THE AMERICAN RECOVERY AND REINVESTMENT ACT STATUTE RELATED TO CER AND COUNCIL

Appropriations

For an additional amount for 'Healthcare Research and Quality' to carry out titles III and IX of the Public Health Service Act, part A of title XI of the Social Security Act, and section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, $700,000,000 for comparative effectiveness research: *Provided,* That of the amount appropriated in this paragraph, $400,000,000 shall be transferred to the Office of the Director of the National Institutes of Health ('Office of the Director') to conduct or support comparative effectiveness research under section 301 and title IV of the Public Health Service Act: *Provided further,* That funds transferred to the Office of the Director may be transferred to the Institutes and Centers of the National Institutes of Health and to the Common Fund established under section 402A(c)(1) of the Public Health Service Act: *Provided further,* That this transfer authority is in addition to any other transfer authority available to the National Institutes of Health: *Provided further,* That within the amount available in this paragraph for the Agency for Healthcare Research and Quality, not more than 1 percent shall be made available for additional full-time equivalents.

In addition, $400,000,000 shall be available for comparative effectiveness research to be allocated at the discretion of the Secretary of Health and Human Services ('Secretary'): *Provided,* That the funding appropriated in this paragraph shall be used to accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that: (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions; and (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data: *Provided further,* That the Secretary shall enter into a contract with the Institute of Medicine, for which no more than $1,500,000 shall be made available from funds provided in this paragraph, to produce and submit a report to the Congress and the Secretary by not later than June 30, 2009, that includes recommendations on the national priorities for comparative effectiveness research to be conducted or supported with the funds provided in this paragraph and that considers input from stakeholders: *Provided further,* That the Secretary shall consider any recommendations of the Federal Coordinating Council for Comparative Effectiveness Research established by section 804 of this Act and any recommendations included in the Institute of Medicine report pursuant to the preceding proviso in designating activities to receive funds provided in this paragraph and may make grants and contracts with appropriate entities, which may include agencies within the Department of Health and Human Services and other governmental agencies, as well as private sector entities, that have demonstrated experience and capacity to achieve the goals of comparative effectiveness research: *Provided further,* That the Secretary shall publish information on grants and contracts awarded with the funds provided under this heading within a reasonable time of the obligation of funds for such grants and contracts and shall disseminate research findings from such grants and contracts to clinicians, patients, and the general public, as appropriate: *Provided further,* That, to the extent feasible, the Secretary shall ensure that the recipients of the funds provided by this paragraph offer an opportunity for public comment on

the research: *Provided further,* That research conducted with funds appropriated under this paragraph shall be consistent with Departmental policies relating to the inclusion of women and minorities in research: *Provided further,* That the Secretary shall provide the Committees on Appropriations of the House of Representatives and the Senate, the Committee on Energy and Commerce and the Committee on Ways and Means of the House of Representatives, and the Committee on Health, Education, Labor, and Pensions and the Committee on Finance of the Senate with an annual report on the research conducted or supported through the funds provided under this heading: *Provided further,* That the Secretary, jointly with the Directors of the Agency for Healthcare Research and Quality and the National Institutes of Health, shall provide the Committees on Appropriations of the House of Representatives and the Senate a fiscal year 2009 operating plan for the funds appropriated under this heading prior to making any Federal obligations of such funds in fiscal year 2009, but not later than July 30, 2009, and a fiscal year 2010 operating plan for such funds prior to making any Federal obligations of such funds in fiscal year 2010, but not later than November 1, 2009, that detail the type of research being conducted or supported, including the priority conditions addressed; and specify the allocation of resources within the Department of Health and Human Services: *Provided further,* That the Secretary, jointly with the Directors of the Agency for Healthcare Research and Quality and the National Institutes of Health, shall provide to the Committees on Appropriations of the House of Representatives and the Senate a report on the actual obligations, expenditures, and unobligated balances for each activity funded under this heading not later than November 1, 2009, and every 6 months thereafter as long as funding provided under this heading is available for obligation or expenditure.

Sec. 804. Federal Coordinating Council for Comparative Effectiveness Research

(a) ESTABLISHMENT— There is hereby established a Federal Coordinating Council for Comparative Effectiveness Research (in this section referred to as the 'Council').

(b) PURPOSE— The Council shall foster optimum coordination of comparative effectiveness and related health services research conducted or supported by relevant Federal departments and agencies, with the goal of reducing duplicative efforts and encouraging coordinated and complementary use of resources.

(c) DUTIES— The Council shall—

(1) assist the offices and agencies of the Federal Government, including the Departments of Health and Human Services, Veterans Affairs, and Defense, and other Federal departments or agencies, to coordinate the conduct or support of comparative effectiveness and related health services research; and

(2) advise the President and Congress on—
(A) strategies with respect to the infrastructure needs of comparative effectiveness research within the Federal Government; and
(B) organizational expenditures for comparative effectiveness research by relevant Federal departments and agencies.

(d) MEMBERSHIP—

(1) NUMBER AND APPOINTMENT— The Council shall be composed of not more than 15 members, all of whom are senior Federal officers or employees with responsibility for health-related programs, appointed by the President, acting through the Secretary of Health and Human Services (in this section referred to as the 'Secretary'). Members shall first be appointed to the Council not later than 30 days after the date of the enactment of this Act.

(2) MEMBERS—
(A) IN GENERAL— The members of the Council shall include one senior officer or employee from each of the following agencies:
(i) The Agency for Healthcare Research and Quality.
(ii) The Centers for Medicare and Medicaid Services.
(iii) The National Institutes of Health.
(iv) The Office of the National Coordinator for Health Information Technology.
(v) The Food and Drug Administration.
(vi) The Veterans Health Administration within the Department of Veterans Affairs.
(vii) The office within the Department of Defense responsible for management of the Department of Defense Military Health Care System.
(B) QUALIFICATIONS— At least half of the members of the Council shall be physicians or other experts with clinical expertise.
(3) CHAIRMAN; VICE CHAIRMAN— The Secretary shall serve as Chairman of the Council and shall designate a member to serve as Vice Chairman.

(e) REPORTS—

(1) INITIAL REPORT— Not later than June 30, 2009, the Council shall submit to the President and the Congress a report containing information describing current Federal activities on comparative effectiveness research and recommendations for such research conducted or supported from funds made available for allotment by the Secretary for comparative effectiveness research in this Act.

(2) ANNUAL REPORT— The Council shall submit to the President and Congress an annual report regarding its activities and recommendations concerning the infrastructure needs, organizational expenditures and opportunities for better coordination of comparative effectiveness research by relevant Federal departments and agencies.

(f) STAFFING; SUPPORT— From funds made available for allotment by the Secretary for comparative effectiveness research in this Act, the Secretary shall make available not more than 1 percent to the Council for staff and administrative support.

(g) RULES OF CONSTRUCTION—

(1) COVERAGE— Nothing in this section shall be construed to permit the Council to mandate coverage, reimbursement, or other policies for any public or private payer.

(2) REPORTS AND RECOMMENDATIONS— None of the reports submitted under this section or recommendations made by the Council shall be construed as mandates or clinical guidelines for payment, coverage, or treatment.

Title VIII—Departments of Labor, Health And Human Services, and Education, and Related Agencies

The Underserved and Health Information Technology: Issues and Opportunities

Prepared by:

Adil Moiduddin
Jonathan Moore

November, 2008

Prepared for:
Office of the Assistant Secretary for Planning and Evaluation (ASPE)
U.S. Department of Health and Human Services (HHS)

This report was produced under the direction of Caroline Taplin and Dale Hitchcock, Project Officers, Office of the Assistant Secretary for Planning and Evaluation (ASPE), Office of Science and Data Policy. The findings and conclusions of this report are those of the authors and do not necessarily represent the views of ASPE or HHS.

This report is available on the Internet at:
http://aspe.hhs.gov/health/sp/reports/2008/underserved/report.html

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Footnotes

Introduction and Objectives

NORC at the University of Chicago is pleased to present this white paper entitled "The Underserved and Health Information Technology: Issues and Opportunities" for the Assistant Secretary for Planning and Evaluation (ASPE) at the U.S. Department of Health and Human Services (HHS). Due to recent advances in technology and greater attention to problems associated with quality and efficiency of health care delivery, we see new opportunities to improve the health and health care for underserved Americans through the use of emerging information technologies.

President George W. Bush announced an Executive Order in 2004 prioritizing the adoption and use of health information technology (health IT) by patients and providers as well as the use of secure health information exchange (HIE) to improve the quality, safety, effectiveness and efficiency of health care delivery in the United States, and creating the Office of the National Coordinator for Health IT (ONC). Several agencies within HHS including the Health Resources and Services Administration (HRSA), the Indian Health Service (IHS), the Office of Minority Health (OMH), the Agency for Healthcare Research and Quality (AHRQ), the Centers for Medicare and Medicaid Services (CMS), the Office of Disease Prevention and Health Promotion (ODPHP), ASPE and others had been working on programs and policies to assure that the nation's most vulnerable Americans are not left behind as the health care sector moves to adopt a more automated information driven approach to promoting health and preventing and treating illness. More recently several States have joined in these activities by sponsoring their own programs to encourage health IT adoption.

This purpose of this paper is to summarize a selected set of programs, policies and research findings that demonstrate both the potential for health IT to improve health and health care to underserved Americans as well as the challenges and barriers facing effective use of these technologies. We will attempt to cover an array of technologies including electronic health records (EHRs), e-Prescribing (eRx), chronic disease registries and clinical decision support systems (CDS) by health care providers predominantly serving the underserved. Additionally, we will examine technologies such as personal health records (PHRs), messaging and reminder systems, patient kiosks and other technologies that are "patient facing" where the theory is that IT can empower patients to take more control over their own health information and health care.

Finally, we will provide summary conclusions regarding what is known and yet to be understood regarding use of health IT among the underserved and highlight areas where further programmatic, policy or research activities sponsored by the federal government or others may be important. We begin with a brief discussion on health and health care challenges facing underserved Americans.

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Why Focus on the Underserved?

While there is no single, universally accepted definition of the underserved, for the purpose of this paper, we characterize this population simply as those living in the United States who do not have adequate access to health care services. They share one or more of these characteristics: they may be poor; uninsured; have limited English language proficiency and/or lack familiarity with the health care delivery system; or live in locations where providers are not readily available to meet

their needs. Members of ethnic and racial minority groups are not by definition "underserved", but are disproportionately found among their numbers. Disparities in health status and access to health care that leave these populations worse off relative to others have been well documented by HHS over the last fifteen years.

Of the diverse set of groups represented among the underserved, perhaps disparities in health and health care of racial and ethnic minorities have been most thoroughly documented. Recently the Commonwealth Fund released a chart book analyzing data on the status of racial and ethnic minorities in the United States (available at http://www.commonwealthfund.org/publications). This effort compliments the primary federal government publication on health disparities, the "National Healthcare Disparities Report", released annually by AHRQ with the last release for 2007 occurring in February of this year (available at http://www.ahrq.gov/qual/qrdr07.htm).[1] Findings from both publications demonstrate persistent disparities in health status, access to health insurance, access to critical health care services such as a primary care, home and specialty care, and in the quality of care received by disadvantaged groups.[2] The Commonwealth summary shows that as of 2006, data from surveys conducted by federal agencies including AHRQ, the National Center for Health Statistics (NCHS) of the Centers for Disease Control and Prevention (CDC) and the National Institutes of Health (NIH) demonstrate higher rates of self-reported poor health status, chronic disease and disability, life expectancy at birth, obesity, cancer (especially breast and colorectal), heart disease, diabetes, HIV/AIDS and other chronic illnesses among blacks compared to non-Hispanic whites in the United States. The chartbook also demonstrates specific types of behavioral and health status disparities for other racial and ethnic groups such as higher smoking prevalence and mental distress among American Indian and Alaska Native populations, and higher incidence of some infection-related cancers among Hispanics.

In terms of access to health care, the Commonwealth chart book shows greater percentages of Hispanic adults and adults of other racial and ethnic minorities reporting they have no doctor compared to whites, with the Hispanic average for this question being 2.5 times that of whites. They also show that blacks and Hispanics are less likely to report private physicians as their usual source of care relative to whites and are more likely to report going to community health centers and emergency rooms as their usual source of care or having no usual source of care. Hispanics also are much more likely than whites to report being uninsured during a given year. Some of these disparities are heavily mediated by income and are reduced or eliminated by controlling for this characteristic, suggesting the importance of viewing the low income population broadly as part of the underserved. However, many of these disparities, particularly as they relate to health status among blacks and access and insurance disparities issues among Hispanics persist even after adjusting for income.

Finally, the data on racial and ethnic disparities also demonstrates important differences in the quality of care delivered to ethnic and racial minorities. Primary care physicians who treat predominantly black patients report being unable to provide high quality care to all of their patients at a higher rate than similar physicians who treat a predominantly white population. Hispanics and Asians, on average, report wait times longer than others to get a doctor's appointment. Hispanics also report being less likely to have received recommended screenings such as blood cholesterol and cancer screenings (colorectal and cervical) relative to others. Racial and ethnic minorities in general are less likely to have received a pneumococcal vaccination, a dental visit in the past year (for children), and first trimester prenatal care (women) than the equivalent non minority populations.[3]

Racial and ethnic minorities aside, evidence from research shows that low-income populations

generally experience more disjointed care, being twice as likely to lack a regular source of care.[4] Those with low socioeconomic status (SES) have limited access to quality health care as it relates to primary, specialty, dental and behavioral care relative to others.[5] Additionally, lower SES patients experience lower rates of preventive care and chronic disease management.[6] [7] We found no definitive evidence that rural residents, as a group, experience lower quality care; however, low population density in rural areas makes care less convenient and more costly. Additionally, rural populations are more likely to be uninsured and of low income, making these areas more susceptible to the barriers faced by low-income populations.[8]

The AHRQ National Healthcare Disparities report cited above focuses heavily on disparities in measures that address quality of care. AHRQ and the Inter-Agency HHS work group that contributes to this report provide data on 211 measures including 41 core measures on quality and access. Comparisons are produced across various demographic groups including blacks compared to whites, Asians compared to whites, Hispanics compared to non-Hispanics, poor compared to high income and American Indian and Alaskan Native compared to whites. Findings from the latest version of this report for 2007 show some reduction in previously identified disparities facing segments of the underserved for measures such as adequacy of hemodialysis treatment and childhood vaccination rates for blacks versus whites. However, the findings also demonstrate the persistence of disparities and little or no improvement over time in the disparities associated with many measures since the start of this initiative.[9]

Given findings from the research and analysis presented above, as well as a wealth of additional evidence suggesting similar trends of poor health and access to quality health care among groups comprising the underserved, improving care to this group represents an important challenge for policy makers and program leaders in federal and State government. Some have speculated that increased automation in health care and use of advanced IT could exacerbate rather than address these disparities if a "digital divide" threatens to leave underserved populations behind, resulting in a situation where only those who have historically enjoyed steady health insurance and access to private medical providers would benefit from these advances. At the same time, many of the most promising potential improvements in care due to health IT adoption including clinical decision support to alert providers to the need for increasing use of screenings, preventive care, and behavioral counseling and patient registries that facilitate active management of chronic illness, could be of disproportionate benefit to the underserved. Finally, as the use of technologies such as PHRs serve to empower patients to take a more active role in their health care and overall well-being, it is increasingly important to recognize and address barriers to making the benefits of these technologies readily available to underserved populations who stand to benefit the most.

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What Role Can Health IT Play?

Researchers have struggled to definitively characterize all the factors associated with disparities in health and health care for the underserved and assign appropriate importance to each factor. There is, however, a consensus regarding the significance of these problems and a solid interest among program and policy leaders to understand the manner in which advances in public health, health care delivery and health care financing can work to address these disparities. While it is clear that these disparities are a result of a complex network of factors including personal, family, cultural, neighborhood and economic variables in addition to those associated with the direct provision of necessary health care services, many have identified health IT as a means to facilitate behavioral

and organizational changes to improve the health and health care of the underserved.

For the purpose of this paper we define health IT as technology that enables patients and providers to support better health and health care by providing targeted information meant to inform, educate or generally allow for improved decision making. In some cases, the information provided by these technologies is traditionally accessed by other means such as paper patient charts, evaluations and clinical summaries transferred via fax or by hand between providers or even clinical flow sheets that outline appropriate care for specific clinical situations based on accepted clinical guidelines.

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Exhibit 1: Health IT Applications [10]

Health IT Applications

Product or Functionality	Description
Electronic Health Record (EHR)	An electronic record of health-related information on an individual that conforms to nationally recognized interoperability standards and that can be created, managed, and consulted by authorized clinicians and staff across more than one health care organization.
Electronic Medical Record (EMR)	An electronic record of health-related information on an individual that can be created, gathered, managed, and consulted by authorized clinicians and staff within one health care organization.
e-Prescribing (eRx)	Enables a physician to transmit a prescription electronically to the patient's choice of pharmacy. It also enables physicians and pharmacies to obtain information about the patient's eligibility and medication history from drug plans. Often comes with built in alerts for drug-drug, drug-allergy and drug-disease interactions.
Personal Health Records	An electronic record of health-related information on an individual that conforms to nationally recognized interoperability standards and that can be drawn from multiple sources while being managed, shared, and controlled by the individual.
Computerized Physician Order Entry (CPOE)	Refers to a computer-based system of ordering medications and often other tests. Physicians directly enter orders into a computer system that can have varying levels of sophistication. Basic CPOE ensures standardized, legible, complete orders, and thus primarily reduces errors due to poor handwriting and ambiguous abbreviations.
Clinical Decision Support (CDS)	Any system designed to improve clinical decision making related to diagnostic or therapeutic processes of care. CDS addresses activities ranging from the selection of drugs (e.g., the optimal antibiotic choice given specific microbiologic data) or diagnostic tests to detailed support for optimal drug dosing and support for resolving diagnostic dilemmas. Often incorporated as part of CPOE or EMR/EHR systems.
Disease Registries	A database feature that includes key clinical data on a subset of chronically ill patients for the purpose of tracking their condition and managing treatment.

In other cases, health IT enables providers and patients to access information that they otherwise would not be able to access. Exhibit 1 above outlines some basic definitions of health IT applications. Implemented properly, these technologies can create tremendous efficiencies and

improvements in health and health care by providing "the right information to the right person at the right time". This results in benefits such as greater adherence to evidenced based guidelines for screenings and vaccinations, better patient compliance with treatment instructions, reductions in medication errors and improved patient education.

As noted above, specific technologies that are most commonly referred to as health IT include electronic health records (EHRs) and personal health records (PHRs), the former being the systems used by providers to electronically record and maintain patient demographic information and information on activities, diagnoses and orders associated with a clinical episode, and the latter being an application for use by patients to access and update information related to their own health and health care or that of a family member or loved one. One key distinction between EHRs and PHRs lies in defining the locus of usage and control. EHRs are controlled and used primarily by the provider whereas PHRs are used and controlled by patients and their surrogates.
Additional applications include functionalities that may be considered add-ons to these core concepts, including clinical decision support (CDS) which in the context of an EHR would provide patient specific information on an appropriate course of treatment based on clinical effectiveness research; issue alerts if an order entered by the clinician is counter-indicated based on the patient's profile; or provide reminders regarding the need to order specific interventions such as screenings, vaccinations, blood tests or foot exams.

In addition to PHRs, other health IT applications aimed at patients include health kiosks, where patients could obtain information on health conditions or access to information on their own health and health care using publicly available computer terminals set up within the community. These may also include messaging systems that allow transmission of reminders, information to guide healthful behavior, or even direct communication with providers through email or short message service (SMS) messaging accessed via cell phones or personal digital assistants (PDAs). In a 2006 report entitled, "Expanding the Reach and Impact of Consumer e-Health Tools", the Office of Disease Prevention and Health Promotion at HHS identified a series of functions that might be considered some of the key potential benefits to patients using health IT. These functions included provision of health information to patients in a searchable format to help with researching treatment options; support for behavior modification and self management of a healthy lifestyle; access to online communities for interacting with others on health issues; and functions that allow joint management and tracking of treatment involving online collaboration between patients and clinicians.[11]

Another use of health IT involves health information exchange (HIE) which refers to the electronic exchange of data on a particular patient in a secure format between relevant administrative and clinical stakeholders such as other clinicians, payers and patients themselves. HIE can enable more efficient exchange of data between different types of providers (e.g., ambulatory and acute care providers or between primary care and specialty care) or between providers and patients (EHRs to PHRs). Some HIE functionality can also be built into PHRs and EHRs to enable specific services such as electronic prescribing (eRx) and refilling of prescription medications, ordering tests, receiving results from clinical laboratories, and maintaining ready access to radiology reports and results from tests conducted in an inpatient environment.

A central premise of our paper is that health IT applications such as the ones described in Exhibit 1 represent potentially effective mechanisms for achieving basic goals associated with improving health, health care and access to care for the underserved. As has been often noted, there is no evidence to suggest that health IT adoption in and of itself will transform the health or health care of the underserved. However, if based in broader initiatives for increasing enrollment in public

programs, improving quality of care, empowering patients through improved access to information and streamlining and simplifying health care delivery, health IT may be a critical ingredient to achieving important changes.

In particular, because all IT and systems initiatives are designed around managing information in a standardized and efficient manner, these technologies can play an important role in assuring that the right information, in the right format is available to the right person and the right time to improve family and provider decision making, improve access to care and better target resources. Over time, health IT applications may lead to a richer set of data from which population health care trends can be assessed, thereby contributing to the development of better knowledge on the causes of disparities affecting health and health care for the underserved, getting a better sense of the barriers to improving their status relative to those disparities and contributing to programmatic and policy initiatives informed by a richer set of data than are currently available.

In the remainder of this paper we describe in greater detail opportunities and challenges associated with the use of health IT to improve health and health care of the underserved. For the sake of simplicity, in some places we organize this discussion between those technologies that can be considered primarily provider facing, such as EHRs, and those technologies that are primarily patient facing, such as PHRs. We note however that this is a somewhat artificial distinction as effective approaches to improving health may need to take advantage of technologies that enable direct electronic communication between providers and patients through direct messaging and are, in that sense, both provider and patient facing. We end the paper with summary conclusions for different health care sector stakeholders and address areas that merit further exploration through research and evaluation.

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Current State of Health IT and the Underserved

Although there is little population-based research on the use of health IT by the underserved and those that treat them specifically, there is some evidence that the groups disproportionately represented among the underserved and those that treat them are currently less likely to use health IT compared to other groups. For example, in the paragraphs below we cite recent research that shows that lower income Americans, some ethnic and racial minorities as well as residents in rural areas are less likely to have broadband access and are less likely to use the Internet than others, that individuals who lack steady access to health care are less likely to go online to get health related information and that safety net health centers that disproportionately serve the poor and uninsured are less likely than other providers to use electronic health records. [12] [13] [14] [15]

On the other hand, other research suggests that the income and education related factors are not as important in predicting Internet use as it has been in the past and that among individuals who use the Internet regularly, there is no difference by income, age or gender in the propensity to go online for communicating with providers or seeking information on health and health care.[16] There is also evidence that many safety net health care providers have adopted health IT through the support of federal grants and are leaders in ambulatory health IT use. It is important to note however that adoption and access to health IT does not automatically translate to improvements in quality of care or health status.

Most of the research conducted to date has been limited in scope, looking at the potential for specific health IT interventions to improve limited aspects of quality of care for minorities or other

groups represented in the underserved populations. While there has been some survey work conducted examining the use of health IT among health care providers that treat the underserved, there is almost no information on the use of patient-facing health IT applications by underserved individuals and families in the United States. This is predominantly because the extent to which PHRs are used by any group within the United States is largely unknown but considered to be limited due to the novel nature of these technologies, lack of knowledge of them among the public, and limited availability of PHRs sponsored by traditional sources of health care information for patients: providers, payers and purchasers. There are currently efforts underway sponsored by AHRQ and others to elucidate these issues.[17]

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Direct Use of Health IT by Underserved Populations

As noted above, there is no research that looks at underserved populations as a whole and assesses use of patient-facing health IT applications such as PHRs, health kiosks or SMS-based messaging to generate evidence on the prevalence of use or attitudes towards these technologies among underserved Americans. However, there are data that can be used as proxies to assess the extent to which these technologies are accessible to the underserved and the likelihood that they will be adopted in the near future. These proxies include evidence regarding access to and use of information technology and communications technologies by groups represented among the underserved generally and in the context of health in particular. We explore available evidence on these questions below.

Some differences persist between underserved populations and the broader population with regard to internet usage and online health information seeking behavior. While current estimates of Internet usage suggest that more than half of individuals in all income and racial and ethnic groups have access to the Internet and use it on a consistent basis, there is still evidence that some racial and ethnic minorities as well as lower income individuals use the Internet in lower proportions compared to the general population.[18] Additionally, urban and suburban residents are more likely than rural residents to use the Internet regularly, with 77 percent of urban and suburban residents reporting usage compared with 64 percent of rural residents in 2006.[19] Thus, it is reasonable to conclude that the underserved are less likely than others to have access to the Internet and, if they have access, use it on a less frequent basis than others.

There is also evidence that those with a longer history of Internet use and who visit the doctor regularly are more likely than others to go online to access information on health.[20] Again, this trend suggests that the underserved, may be less likely than others to use the Internet as a tool for improving their health or health care as they are disproportionately "new" users of the Internet. However, it is important to note that recent research by both the Pew Internet and the American Life Project and the National Cancer Institutes National Health Information Trends Survey (HINTS) suggests that, among the group of individuals who are Internet users, income and education are not associated with use of the Internet for communicating with providers or seeking health related information.[21] [22]

Because the model for most patient-facing health IT applications involves use of the Internet as a major conduit for accessing, exchanging and maintaining information relevant to the health and health care of a an individual or family, many of these technologies may be less useful for underserved Americans compared to other groups.[23] While this may signal the importance of

increasing general Internet access and usage among the underserved population, there is also evidence that Internet usage alone may not lead to adoption of patient-facing health IT applications among the underserved.[24] As such, developers of patient-facing health IT are increasingly looking to applications built on other platforms to meet the same objectives of giving patients access to the "right information at the right time" to make good decisions regarding their health. For example, there is increased attention to the potential of building health interventions around SMS or "text messages" via cell phones and PDAs. These interventions are meant to support maintenance of a healthy lifestyle by initiating reminders and alerts to patients or sharing test results and instructions with patients and there is some evidence, particularly from the developing world, that cell phones represent a cost effective medium for transferring critical health and health care messages to an underserved population.[25] [26]

Even though patient facing health IT applications are in early stages of adoption and usage, there are many targeted applications that have been developed, implemented and even evaluated on a small scale. In the ODPHP report referenced above, the authors identified and reviewed 40 separate "consumer e-Health tools" ranging from informational websites to PHRs with a vast array of functions. Selected examples of patient-facing health IT that are specifically targeted to underserved populations are reviewed below along with evidence regarding their success.

PHRs for migrant workers. [27] MiVIA, a PHR designed for migrant, Spanish-speaking populations in California, is web-based tool that allows for documentation of clinical visits, health conditions, allergies, medications and other information critical to maintaining continuity of care. Importantly, the tool also provides its target audience with other valuable services including a picture ID, a stable email address, access to Medline and provider websites and emergency ID information and is set up to allow access not only to the individual patient but also family and surrogates.

Finally, there is a provider view where clinicians are allowed to populate, view and update data on patients they are treating based on a specific episode of care. While we found no published independent evaluation of MiVIA, anecdotal information and internally conducted assessments suggest that a significant percentage of individuals signed up for and have accounts with MiVIA use it regularly. Additionally, clinicians report that use of MiVIA has dramatically improved their ability to provide effective health care to this highly mobile population.

Implementers of MiVIA note the importance of not simply making the tool available and publicizing it, but providing comprehensive education and support to assist individuals with every facet of using the application effectively. This included providing basic training on computer and Internet usage, providing information regarding locations where clients could access computers with Internet connections, as well as extensive repetitive training on the use of the application itself. Support and training are provided by "promoters de salud" or community health workers who are imbedded among the migrant workers and are able to conduct the training in a culturally appropriate manner using language that is familiar to clients. While MiVIA started as a local collaborative effort between labor and health care providers in Sonoma County California, it has expanded in recent years to other migrant farm communities on the West Coast and is being touted as a an optimal PHR solution for homeless individuals, children and others.

Online education and support systems for cancer patients.[28] [29] While MiVIA is an example of a relatively broad based PHR that is targeted to a specific demographic population regardless of their individual health status or needs, many patient facing health IT applications focus on providing targeted functionality to individuals who have similar health and health care characteristics. One such example is the Comprehensive Health Enhancement Support System (CHESS) developed by

the University of Wisconsin and the Cancer Information Service (CIS) which is part of the National Institutes of Health's (NIH), National Cancer Institute (NCI). CHESS is an online system that provides users with 11 services designed to improve quality of life for women diagnosed with breast cancer.

These include what are referred to as "information services" such as a static "Q&A" section; a library of reference articles on breast cancer topics; and resource guides on topics such as selecting providers, resource directories and links to other useful sites. In addition, the system offers "support services" allowing users to ask direct questions to clinical experts and gain emotional support by viewing text and video accounts from other cancer patients discussing how they coped with the disease. And, finally, it provides "decision services" that allow users to take emotional status assessments and receive tailored advice on coping, and use online health charts to track their health status. These health charts also direct users to information on their own specific health concerns and decision aids that help identify options, assign values, and elucidate potential consequences associated with key treatment and lifestyle decisions.

Recently published literature on the system demonstrates that CHESS is effective in improving social support, comfort with their doctors and the care they are receiving, information competence and quality of life among women with cancer. Research also suggests that CHESS is particularly effective on these measures for women considered "disadvantaged" based on income and insurance status because these individuals are the ones that are most likely to lack any resources in the absence of having access to a system such as CHESS.

In a subsequent study, researchers assessed the relative effectiveness of different approaches to disseminating and encouraging take up of CHESS among underserved women and found that while referrals from hospitals and doctors were effective in encouraging underserved women to make use of CHESS, different approaches were important for different communities. For example, they found that publicizing CHESS through radio advertisements was more effective among black women compared to others. They also demonstrated the benefits of extensive in person training for underserved women, not only on navigating and using CHESS, but more basic skills around use of computers and the Internet.

Using health kiosks.[30] [31] Standalone health kiosks offer some of the same functionality of PHRs and online support systems, but combine computer hardware and software in a single unit. This combination helps to address issues surrounding computer and internet access among some underserved groups. Additionally, physically placing kiosks in targeted locations (such as physician offices or health centers) could allow trained health assistants to assist users when needed. Hardware and software specifications also allow kiosk designers to limit internet and data access to a greater degree than would be possible in a home web-based system. In one pilot implementation among Australian aboriginals, health kiosks served as a first step toward greater health IT familiarity. The kiosks helped to address the connectivity and hardware issues facing those with the most limited experience with and access to new technologies.

Closer to home, a project initiated by the Duke University Medical Center tested the potential impact of kiosks to address the needs for undeserved residents in North Carolina[32]. Duke created a cluster of nine health kiosks in three counties in the state. DERICKs (Durham e-Health Resource Information Center Kiosks) are located in community health centers, medical centers, local departments of social service and emergency departments. The kiosks are used to help patients identify and overcome personal barriers to accessing care (e.g. transportation issues may lead the kiosk to recommend contacting a local transportation agency). To that end, the kiosk asks a series

of preliminary questions to gauge users' language preferences, education and literacy levels and levels of computer literacy. The responses to these questions help to tailor the questions related to access later in the kiosk application. All kiosk text is available in both English and Spanish and videos are available to walk users through all steps in the process. Users are also able to print out pamphlets via a built-in printer. These pamphlets document specific resources and provide customized guidance to help overcome barriers to better health. To date, the average DERICK user prints out three pamphlets.

While anyone can use DERICKs, they offer additional functionality for Medicaid beneficiaries. DERICK allows users to enter their Medicaid ID numbers. DERICK documents beneficiaries' barriers and transmits them electronically to their assigned case managers. This provides another point of access to the social service safety net for beneficiaries who may not have time to update their case managers after every ED or primary care visit. Data gathered through kiosks are transmitted through a local HIE, COACH (Community-Oriented Approach to Coordinated Healthcare). This connectivity allows other local providers to access information gathered via DERICK. While specific details remain unclear, Medicaid beneficiaries may be able to access medical records using kiosks in the future.

Others in the field have expressed greater skepticism toward the use of health kiosks. While customization for targeted populations is a clear benefit for underserved groups, little has been done to clarify what such changes would look like. Additionally, some research indicates that any benefit to the low running costs of health kiosks would be negated as health kiosk user satisfaction generally wanes over time. High initial costs suggest that health kiosks could amount to an expensive novelty for underserved communities.

Use of text messaging system. One emerging set of interventions aimed at improving the health of the undeserved through use of health IT takes advantage of wide adoption of cell phones that use SMS technologies to enable text messaging of health content directly to specific targeted populations. One of the several innovative projects initiated by the Robert Wood Johnson Foundation (RWJF) as part of their Project Health Design PHR initiative takes advantage of the fact that some populations making up the underserved are more likely to have cell phones than regular access to personal computers or laptops. For this initiative, RWJF developed the prototype for a system that would allow patients with heart disease to report on their status on a daily basis and then receive tailored information regarding recommended therapies and behaviors for that day.[33]

This project based at the University of Rochester aims at developing a prototype portable digital assistant with more functionality than the vast majority of cell phones (e.g., sophisticated speech recognition). It does, however, reflect an important opportunity for use of cell phone based text messaging to allow for bi-directional communication. A similar Project Health Design project looks specifically at design issues for interventions that seek to help teens with chronic illness transition from pediatric and adolescent care to adult health care. This project focuses heavily on leveraging existing behaviors among teens, for example text messaging, to facilitate the exchange of information on chronic illnesses.[34]

Finally, though there are some differences in priorities and needs, health improvement projects from the developing world do offer some evidence of "proof in concept" for text messaging based interventions to improve health. A project sponsored by "Compliance Service" a private company in Cape Town, South Africa, has demonstrated, by their own assessment, an effective method to encourage compliance with medication therapy for patients with tuberculosis using targeted SMS-based reminders. This project makes extensive use of open-source software applications and low

cost messaging services to address one of sub-Saharan Africa's most important public health concerns.[35]

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Health IT Use Among Providers Treating the Underserved

There are numerous models by which use of health IT by health care providers either on a community or institutional level can facilitate more effective and efficient delivery of care. Exhibit 2 below outlines some basic objectives of health care providers and demonstrates how health IT functionality can assist in meeting those goals.

Exhibit 2:

Health IT Functionality and Health Center Mission

Provider Objectives		Activities Enabled by Health IT
• Provide Access to the Uninsured • Deliver Evidence-based Care • Actively Manage Chronic Illnesses • Improve Patient Safety • Improve Care Coordination • Maximize 3rd Party Reimbursement • Report to Funding Agencies • Reduce Administrative Costs	Leads to	• Electronic referral to specialty care • Track eligibility for Medicaid • Track care delivered and outcomes • Generate reminders at point of care • Prescribe drugs electronically with built in formulary data and interaction warnings • Automate patient follow-up • Access to patient records online • Bill electronically • Generate custom reports

It should be noted that existing research on EHR adoption nationally shows relatively low rates of adoption, with estimates ranging from 4 percent for adoption of a fully functional EHR to 17 percent for any form of EHR using consensus based definitions.[36] A survey of EHR adoption among federally funded community health centers that treat predominantly low income individuals demonstrates that these providers are less likely than private physicians to have adopted EHRs.[37] The study did show, however, that a majority of federally qualified health centers planned on implementing EHRs in the near future.

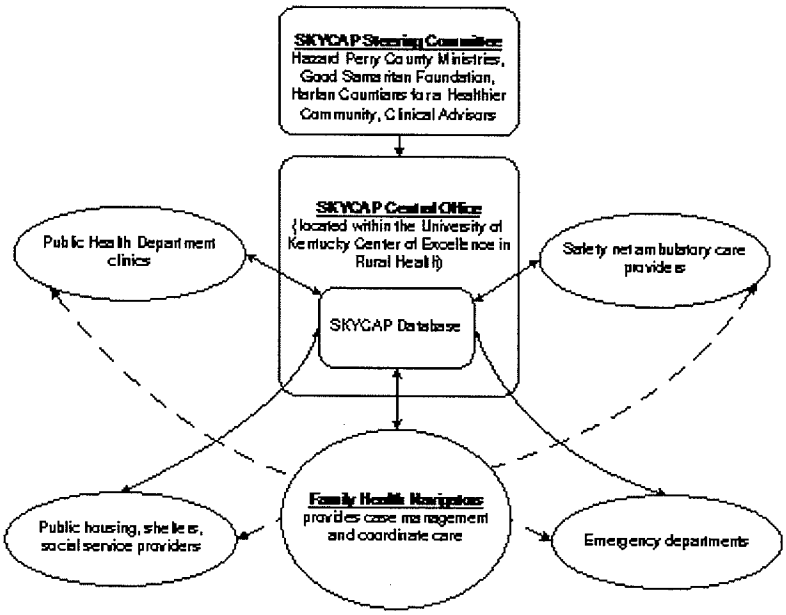
There are numerous examples of the adoption of health IT among health centers and health center networks and that these providers are committed to using patient registries to support quality improvement programs that are proven to improve processes and outcomes of care for patients with chronic illnesses such as diabetes[38]. While still in their early stages, health center networks funded under a series of grant programs sponsored by the Health Resources and Services Administration (HRSA) have demonstrated that collaboration and pooling of resources and expertise can lead to broader scale adoption of EHRs among safety net providers and, with it, the potential to improve the quality of care delivered to the underserved[39].

Another example of effective use of health IT among providers of the underserved is the Indian Health Services (IHS) which has led the way in addressing the needs of a very complex and underserved population. Because they are mandated by Congress to serve a very specific population

with a set of special health risks and needs, for decades the IHS has long used a population health approach to monitor and track the health and health care of their patients through a Resource and Patient Management System (RPMS). In 2007, IHS released a graphical interface designed to work with the RPMS as well as an application called iCare that allows for automated review and tagging of patient level information from RPMS to facilitate timely detection of risk factors and diagnosis of illnesses.[40] The interface along with RPMS and iCare represents a robust EHR with clinical decision support. IHS providers note that systems such as iCare that produce automatic alerts and suggest the likelihood that specific diagnoses and treatment approaches may be appropriate based on systematic, automated review of patient data can help maintain a high level of vigilance for opportunities to detect and prevent disease that disproportionately impact American Indian communities such as diabetes, childhood obesity and depression.

Finally, there are examples of community-based implementation of data warehouses and community tracking systems that seek to integrate patient-level data from providers caring for the underserved including information on demographics, clinical experience, health status and eligibility for public insurance programs in a system that is accessible to health care providers, case workers and other social service providers. These systems attempt to facilitate access to a range of social services. Several examples of these systems were initiated under the Healthy Communities Action Program (HCAP) and, while demonstrating some great potential, have proven difficult to sustain over time. Exhibit 3 below illustrates the model for one such program initiated in the state of Kentucky. The original program, known as SKYCAP originally, has subsequently changed its name to the "Kentucky Homeplace Program" after HCAP funding period ended. Kentucky Homeplace is currently funded by the Kentucky Department of Public Health and operates in Western, South Central, Southern, Southeast and Northeast Kentucky.

Exhibit 3: Overview of Kentucky Community Tracking database
Note: solid lines indicate data transfer; dotted lines indicate referral or other case management contact.



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Issues Facing Use of Health IT in the Underserved

The preceding discussion demonstrates that while the underserved and their providers are in very early stages of making use of health IT, there are a wide range of attractive opportunities and at least several good examples of effective use of health IT to improve the health and health care of the underserved. The discussion of specific examples also illustrates some of the challenges inherent in making the benefits of health IT available for the underserved. While stakeholders responsible for using health IT to improve care to the underserved must also contend with significant challenges associated with the design, adoption, implementation and use of health IT generally, we focus for the following pages on those issues and challenges which are specific to the context of addressing the needs of the underserved.

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Issues for Provider Facing Health IT

Providers who disproportionately care for the underserved often face different circumstances and barriers in effectively providing care. Some of these challenges reappear in implementing health IT among providers working with the underserved. For example, community health centers often work with patients who have complex mental and physical health needs.[41] The complexity of these needs results in a an integrated services approach to care where medical care, mental health care, dental care and case management may be provided by the same institution. While these providers can help to fully address the needs of the most underserved individuals, the provision of a more holistic set of services can make health IT implementations more difficult. Robert Miller and Christopher West explain: "This complexity increases EHR-related costs for CHCs, because it increases the complexity of CIS changes, staff training, and complementary process changes." Additionally, chronic resource constraints make the capital investments necessary for health IT a near impossibility.[42]

While many of the concerns listed above are specific to community health centers and others that disproportionately care for the underserved, many of the barriers to health IT adoption in these settings resemble those faced by providers generally. Issues of workflow redesign, change management and health information exchange appear similarly difficult among those who care for the underserved and other providers. This similarity suggests that wider efforts to address some of the difficulties in health IT adoption may prove effective in underserved environments.

Despite the similarity in barriers, many point to differences in provider adoption rates as a clear sign that progress needs to be made in underserved environments. Some of the key issues and challenges facing safety net providers seeking to implement health IT are elaborated below.

Financing health IT. As might be expected, the most common issue or challenge associated with health IT adoption for providers caring for the underserved is the lack of access to capital to make necessary investments in the start-up and maintenance costs associated with health IT adoption. Because many of these providers are publicly funded, non-profit institutions such as federally qualified health centers or publicly funded hospitals operating on a low or no profit basis, they are not in a position to make significant capital investments that will not result directly in increased revenue through expansion of their patient base or scope of services. Federally qualified health

centers also report feeling financially constrained due to limits on their ability to use grant funds meant predominantly to fund direct services to patients to finance health IT adoption that will enhance the overall quality and efficiency of the services they provide.

Vendor selection and customization. Given the diversity among individuals that could be considered among the underserved at any given time, providers seeking adoption of health IT for quality improvement must pay particular attention to the relationship between the health and cultural characteristics of their target populations and the features, functionalities and customization they will require from their health IT applications. For example, providers who treat a predominantly middle age to elderly African American population may want to assure that their EHR system supports diabetes registries and can transfer any registry data from legacy systems into the new application. In addition, providers treating the underserved often are funded from a variety of sources and are required to generate a variety of specified reports to those funders on a regular basis. Many EHRs have rudimentary applications for running custom reports, so it often takes additional resources and attention to assure that appropriate reports can be produced accurately and efficiently.

Finding the right strategy to empower patients. Given the importance of focusing on health IT as a means to the end of improving health and health care for underserved individuals, providers work to set up systems to best support a clinical workflow that provides the greatest opportunity of empowering patients to take an active role in the management of their own care. In the case of some underserved populations, this would require systems to prompt staff to conduct frequent reminders for patients who are due for specific clinical exams, vaccinations or diagnostic tests. How and when this prompting occurs (i.e., by phone, text message or email in the morning or the evening) may depend on circumstances (e.g., work hours, access to computers) of the individual patients as well as the hours of operation of the health center. Health centers that treat specific populations, e.g., homeless persons, day laborers, farm workers or low skilled or low waged employees may need to institute specific prompting strategies that reflect predominant characteristics among their patients. In addition, among some populations it is important that such prompts be directed to family members or surrogates in addition to the patients themselves.

Maintaining cultural competence and trust in a computerized environment. While there is limited information regarding the attitudes of patients generally with respect to health IT use among providers, some anticipate that patients will have concerns both related to the security and privacy of their health information if it is maintained and used in electronic form and in the potential erosion of some aspects of the doctor patient relationship if there is a computer mediating their interactions in the exam room[43]. There is also some evidence that racial and ethnic minorities, new immigrants and other groups that are disproportionately among the underserved have less trust in the health care system than the general population[44]. As such, it will be important for community leaders and public health officials to reach out to members of their patient community prior to health IT adoption to explain the benefits of health IT adoption and describe exactly how the care they receive and manner in which their health information is handled will or will not change. Furthermore, the extent to which providers refer to the computer during a clinical exam may be different for populations with limited English proficiency who may require more focused communication with the clinician to exchange critical information.

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Issues for Patient Facing Health IT

While there are some significant issues specific to provider adoption health IT to address the needs of the underserved, the issues for patient facing health IT applications are more complex. In part this is due to the fact that highly functional patient facing health IT applications such as PHRs and clinical messaging systems are so new and there are still many challenges associated with their adoption by the general population. It is also due to the vast diversity of personal and community characteristics included among the underserved and the difficulty of any one application to perfectly meet all of their needs. Finally, there are likely some hard limits to what patient facing health IT can accomplish in the face of structural barriers to improved health and health care for some segments of the underserved. These topics and others are elaborated below.

Language and health literacy. Several researchers and authors have noted the significant challenges associated with developing consumer-targeted materials on health and health care issues[45]. It is critical that these materials be comprehensive and accurate, while describing key concepts in terms that are digestible and well understood on the part of the intended audience. In some ways this information needs to be better understood than other materials that individuals may read, because they must understand the information well enough to feel comfortable taking direct action on the conclusions they draw. A Patient's health or quality of life may be directly affected by their ability to take the right action at the right time. Developing audience-appropriate guidance on health care is difficult and costs are compounded when one considers the need to develop materials in a series of different languages or when targeting populations with low reading proficiency in any language.

Technology literacy. As noted earlier in this paper, there is some evidence to suggest that some groups within the underserved population have limited access to computers and the Internet. Even as access to these technologies expands as it has dramatically in recent years, proficiency with use of these applications is highly variable in several key populations. For example, even individuals who are comfortable using the Internet to browse specific sites to obtain information may not feel comfortable enough to interact with others online or conduct financial transactions online. While training is an important component of rolling out any new application, in the case of patient facing health IT, training often need to comprehensively cover all aspects of successful use of the application, from the question of where and how one can access a computer with Internet access, to the basics of using a computer and Internet browser to view and submit information to more detailed training on the specific components and features of the application.

Fear and stigma attached to health problems. One potential barrier to adoption of patient-facing health IT applications and use of the health care system in general may be the fear of learning about and acknowledging health problems, and relatedly, the stigma associated with having particular health conditions in some communities[46]. In these communities motivation for adoption of health IT and the very act of learning about health status and risk factors may be limited.

Structural issues barriers. While patient facing health IT applications can help empower patients by arming them with information relevant to the appropriate care and treatment, this information could be of limited usefulness if structural barriers prevent them from acting effectively in a manner consistent with the information they receive. Structural barriers that can prevent the benefits of having good information at the right time would include a dearth of health care providers for an uninsured or publicly insured individual, as well as the lack of public parks, bike paths and grocery stores in some communities to facilitate healthful living. While being armed with appropriate information from health IT can be tremendously useful to the underserved, its usefulness may be severely limited if structural barriers to health and health care prevent appropriate action.

Effective strategies for adoption. Finally, given individuals' busy lives and the potential that health

and health care issues may not be the most important priority for some underserved families who are struggling for economic survival, it is clear that if patient facing health IT applications are to bring significant benefits to underserved populations, concerted effort must be put into place to make these tools widely and freely available, to educate underserved populations regarding the benefits of taking advantage of these tools, and to offer providers incentives to cooperate the establishment of integrated applications that allow patient and provider coordination on health and health care issues.

Our examples also demonstrate the desirability of coupling patient facing health IT applications with other services that are of immediate day to day use to underserved individuals. For example, one of the rationales behind adding the feature of maintaining a picture ID as part of MiVIA was to motivate individuals who may not otherwise be interested in signing up for a personal health care application to learn about and eventually begin using MiVIA.

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Conclusions

While there is more work to be done, there are several experiences and examples to date that suggest that health IT may be an integral part of broader solutions to address disparities in the health and well being of underserved Americans. There is broad acknowledgement among key stakeholders that moving towards a more digital environment where health data, information and knowledge is generated, captured and shared securely, efficiently and in a targeted manner (right information to the right person at the right time) is an important structural step in improving the nations health care delivery system and public health system. It is important however, as these efforts get 1underway, that appropriate attention is given to the particular needs of the most vulnerable Americans and the institutions that serve their needs to ensure that they are not left behind. We end with a set of conclusions for consideration by policy makers, payers and purchasers, providers and other health care stakeholders as the nation grapples with new approaches to address the health and health care needs of underserved Americans.

Health IT is a means to an end, not a magic bullet. It is important to recognize that health IT does not represent a magic bullet for improving health and health care to the underserved. As with use of health IT to improve quality of care in general, it is just an important component for facilitating these improvements and must be integrated into broader initiatives that focus on understanding and addressing root causes of these disparities, including structural barriers.

The promise is there, but structural and financial challenges persist. Work to date shows that attention to health IT use as it relates to improving health care and the health of the underserved can reap important rewards, in terms of access to care, quality and the patient-centeredness of health care. Federal, state and community based efforts have begun to show anecdotal evidence of improvement.

However, health IT generally, and patient facing health IT in particular, is still in the early stages of use among the underserved and the institutions serving them, and there has been limited formal study of its use and impact among underserved populations. One reason for the relative slowness of adoption relate to structural and financial challenges faced by this population and providers that serve them.

For example, most federally funded health centers and other safety net health care providers have

limited financial margins with which to pursue solutions based in health IT adoption and lack the time and staff necessary to pursue federal grants to initiate this work either individually or as part of a consortium. Similarly, many underserved families are faced with a myriad of daily challenges to assure their own immediate safety and financial health and are less likely to have time to access to online tools to help manage their health and health care and may be less able to use these tools effectively.

Evidence of a digital divide diminishing but still may be a factor. There is still a clear relationship between income and access to the Internet.[47] However, there is increasing evidence that the digital divide, especially as it relates to age, income and education is diminishing.[48] There is also evidence, that among, individuals who regularly use the use Internet, there is no correlation between key income or education and one's likelihood to communicate electronically with providers.[49] However, other studies do suggest that individuals who have less access to traditional health care are also less likely to use online tools to get or exchange information about their health. What is clear, is that even at the lowest income levels the majority of adults in the United States now have access to and use the Internet and that this represents an important opportunity for patient facing health IT.

Training and education are essential to achieve potential benefits. As the underserved as a whole begins to get better access to IT and online tools, the question will become how to translate better access to these resources to improvements in health and health care. Pairing technology initiatives with human support and training appears to be the most significant way to increase adoption and promote effective use over time. In many pilot programs, in-person assistance helped to address unexpected barriers and population-specific challenges. It appears that few if any have been able to develop purely automated approaches to culturally appropriate training and support, although some have tried to make educational resources more culturally relevant. In some cases, in-person training and assistance also appears to overcome the negative effects of lower computer ownership and internet usage rates. It remains to be seen whether similar results can be duplicated by building training and support into the technology itself.[50]

Personal computers are not the only mechanism for reaching the underserved. Lack of computer literacy may not be as large a problem as anticipated because of increased access to computers and the Internet among all families in the United States.[51] Still, stakeholders should take lessons learned from other parts of the world where SMS, text messaging, and use of cell phones have been employed to facilitate improvements in population health.[52]

Greater engagement between the community of providers, case workers and social workers serving the underserved and the health IT industry is needed. Community-based systems that facilitate sharing of individual level information across health care and social service providers offer the most promise for being able to address structural and multi-factorial barriers to health and health care improvement. However, these are difficult to sustain because of the need for centralized coordination among distinct entities with limited financial incentive to coordinate and integrate.

Underserved communities are different from other communities and from each other. Evolving EHR and PHR certification efforts may be able to help address potential disconnects between the design of health IT applications and the needs of communities, safety net providers and the underserved themselves by offering special certificates to applications that meet requirements around treating patients with limited English proficiency or who may have special needs with respect to culturally competent care. However, it is also important to note that the underserved

represent an array of different segments of our population and that health IT based interventions or approaches that work among one segment of the underserved will not work for all other segments. Understanding differences across segments of the underserved is particularly important for designing patient facing technologies that can be effectively used by a particular community for improving behaviors associated with better health.

Additional research is needed. Existing evaluation data focus on specific interventions and their impact on specific populations. More evaluation is needed on this level. In addition, more research is needed to systematically review and synthesize these studies and to draw broader conclusions regarding the potential impact of health IT on the underserved. Finally, it may be advisable for funders to work closely with researchers to identify consistent domains and measures for evaluation of the impact of health IT on the underserved to allow for meta-analyses or more robust syntheses across distinct evaluation efforts over time.

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The Underserved and Health Information Technology- Issues and Opportunities

Adil Moiduddin
Jonathan Moore

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Introduction and Objectives

NORC at the University of Chicago is pleased to present this white paper entitled "The Underserved and Health Information Technology: Issues and Opportunities" for the Assistant Secretary for Planning and Evaluation (ASPE) at the U.S. Department of Health and Human Services (HHS). Due to recent advances in technology and greater attention to problems associated with quality and efficiency of health care delivery, we see new opportunities to improve the health and health care for underserved Americans through the use of emerging information technologies.

President George W. Bush announced an Executive Order in 2004 prioritizing the adoption and use of health information technology (health IT) by patients and providers as well as the use of secure health information exchange (HIE) to improve the quality, safety, effectiveness and efficiency of health care delivery in the United States, and creating the Office of the National Coordinator for Health IT (ONC). Several agencies within HHS including the Health Resources and Services Administration (HRSA), the Indian Health Service (IHS), the Office of Minority Health (OMH), the Agency for Healthcare Research and Quality (AHRQ), the Centers for Medicare and Medicaid Services (CMS), the Office of Disease Prevention and Health Promotion (ODPHP), ASPE and others had been working on programs and policies to assure that the nation's most vulnerable Americans are not left behind as the health care sector moves to adopt a more automated information driven approach to promoting health and preventing and treating illness. More recently several States have joined in these activities by sponsoring their own programs to encourage health IT adoption.

This purpose of this paper is to summarize a selected set of programs, policies and research findings that demonstrate both the potential for health IT to improve health and health care to underserved Americans as well as the challenges and barriers facing effective use of these technologies. We will attempt to cover an array of technologies including electronic health records (EHRs), e-Prescribing (eRx), chronic disease registries and clinical decision support systems (CDS) by health care providers predominantly serving the underserved. Additionally, we will examine technologies such as personal health records (PHRs), messaging and reminder systems, patient kiosks and other technologies that are "patient facing" where the theory is that IT can empower patients to take more control over their own health information and health care.

Finally, we will provide summary conclusions regarding what is known and yet to be understood regarding use of health IT among the underserved and highlight areas where further programmatic, policy or research activities sponsored by the federal government or others may be important. We begin with a brief discussion on health and health care challenges facing underserved Americans.

Why Focus on the Underserved?

While there is no single, universally accepted definition of the underserved, for the purpose of this paper, we characterize this population simply as those living in the United States who do not have adequate access to health care services. They share one or more of these characteristics: they may be poor; uninsured; have limited English language proficiency and/or lack familiarity with the health care delivery system; or live in locations where providers are not readily available to meet their needs. Members of ethnic and racial minority groups are not by definition "underserved", but are disproportionately found among their numbers. Disparities in health status and access to health care that leave these populations worse off relative to others have been well documented by HHS over the last fifteen years.

Of the diverse set of groups represented among the underserved, perhaps disparities in health and health care of racial and ethnic minorities have been most thoroughly documented. Recently the Commonwealth Fund released a chart book analyzing data on the status of racial and ethnic minorities in the United States (available at http://www.commonwealthfund.org/publications). This effort compliments the primary federal government publication on health disparities, the "National Healthcare Disparities Report", released annually by AHRQ with the last release for 2007 occurring in February of this year (available at http://www.ahrq.gov/qual/qrdr07.htm).[1] Findings from both publications demonstrate persistent disparities in health status, access to health insurance, access to critical health care services such as a primary care, home and specialty care, and in the quality of care received by disadvantaged groups.[2] The Commonwealth summary shows that as of 2006, data from surveys conducted by federal agencies including AHRQ, the National Center for Health Statistics (NCHS) of the Centers

for Disease Control and Prevention (CDC) and the National Institutes of Health (NIH) demonstrate higher rates of self-reported poor health status, chronic disease and disability, life expectancy at birth, obesity, cancer (especially breast and colorectal), heart disease, diabetes, HIV/AIDS and other chronic illnesses among blacks compared to non-Hispanic whites in the United States. The chartbook also demonstrates specific types of behavioral and health status disparities for other racial and ethnic groups such as higher smoking prevalence and mental distress among American Indian and Alaska Native populations, and higher incidence of some infection-related cancers among Hispanics.

In terms of access to health care, the Commonwealth chart book shows greater percentages of Hispanic adults and adults of other racial and ethnic minorities reporting they have no doctor compared to whites, with the Hispanic average for this question being 2.5 times that of whites. They also show that blacks and Hispanics are less likely to report private physicians as their usual source of care relative to whites and are more likely to report going to community health centers and emergency rooms as their usual source of care or having no usual source of care. Hispanics also are much more likely than whites to report being uninsured during a given year. Some of these disparities are heavily mediated by income and are reduced or eliminated by controlling for this characteristic, suggesting the importance of viewing the low income population broadly as part of the underserved. However, many of these disparities, particularly as they relate to health status among blacks and access and insurance disparities issues among Hispanics persist even after adjusting for income.

Finally, the data on racial and ethnic disparities also demonstrates important differences in the quality of care delivered to ethnic and racial minorities. Primary care physicians who treat predominantly black patients report being unable to provide high quality care to all of their patients at a higher rate than similar physicians who treat a predominantly white population. Hispanics and Asians, on average, report wait times longer than others to get a doctor's appointment. Hispanics also report being less likely to have received recommended screenings such as blood cholesterol and cancer screenings (colorectal and cervical) relative to others. Racial and ethnic minorities in general are less likely to have received a pneumococcal vaccination, a dental visit in the past year (for children), and first trimester prenatal care (women) than the equivalent non minority populations.[3]

Racial and ethnic minorities aside, evidence from research shows that low-income populations generally experience more disjointed care, being twice as likely to lack a regular source of care.[4] Those with low socioeconomic status (SES) have limited access to quality health care as it relates to primary, specialty, dental and behavioral care relative to others.[5] Additionally, lower SES patients experience lower rates of preventive care and chronic disease management.[6] [7] We found no definitive evidence that rural residents, as a group, experience lower quality care; however, low population density in rural areas makes care less convenient and more costly. Additionally, rural populations are more likely to be uninsured and of low income, making these areas more susceptible to the barriers faced by low-income populations.[8]

The AHRQ National Healthcare Disparities report cited above focuses heavily on disparities in measures that address quality of care. AHRQ and the Inter-Agency HHS work group that contributes to this report provide data on 211 measures including 41 core measures on quality and access. Comparisons are produced across various demographic groups including blacks compared to whites, Asians compared to whites, Hispanics compared to non-Hispanics, poor compared to high income and American Indian and Alaskan Native compared to whites. Findings from the latest version of this report for 2007 show some reduction in previously identified disparities facing segments of the underserved for measures such as adequacy of hemodialysis treatment and childhood vaccination rates for blacks versus whites. However, the findings also demonstrate the persistence of disparities and little or no improvement over time in the disparities associated with many measures since the start of this initiative.[9]

Given findings from the research and analysis presented above, as well as a wealth of additional evidence suggesting similar trends of poor health and access to quality health care among groups comprising the underserved, improving care to this group represents an important challenge for policy makers and program leaders in federal and State government. Some have speculated that increased automation in health care and use of advanced IT could exacerbate rather than address these disparities if a "digital divide" threatens to leave underserved populations behind, resulting in a situation where only those who have historically enjoyed steady health insurance and access to private medical providers would benefit from these advances. At the same time, many of the most promising potential improvements in care due to health IT adoption including clinical decision support to alert providers to the need for increasing use of screenings, preventive care, and behavioral counseling

and patient registries that facilitate active management of chronic illness, could be of disproportionate benefit to the underserved. Finally, as the use of technologies such as PHRs serve to empower patients to take a more active role in their health care and overall well-being, it is increasingly important to recognize and address barriers to making the benefits of these technologies readily available to underserved populations who stand to benefit the most.

What Role Can Health IT Play?

Researchers have struggled to definitively characterize all the factors associated with disparities in health and health care for the underserved and assign appropriate importance to each factor. There is, however, a consensus regarding the significance of these problems and a solid interest among program and policy leaders to understand the manner in which advances in public health, health care delivery and health care financing can work to address these disparities. While it is clear that these disparities are a result of a complex network of factors including personal, family, cultural, neighborhood and economic variables in addition to those associated with the direct provision of necessary health care services, many have identified health IT as a means to facilitate behavioral and organizational changes to improve the health and health care of the underserved.

For the purpose of this paper we define health IT as technology that enables patients and providers to support better health and health care by providing targeted information meant to inform, educate or generally allow for improved decision making. In some cases, the information provided by these technologies is traditionally accessed by other means such as paper patient charts, evaluations and clinical summaries transferred via fax or by hand between providers or even clinical flow sheets that outline appropriate care for specific clinical situations based on accepted clinical guidelines.

Exhibit 1: Health IT Applications [10]

Product or Functionality	Description
Electronic Health Record (EHR)	An electronic record of health-related information on an individual that conforms to nationally recognized interoperability standards and that can be created, managed, and consulted by authorized clinicians and staff across more than one health care organization.
Electronic Medical Record (EMR)	An electronic record of health-related information on an individual that can be created, gathered, managed, and consulted by authorized clinicians and staff within one health care organization.
e-Prescribing (eRx)	Enables a physician to transmit a prescription electronically to the patient's choice of pharmacy. It also enables physicians and pharmacies to obtain information about the patient's eligibility and medication history from drug plans. Often comes with built in alerts for drug-drug, drug-allergy and drug-disease interactions.
Personal Health Records	An electronic record of health-related information on an individual that conforms to nationally recognized interoperability standards and that can be drawn from multiple sources while being managed, shared, and controlled by the individual.
Computerized Physician Order Entry (CPOE)	Refers to a computer-based system of ordering medications and often other tests. Physicians directly enter orders into a computer system that can have varying levels of sophistication. Basic CPOE ensures standardized, legible, complete orders, and thus primarily reduces errors due to poor handwriting and ambiguous abbreviations.
Clinical Decision Support (CDS)	Any system designed to improve clinical decision making related to diagnostic or therapeutic processes of care. CDS addresses activities ranging from the selection of drugs (e.g., the optimal antibiotic choice given specific microbiologic data) or diagnostic tests to detailed support for optimal drug dosing and support for resolving diagnostic dilemmas. Often incorporated as part of CPOE or EMR/EHR systems.
Disease Registries	A database feature that includes key clinical data on a subset of chronically ill patients for the purpose of tracking their condition and managing treatment.

In other cases, health IT enables providers and patients to access information that they otherwise would not be able to access. Exhibit 1 above outlines some basic definitions of health IT applications. Implemented properly, these technologies can create tremendous efficiencies and improvements in health and health care by providing "the right information to the right person at the right time". This results in benefits such as greater adherence to evidenced based guidelines for screenings and vaccinations, better patient compliance with treatment instructions, reductions in medication errors and improved patient education.

As noted above, specific technologies that are most commonly referred to as health IT include electronic health records (EHRs) and personal health records (PHRs), the former being the systems used by providers to electronically record and maintain patient demographic information and information on activities, diagnoses and orders associated with a clinical episode, and the latter being an application for use by patients to access and update information related to their own health and health care or that of a family member or loved one. One key distinction between EHRs and PHRs lies in defining the locus of usage and control. EHRs are controlled and used primarily by the provider whereas PHRs are used and controlled by patients and their surrogates.

Additional applications include functionalities that may be considered add-ons to these core concepts, including clinical decision support (CDS) which in the context of an EHR would provide patient specific information on an appropriate course of treatment based on clinical effectiveness research; issue alerts if an order entered by the clinician is counter-indicated based on the patient's profile; or provide reminders regarding the need to order specific interventions such as screenings, vaccinations, blood tests or foot exams.

In addition to PHRs, other health IT applications aimed at patients include health kiosks, where patients could obtain information on health conditions or access to information on their own health and health care using publicly available computer terminals set up within the community. These may also include messaging systems that allow transmission of reminders, information to guide healthful behavior, or even direct communication with providers through email or short message service (SMS) messaging accessed via cell phones or personal digital assistants (PDAs). In a 2006 report entitled, "Expanding the Reach and Impact of Consumer e-Health Tools", the Office of Disease Prevention and Health Promotion at HHS identified a series of functions that might be considered some of the key potential benefits to patients using health IT. These functions included provision of health information to patients in a searchable format to help with researching treatment options; support for behavior modification and self management of a healthy lifestyle; access to online communities for interacting with others on health issues; and functions that allow joint management and tracking of treatment involving online collaboration between patients and clinicians.[11]

Another use of health IT involves health information exchange (HIE) which refers to the electronic exchange of data on a particular patient in a secure format between relevant administrative and clinical stakeholders such as other clinicians, payers and patients themselves. HIE can enable more efficient exchange of data between different types of providers (e.g., ambulatory and acute care providers or between primary care and specialty care) or between providers and patients (EHRs to PHRs). Some HIE functionality can also be built into PHRs and EHRs to enable specific services such as electronic prescribing (eRx) and refilling of prescription medications, ordering tests, receiving results from clinical laboratories, and maintaining ready access to radiology reports and results from tests conducted in an inpatient environment.

A central premise of our paper is that health IT applications such as the ones described in Exhibit 1 represent potentially effective mechanisms for achieving basic goals associated with improving health, health care and access to care for the underserved. As has been often noted, there is no evidence to suggest that health IT adoption in and of itself will transform the health or health care of the underserved. However, if based in broader initiatives for increasing enrollment in public programs, improving quality of care, empowering patients through improved access to information and streamlining and simplifying health care delivery, health IT may be a critical ingredient to achieving important changes.

In particular, because all IT and systems initiatives are designed around managing information in a standardized and efficient manner, these technologies can play an important role in assuring that the right information, in the right format is available to the right person and the right time to improve family and provider decision making, improve access to care and better target resources. Over time, health IT applications may lead to a richer set of data from which population health care trends can be assessed, thereby contributing to the development of better knowledge on the causes of disparities affecting health and health care for the underserved, getting a better sense of the barriers to improving their status relative to those disparities and contributing to programmatic and policy initiatives informed by a richer set of data than are currently available.

In the remainder of this paper we describe in greater detail opportunities and challenges associated with the use of health IT to improve health and health care of the underserved. For the sake of simplicity, in some places we organize this discussion between those technologies that can be considered primarily provider facing, such as EHRs, and those technologies that are primarily patient facing, such as PHRs. We note however that this is a

somewhat artificial distinction as effective approaches to improving health may need to take advantage of technologies that enable direct electronic communication between providers and patients through direct messaging and are, in that sense, both provider and patient facing. We end the paper with summary conclusions for different health care sector stakeholders and address areas that merit further exploration through research and evaluation.

Current State of Health IT and the Underserved

Although there is little population-based research on the use of health IT by the underserved and those that treat them specifically, there is some evidence that the groups disproportionately represented among the underserved and those that treat them are currently less likely to use health IT compared to other groups. For example, in the paragraphs below we cite recent research that shows that lower income Americans, some ethnic and racial minorities as well as residents in rural areas are less likely to have broadband access and are less likely to use the Internet than others, that individuals who lack steady access to health care are less likely to go online to get health related information and that safety net health centers that disproportionately serve the poor and uninsured are less likely than other providers to use electronic health records. [12] [13] [14] [15]

On the other hand, other research suggests that the income and education related factors are not as important in predicting Internet use as it has been in the past and that among individuals who use the Internet regularly, there is no difference by income, age or gender in the propensity to go online for communicating with providers or seeking information on health and health care.[16] There is also evidence that many safety net health care providers have adopted health IT through the support of federal grants and are leaders in ambulatory health IT use. It is important to note however that adoption and access to health IT does not automatically translate to improvements in quality of care or health status.

Most of the research conducted to date has been limited in scope, looking at the potential for specific health IT interventions to improve limited aspects of quality of care for minorities or other groups represented in the underserved populations. While there has been some survey work conducted examining the use of health IT among health care providers that treat the underserved, there is almost no information on the use of patient-facing health IT applications by underserved individuals and families in the United States. This is predominantly because the extent to which PHRs are used by any group within the United States is largely unknown but considered to be limited due to the novel nature of these technologies, lack of knowledge of them among the public, and limited availability of PHRs sponsored by traditional sources of health care information for patients: providers, payers and purchasers. There are currently efforts underway sponsored by AHRQ and others to elucidate these issues.[17]

Direct Use of Health IT by Underserved Populations

As noted above, there is no research that looks at underserved populations as a whole and assesses use of patient-facing health IT applications such as PHRs, health kiosks or SMS-based messaging to generate evidence on the prevalence of use or attitudes towards these technologies among underserved Americans. However, there are data that can be used as proxies to assess the extent to which these technologies are accessible to the underserved and the likelihood that they will be adopted in the near future. These proxies include evidence regarding access to and use of information technology and communications technologies by groups represented among the underserved generally and in the context of health in particular. We explore available evidence on these questions below.

Some differences persist between underserved populations and the broader population with regard to internet usage and online health information seeking behavior. While current estimates of Internet usage suggest that more than half of individuals in all income and racial and ethnic groups have access to the Internet and use it on a consistent basis, there is still evidence that some racial and ethnic minorities as well as lower income individuals use the Internet in lower proportions compared to the general population.[18] Additionally, urban and suburban residents are more likely than rural residents to use the Internet regularly, with 77 percent of urban and suburban residents reporting usage compared with 64 percent of rural residents in 2006.[19] Thus, it is reasonable to conclude that the underserved are less likely than others to have access to the Internet and, if they have access, use it on a less frequent basis than others.

There is also evidence that those with a longer history of Internet use and who visit the doctor regularly are more likely than others to go online to access information on health. [20] Again, this trend suggests that the underserved, may be less likely than others to use the Internet as a tool for improving their health or health care as they are

disproportionately "new" users of the Internet. However, it is important to note that recent research by both the Pew Internet and the American Life Project and the National Cancer Institutes National Health Information Trends Survey (HINTS) suggests that, among the group of individuals who are Internet users, income and education are not associated with use of the Internet for communicating with providers or seeking health related information.[21] [22]

Because the model for most patient-facing health IT applications involves use of the Internet as a major conduit for accessing, exchanging and maintaining information relevant to the health and health care of a an individual or family, many of these technologies may be less useful for underserved Americans compared to other groups.[23] While this may signal the importance of increasing general Internet access and usage among the underserved population, there is also evidence that Internet usage alone may not lead to adoption of patient-facing health IT applications among the underserved.[24] As such, developers of patient-facing health IT are increasingly looking to applications built on other platforms to meet the same objectives of giving patients access to the "right information at the right time" to make good decisions regarding their health. For example, there is increased attention to the potential of building health interventions around SMS or "text messages" via cell phones and PDAs. These interventions are meant to support maintenance of a healthy lifestyle by initiating reminders and alerts to patients or sharing test results and instructions with patients and there is some evidence, particularly from the developing world, that cell phones represent a cost effective medium for transferring critical health and health care messages to an underserved population.[25] [26]

Even though patient facing health IT applications are in early stages of adoption and usage, there are many targeted applications that have been developed, implemented and even evaluated on a small scale. In the ODPHP report referenced above, the authors identified and reviewed 40 separate "consumer e-Health tools" ranging from informational websites to PHRs with a vast array of functions. Selected examples of patient-facing health IT that are specifically targeted to underserved populations are reviewed below along with evidence regarding their success.

PHRs for migrant workers. [27] MiVIA, a PHR designed for migrant, Spanish-speaking populations in California, is web-based tool that allows for documentation of clinical visits, health conditions, allergies, medications and other information critical to maintaining continuity of care. Importantly, the tool also provides its target audience with other valuable services including a picture ID, a stable email address, access to Medline and provider websites and emergency ID information and is set up to allow access not only to the individual patient but also family and surrogates.

Finally, there is a provider view where clinicians are allowed to populate, view and update data on patients they are treating based on a specific episode of care. While we found no published independent evaluation of MiVIA, anecdotal information and internally conducted assessments suggest that a significant percentage of individuals signed up for and have accounts with MiVIA use it regularly. Additionally, clinicians report that use of MiVIA has dramatically improved their ability to provide effective health care to this highly mobile population.

Implementers of MiVIA note the importance of not simply making the tool available and publicizing it, but providing comprehensive education and support to assist individuals with every facet of using the application effectively. This included providing basic training on computer and Internet usage, providing information regarding locations where clients could access computers with Internet connections, as well as extensive repetitive training on the use of the application itself. Support and training are provided by "promoters de salud" or community health workers who are imbedded among the migrant workers and are able to conduct the training in a culturally appropriate manner using language that is familiar to clients. While MiVIA started as a local collaborative effort between labor and health care providers in Sonoma County California, it has expanded in recent years to other migrant farm communities on the West Coast and is being touted as a an optimal PHR solution for homeless individuals, children and others.

Online education and support systems for cancer patients.[28] [29] While MiVIA is an example of a relatively broad based PHR that is targeted to a specific demographic population regardless of their individual health status or needs, many patient facing health IT applications focus on providing targeted functionality to individuals who have similar health and health care characteristics. One such example is the Comprehensive Health Enhancement Support System (CHESS) developed by the University of Wisconsin and the Cancer Information Service (CIS) which is part of the National Institutes of Health's (NIH), National Cancer Institute (NCI). CHESS is an online

system that provides users with 11 services designed to improve quality of life for women diagnosed with breast cancer.

These include what are referred to as "information services" such as a static "Q&A" section; a library of reference articles on breast cancer topics; and resource guides on topics such as selecting providers, resource directories and links to other useful sites. In addition, the system offers "support services" allowing users to ask direct questions to clinical experts and gain emotional support by viewing text and video accounts from other cancer patients discussing how they coped with the disease. And, finally, it provides "decision services" that allow users to take emotional status assessments and receive tailored advice on coping, and use online health charts to track their health status. These health charts also direct users to information on their own specific health concerns and decision aids that help identify options, assign values, and elucidate potential consequences associated with key treatment and lifestyle decisions.

Recently published literature on the system demonstrates that CHESS is effective in improving social support, comfort with their doctors and the care they are receiving, information competence and quality of life among women with cancer. Research also suggests that CHESS is particularly effective on these measures for women considered "disadvantaged" based on income and insurance status because these individuals are the ones that are most likely to lack any resources in the absence of having access to a system such as CHESS.

In a subsequent study, researchers assessed the relative effectiveness of different approaches to disseminating and encouraging take up of CHESS among underserved women and found that while referrals from hospitals and doctors were effective in encouraging underserved women to make use of CHESS, different approaches were important for different communities. For example, they found that publicizing CHESS through radio advertisements was more effective among black women compared to others. They also demonstrated the benefits of extensive in person training for underserved women, not only on navigating and using CHESS, but more basic skills around use of computers and the Internet.

Using health kiosks.[30] [31] Standalone health kiosks offer some of the same functionality of PHRs and online support systems, but combine computer hardware and software in a single unit. This combination helps to address issues surrounding computer and internet access among some underserved groups. Additionally, physically placing kiosks in targeted locations (such as physician offices or health centers) could allow trained health assistants to assist users when needed. Hardware and software specifications also allow kiosk designers to limit internet and data access to a greater degree than would be possible in a home web-based system. In one pilot implementation among Australian aboriginals, health kiosks served as a first step toward greater health IT familiarity. The kiosks helped to address the connectivity and hardware issues facing those with the most limited experience with and access to new technologies.

Closer to home, a project initiated by the Duke University Medical Center tested the potential impact of kiosks to address the needs for undeserved residents in North Carolina[32]. Duke created a cluster of nine health kiosks in three counties in the state. DERICKs (Durham e-Health Resource Information Center Kiosks) are located in community health centers, medical centers, local departments of social service and emergency departments. The kiosks are used to help patients identify and overcome personal barriers to accessing care (e.g. transportation issues may lead the kiosk to recommend contacting a local transportation agency). To that end, the kiosk asks a series of preliminary questions to gauge users' language preferences, education and literacy levels and levels of computer literacy. The responses to these questions help to tailor the questions related to access later in the kiosk application. All kiosk text is available in both English and Spanish and videos are available to walk users through all steps in the process. Users are also able to print out pamphlets via a built-in printer. These pamphlets document specific resources and provide customized guidance to help overcome barriers to better health. To date, the average DERICK user prints out three pamphlets.

While anyone can use DERICKs, they offer additional functionality for Medicaid beneficiaries. DERICK allows users to enter their Medicaid ID numbers. DERICK documents beneficiaries' barriers and transmits them electronically to their assigned case managers. This provides another point of access to the social service safety net for beneficiaries who may not have time to update their case managers after every ED or primary care visit. Data gathered through kiosks are transmitted through a local HIE, COACH (Community-Oriented Approach to Coordinated Healthcare). This connectivity allows other local providers to access information gathered via

DERICK. While specific details remain unclear, Medicaid beneficiaries may be able to access medical records using kiosks in the future.

Others in the field have expressed greater skepticism toward the use of health kiosks. While customization for targeted populations is a clear benefit for underserved groups, little has been done to clarify what such changes would look like. Additionally, some research indicates that any benefit to the low running costs of health kiosks would be negated as health kiosk user satisfaction generally wanes over time. High initial costs suggest that health kiosks could amount to an expensive novelty for underserved communities.

Use of text messaging system. One emerging set of interventions aimed at improving the health of the undeserved through use of health IT takes advantage of wide adoption of cell phones that use SMS technologies to enable text messaging of health content directly to specific targeted populations. One of the several innovative projects initiated by the Robert Wood Johnson Foundation (RWJF) as part of their Project Health Design PHR initiative takes advantage of the fact that some populations making up the underserved are more likely to have cell phones than regular access to personal computers or laptops. For this initiative, RWJF developed the prototype for a system that would allow patients with heart disease to report on their status on a daily basis and then receive tailored information regarding recommended therapies and behaviors for that day.[33]

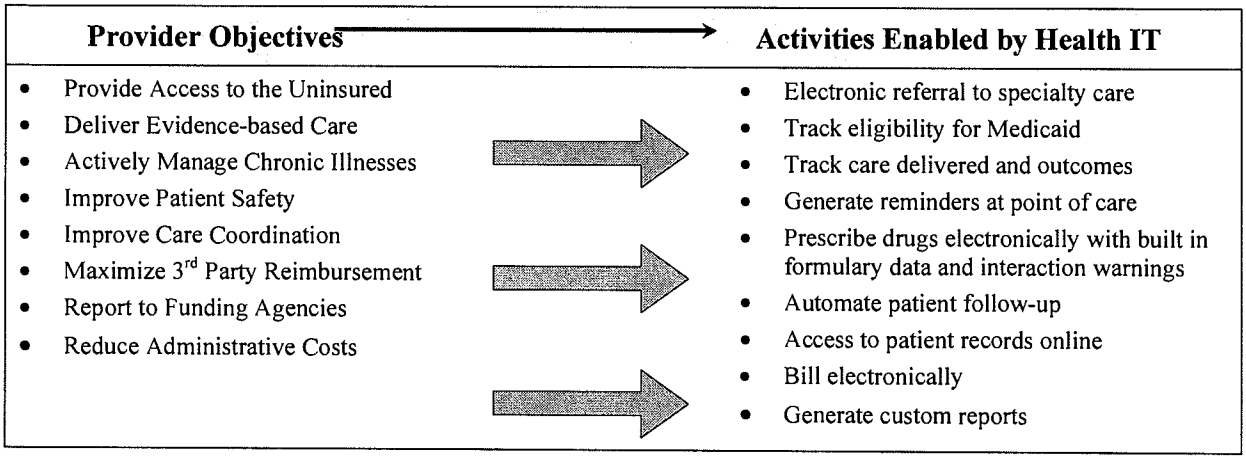
This project based at the University of Rochester aims at developing a prototype portable digital assistant with more functionality than the vast majority of cell phones (e.g., sophisticated speech recognition). It does, however, reflect an important opportunity for use of cell phone based text messaging to allow for bi-directional communication. A similar Project Health Design project looks specifically at design issues for interventions that seek to help teens with chronic illness transition from pediatric and adolescent care to adult health care. This project focuses heavily on leveraging existing behaviors among teens, for example text messaging, to facilitate the exchange of information on chronic illnesses.[34]

Finally, though there are some differences in priorities and needs, health improvement projects from the developing world do offer some evidence of "proof in concept" for text messaging based interventions to improve health. A project sponsored by "Compliance Service" a private company in Cape Town, South Africa, has demonstrated, by their own assessment, an effective method to encourage compliance with medication therapy for patients with tuberculosis using targeted SMS-based reminders. This project makes extensive use of open-source software applications and low cost messaging services to address one of sub-Saharan Africa's most important public health concerns.[35]

Health IT Use Among Providers Treating the Underserved

There are numerous models by which use of health IT by health care providers either on a community or institutional level can facilitate more effective and efficient delivery of care. Exhibit 2 below outlines some basic objectives of health care providers and demonstrates how health IT functionality can assist in meeting those goals.

Exhibit 2: Health IT Functionality and Health Center Mission



It should be noted that existing research on EHR adoption nationally shows relatively low rates of adoption, with estimates ranging from 4 percent for adoption of a fully functional EHR to 17 percent for any form of EHR using consensus based definitions.[36] A survey of EHR adoption among federally funded community health centers that treat predominantly low income individuals demonstrates that these providers are less likely than private physicians to have adopted EHRs.[37] The study did show, however, that a majority of federally qualified health centers planned on implementing EHRs in the near future.

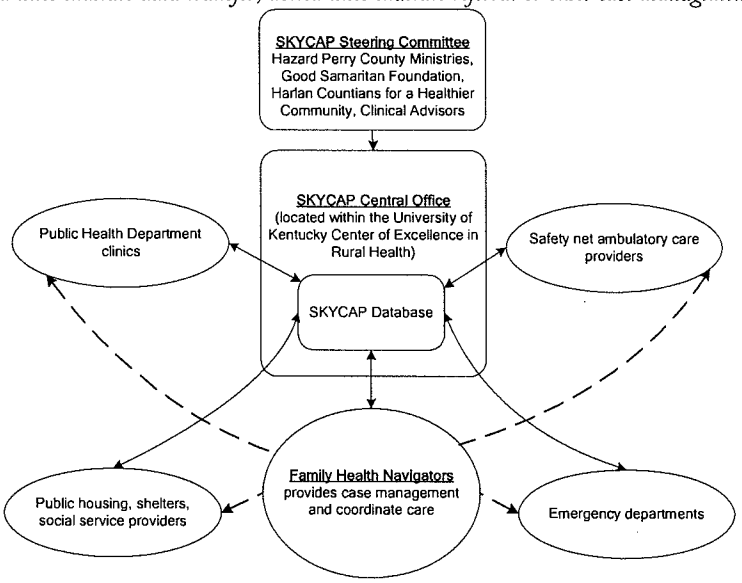
There are numerous examples of the adoption of health IT among health centers and health center networks and that these providers are committed to using patient registries to support quality improvement programs that are proven to improve processes and outcomes of care for patients with chronic illnesses such as diabetes[38]. While still in their early stages, health center networks funded under a series of grant programs sponsored by the Health Resources and Services Administration (HRSA) have demonstrated that collaboration and pooling of resources and expertise can lead to broader scale adoption of EHRs among safety net providers and, with it, the potential to improve the quality of care delivered to the underserved[39].

Another example of effective use of health IT among providers of the underserved is the Indian Health Services (IHS) which has led the way in addressing the needs of a very complex and underserved population. Because they are mandated by Congress to serve a very specific population with a set of special health risks and needs, for decades the IHS has long used a population health approach to monitor and track the health and health care of their patients through a Resource and Patient Management System (RPMS). In 2007, IHS released a graphical interface designed to work with the RPMS as well as an application called iCare that allows for automated review and tagging of patient level information from RPMS to facilitate timely detection of risk factors and diagnosis of illnesses.[40] The interface along with RPMS and iCare represents a robust EHR with clinical decision support. IHS providers note that systems such as iCare that produce automatic alerts and suggest the likelihood that specific diagnoses and treatment approaches may be appropriate based on systematic, automated review of patient data can help maintain a high level of vigilance for opportunities to detect and prevent disease that disproportionately impact American Indian communities such as diabetes, childhood obesity and depression.

Finally, there are examples of community-based implementation of data warehouses and community tracking systems that seek to integrate patient-level data from providers caring for the underserved including information on demographics, clinical experience, health status and eligibility for public insurance programs in a system that is accessible to health care providers, case workers and other social service providers. These systems attempt to facilitate access to a range of social services. Several examples of these systems were initiated under the Healthy Communities Action Program (HCAP) and, while demonstrating some great potential, have proven difficult to sustain over time. Exhibit 3 below illustrates the model for one such program initiated in the state of Kentucky. The original program, known as SKYCAP originally, has subsequently changed its name to the "Kentucky Homeplace Program" after HCAP funding period ended. Kentucky Homeplace is currently funded by the Kentucky Department of Public Health and operates in Western, South Central, Southern, Southeast and Northeast Kentucky.

Exhibit 3: Overview of Kentucky Community Tracking database

Note: solid lines indicate data transfer; dotted lines indicate referral or other case management contact.



Issues Facing Use of Health IT in the Underserved

The preceding discussion demonstrates that while the underserved and their providers are in very early stages of making use of health IT, there are a wide range of attractive opportunities and at least several good examples of effective use of health IT to improve the health and health care of the underserved. The discussion of specific examples also illustrates some of the challenges inherent in making the benefits of health IT available for the underserved. While stakeholders responsible for using health IT to improve care to the underserved must also contend with significant challenges associated with the design, adoption, implementation and use of health IT generally, we focus for the following pages on those issues and challenges which are specific to the context of addressing the needs of the underserved.

Issues for Provider Facing Health IT

Providers who disproportionately care for the underserved often face different circumstances and barriers in effectively providing care. Some of these challenges reappear in implementing health IT among providers working with the underserved. For example, community health centers often work with patients who have complex mental and physical health needs.[41] The complexity of these needs results in a an integrated services approach to care where medical care, mental health care, dental care and case management may be provided by the same institution. While these providers can help to fully address the needs of the most underserved individuals, the provision of a more holistic set of services can make health IT implementations more difficult. Robert Miller and Christopher West explain: "This complexity increases EHR-related costs for CHCs, because it increases the complexity of CIS changes, staff training, and complementary process changes." Additionally, chronic resource constraints make the capital investments necessary for health IT a near impossibility.[42]

While many of the concerns listed above are specific to community health centers and others that disproportionately care for the underserved, many of the barriers to health IT adoption in these settings resemble those faced by providers generally. Issues of workflow redesign, change management and health information exchange appear similarly difficult among those who care for the underserved and other providers. This similarity suggests that wider efforts to address some of the difficulties in health IT adoption may prove effective in underserved environments.

Despite the similarity in barriers, many point to differences in provider adoption rates as a clear sign that progress needs to be made in underserved environments. Some of the key issues and challenges facing safety net providers seeking to implement health IT are elaborated below.

Financing health IT. As might be expected, the most common issue or challenge associated with health IT adoption for providers caring for the underserved is the lack of access to capital to make necessary investments in the start-up and maintenance costs associated with health IT adoption. Because many of these providers are publicly funded, non-profit institutions such as federally qualified health centers or publicly funded hospitals operating on a low or no profit basis, they are not in a position to make significant capital investments that will not result directly in increased revenue through expansion of their patient base or scope of services. Federally qualified health centers also report feeling financially constrained due to limits on their ability to use grant funds meant predominantly to fund direct services to patients to finance health IT adoption that will enhance the overall quality and efficiency of the services they provide.

Vendor selection and customization. Given the diversity among individuals that could be considered among the underserved at any given time, providers seeking adoption of health IT for quality improvement must pay particular attention to the relationship between the health and cultural characteristics of their target populations and the features, functionalities and customization they will require from their health IT applications. For example, providers who treat a predominantly middle age to elderly African American population may want to assure that their EHR system supports diabetes registries and can transfer any registry data from legacy systems into the new application. In addition, providers treating the underserved often are funded from a variety of sources and are required to generate a variety of specified reports to those funders on a regular basis. Many EHRs have rudimentary applications for running custom reports, so it often takes additional resources and attention to assure that appropriate reports can be produced accurately and efficiently.

Finding the right strategy to empower patients. Given the importance of focusing on health IT as a means to the end of improving health and health care for underserved individuals, providers work to set up systems to best support a clinical workflow that provides the greatest opportunity of empowering patients to take an active role in the management of their own care. In the case of some underserved populations, this would require systems to prompt staff to conduct frequent reminders for patients who are due for specific clinical exams, vaccinations or diagnostic tests. How and when this prompting occurs (i.e., by phone, text message or email in the morning or the evening) may depend on circumstances (e.g., work hours, access to computers) of the individual patients as well as the hours of operation of the health center. Health centers that treat specific populations, e.g., homeless persons, day laborers, farm workers or low skilled or low waged employees may need to institute specific prompting strategies that reflect predominant characteristics among their patients. In addition, among some populations it is important that such prompts be directed to family members or surrogates in addition to the patients themselves.

Maintaining cultural competence and trust in a computerized environment. While there is limited information regarding the attitudes of patients generally with respect to health IT use among providers, some anticipate that patients will have concerns both related to the security and privacy of their health information if it is maintained and used in electronic form and in the potential erosion of some aspects of the doctor patient relationship if there is a computer mediating their interactions in the exam room[43]. There is also some evidence that racial and ethnic minorities, new immigrants and other groups that are disproportionately among the underserved have less trust in the health care system than the general population[44]. As such, it will be important for community leaders and public health officials to reach out to members of their patient community prior to health IT adoption to explain the benefits of health IT adoption and describe exactly how the care they receive and manner in which their health information is handled will or will not change. Furthermore, the extent to which providers refer to the computer during a clinical exam may be different for populations with limited English proficiency who may require more focused communication with the clinician to exchange critical information.

Issues for Patient Facing Health IT

While there are some significant issues specific to provider adoption health IT to address the needs of the underserved, the issues for patient facing health IT applications are more complex. In part this is due to the fact that highly functional patient facing health IT applications such as PHRs and clinical messaging systems are so new and there are still many challenges associated with their adoption by the general population. It is also due to the vast diversity of personal and community characteristics included among the underserved and the difficulty of any one application to perfectly meet all of their needs. Finally, there are likely some hard limits to what patient facing health IT can accomplish in the face of structural barriers to improved health and health care for some segments of the underserved. These topics and others are elaborated below.

Language and health literacy. Several researchers and authors have noted the significant challenges associated with developing consumer-targeted materials on health and health care issues[45]. It is critical that these materials be comprehensive and accurate, while describing key concepts in terms that are digestible and well understood on the part of the intended audience. In some ways this information needs to be better understood than other materials that individuals may read, because they must understand the information well enough to feel comfortable taking direct action on the conclusions they draw. A Patient's health or quality of life may be directly affected by their ability to take the right action at the right time. Developing audience-appropriate guidance on health care is difficult and costs are compounded when one considers the need to develop materials in a series of different languages or when targeting populations with low reading proficiency in any language.

Technology literacy. As noted earlier in this paper, there is some evidence to suggest that some groups within the underserved population have limited access to computers and the Internet. Even as access to these technologies expands as it has dramatically in recent years, proficiency with use of these applications is highly variable in several key populations. For example, even individuals who are comfortable using the Internet to browse specific sites to obtain information may not feel comfortable enough to interact with others online or conduct financial transactions online. While training is an important component of rolling out any new application, in the case of patient facing health IT, training often need to comprehensively cover all aspects of successful use of the application, from the question of where and how one can access a computer with Internet access, to the basics of using a computer and Internet browser to view and submit information to more detailed training on the specific components and features of the application.

Fear and stigma attached to health problems. One potential barrier to adoption of patient-facing health IT applications and use of the health care system in general may be the fear of learning about and acknowledging health problems, and relatedly, the stigma associated with having particular health conditions in some communities[46]. In these communities motivation for adoption of health IT and the very act of learning about health status and risk factors may be limited.

Structural issues barriers. While patient facing health IT applications can help empower patients by arming them with information relevant to the appropriate care and treatment, this information could be of limited usefulness if structural barriers prevent them from acting effectively in a manner consistent with the information they receive. Structural barriers that can prevent the benefits of having good information at the right time would include a dearth of health care providers for an uninsured or publicly insured individual, as well as the lack of public parks, bike paths and grocery stores in some communities to facilitate healthful living. While being armed with appropriate information from health IT can be tremendously useful to the underserved, its usefulness may be severely limited if structural barriers to health and health care prevent appropriate action.

Effective strategies for adoption. Finally, given individuals' busy lives and the potential that health and health care issues may not be the most important priority for some underserved families who are struggling for economic survival, it is clear that if patient facing health IT applications are to bring significant benefits to underserved populations, concerted effort must be put into place to make these tools widely and freely available, to educate underserved populations regarding the benefits of taking advantage of these tools, and to offer providers incentives to cooperate the establishment of integrated applications that allow patient and provider coordination on health and health care issues.

Our examples also demonstrate the desirability of coupling patient facing health IT applications with other services that are of immediate day to day use to underserved individuals. For example, one of the rationales behind adding the feature of maintaining a picture ID as part of MiVIA was to motivate individuals who may not otherwise be interested in signing up for a personal health care application to learn about and eventually begin using MiVIA.

Conclusions

While there is more work to be done, there are several experiences and examples to date that suggest that health IT may be an integral part of broader solutions to address disparities in the health and well being of underserved Americans. There is broad acknowledgement among key stakeholders that moving towards a more digital environment where health data, information and knowledge is generated, captured and shared securely, efficiently and in a targeted manner (right information to the right person at the right time) is an important structural step in improving the nations health care delivery system and public health system. It is important however, as these efforts

get lunderway, that appropriate attention is given to the particular needs of the most vulnerable Americans and the institutions that serve their needs to ensure that they are not left behind. We end with a set of conclusions for consideration by policy makers, payers and purchasers, providers and other health care stakeholders as the nation grapples with new approaches to address the health and health care needs of underserved Americans.

Health IT is a means to an end, not a magic bullet. It is important to recognize that health IT does not represent a magic bullet for improving health and health care to the underserved. As with use of health IT to improve quality of care in general, it is just an important component for facilitating these improvements and must be integrated into broader initiatives that focus on understanding and addressing root causes of these disparities, including structural barriers.

The promise is there, but structural and financial challenges persist. Work to date shows that attention to health IT use as it relates to improving health care and the health of the underserved can reap important rewards, in terms of access to care, quality and the patient-centeredness of health care. Federal, state and community based efforts have begun to show anecdotal evidence of improvement.

However, health IT generally, and patient facing health IT in particular, is still in the early stages of use among the underserved and the institutions serving them, and there has been limited formal study of its use and impact among underserved populations. One reason for the relative slowness of adoption relate to structural and financial challenges faced by this population and providers that serve them.

For example, most federally funded health centers and other safety net health care providers have limited financial margins with which to pursue solutions based in health IT adoption and lack the time and staff necessary to pursue federal grants to initiate this work either individually or as part of a consortium. Similarly, many underserved families are faced with a myriad of daily challenges to assure their own immediate safety and financial health and are less likely to have time to access to online tools to help manage their health and health care and may be less able to use these tools effectively.

Evidence of a digital divide diminishing but still may be a factor. There is still a clear relationship between income and access to the Internet.[47] However, there is increasing evidence that the digital divide, especially as it relates to age, income and education is diminishing.[48] There is also evidence, that among, individuals who regularly use the use Internet, there is no correlation between key income or education and one's likelihood to communicate electronically with providers.[49] However, other studies do suggest that individuals who have less access to traditional health care are also less likely to use online tools to get or exchange information about their health. What is clear, is that even at the lowest income levels the majority of adults in the United States now have access to and use the Internet and that this represents an important opportunity for patient facing health IT.

Training and education are essential to achieve potential benefits. As the underserved as a whole begins to get better access to IT and online tools, the question will become how to translate better access to these resources to improvements in health and health care. Pairing technology initiatives with human support and training appears to be the most significant way to increase adoption and promote effective use over time. In many pilot programs, in-person assistance helped to address unexpected barriers and population-specific challenges. It appears that few if any have been able to develop purely automated approaches to culturally appropriate training and support, although some have tried to make educational resources more culturally relevant. In some cases, in-person training and assistance also appears to overcome the negative effects of lower computer ownership and internet usage rates. It remains to be seen whether similar results can be duplicated by building training and support into the technology itself.[50]

Personal computers are not the only mechanism for reaching the underserved. Lack of computer literacy may not be as large a problem as anticipated because of increased access to computers and the Internet among all families in the United States.[51] Still, stakeholders should take lessons learned from other parts of the world where SMS, text messaging, and use of cell phones have been employed to facilitate improvements in population health.[52]

Greater engagement between the community of providers, case workers and social workers serving the underserved and the health IT industry is needed. Community-based systems that facilitate sharing of individual level information across health care and social service providers offer the most promise for being able to address structural and multi-factorial barriers to health and health care improvement. However, these are difficult to sustain

because of the need for centralized coordination among distinct entities with limited financial incentive to coordinate and integrate.

Underserved communities are different from other communities and from each other. Evolving EHR and PHR certification efforts may be able to help address potential disconnects between the design of health IT applications and the needs of communities, safety net providers and the underserved themselves by offering special certificates to applications that meet requirements around treating patients with limited English proficiency or who may have special needs with respect to culturally competent care. However, it is also important to note that the underserved represent an array of different segments of our population and that health IT based interventions or approaches that work among one segment of the underserved will not work for all other segments. Understanding differences across segments of the underserved is particularly important for designing patient facing technologies that can be effectively used by a particular community for improving behaviors associated with better health.

Additional research is needed. Existing evaluation data focus on specific interventions and their impact on specific populations. More evaluation is needed on this level. In addition, more research is needed to systematically review and synthesize these studies and to draw broader conclusions regarding the potential impact of health IT on the underserved. Finally, it may be advisable for funders to work closely with researchers to identify consistent domains and measures for evaluation of the impact of health IT on the underserved to allow for meta-analyses or more robust syntheses across distinct evaluation efforts over time.

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Federal Coordinating Council for Comparative Effectiveness Research Agenda
April 10, 2009
1:30-3:00pm
Room 425-A
Call-in Number: 866-762-7985 passcode: 6866388

1. Comparative Effectiveness Research Background and Draft Initial Framework

2. Comparative Effectiveness Research Current Portfolios and Future Plans for CER

    a. AHRQ
    b. NIH
    c. VA

3. Listening Session Format

4. Other

AHRQ Comparative Effectiveness Research: Current and Future

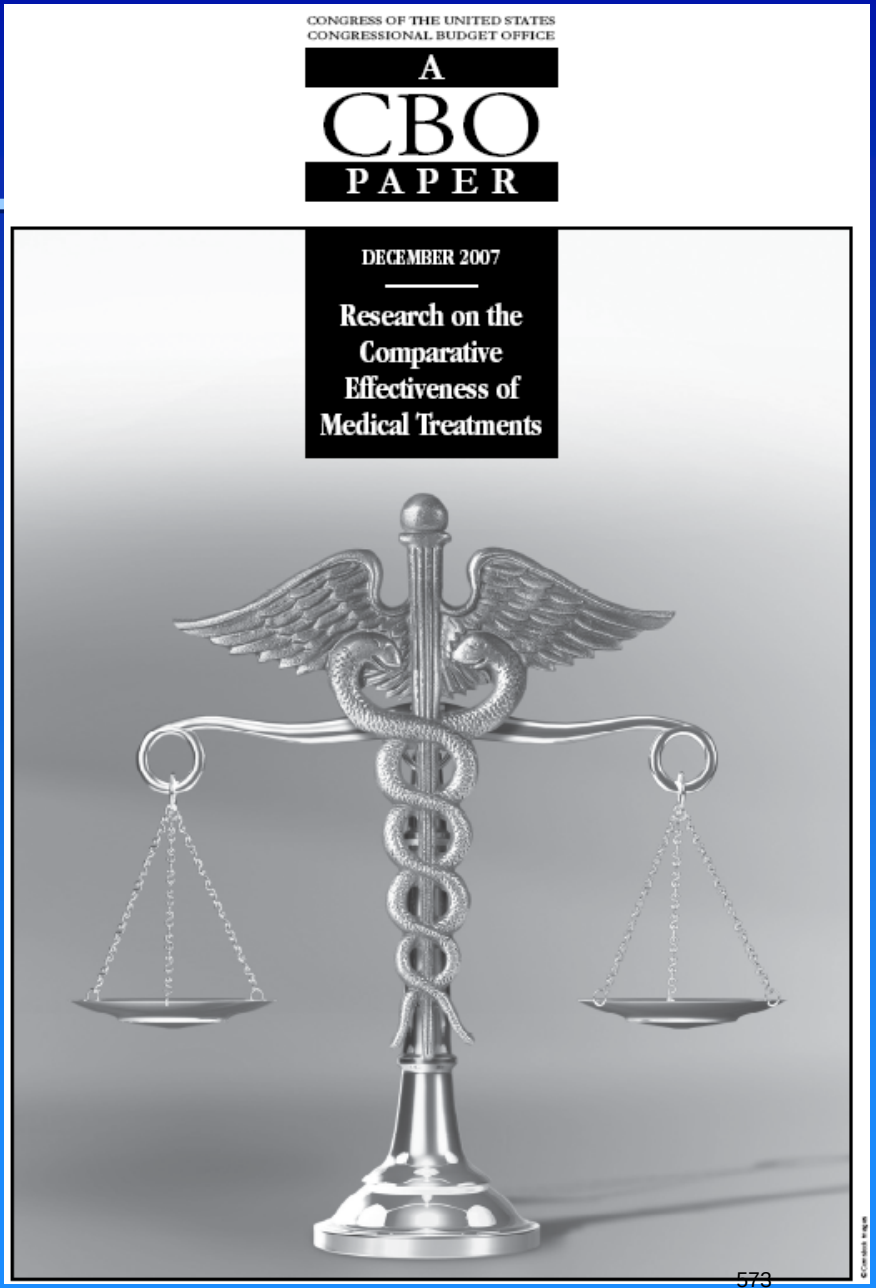
Federal Coordinating Council Meeting

April 10, 2009

Comparative Effectiveness

"…a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients."

CBO, 2007



Building on Previous Work

Research activities will be performed using rigorous scientific methods within a previously-established process that emphasizes stakeholder involvement and transparency, that was designed to prioritize among pressing health issues, and whose products are designed for maximum usefulness for health care decision makers.

Comparative Effectiveness Research at AHRQ

- Created in 2005, authorized by Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003
- AHRQ shall conduct and support research on:
  - "the outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs)"
- Goal: to provide patients, clinicians and policy makers with reliable, evidence-based healthcare information

AHRQ's CER Role under ARRA

- AHRQ to continue its CER work under the auspices of section 1013 of MMA
- AHRQ will continue to have a collaborative, open, and transparent process for comparative effectiveness that allows for input from all perspectives.
- AHRQ will continue to involve all stakeholders in the research process.
- To determine what priorities and projects will be funded under this new authority, we are undertaking a process to determine what will be funded, working with the Office of the Secretary, NIH, and IOM. We also will be seeking external input as AHRQ has done in the past.

Effective Health Care Program

- **To improve the quality, effectiveness, and efficiency of health care delivered through Medicare, Medicaid, and S-CHIP programs**
  - Focus is on what is known ***now***: ensuring programs benefit from ***past*** investments in research and what research ***gaps*** are critical to fill
  - Focus is on ***clinical effectiveness***

Priority Conditions for the Effective Health Care Program

- Arthritis and non-traumatic joint disorders
- Cancer
- Cardiovascular disease, including stroke and hypertension
- Dementia, including Alzheimer Disease
- Depression and other mental health disorders
- Developmental delays, attention-deficit hyperactivity disorder and autism
- Diabetes Mellitus
- Functional limitations and disability
- Infectious diseases including HIV/AIDS
- Obesity
- Peptic ulcer disease and dyspepsia
- Pregnancy including pre-term birth
- Pulmonary disease/Asthma
- Substance abuse

http//:effectivehealthcare.ahrq.gov

Comparative Effectiveness Reviews: Coming soon…

- Comparative Effectiveness Reviews
  - Stable Ischemic Heart Disease (draft report)
  - Particle Beam Therapies for Cancer (Technical Brief)
  - Stereotactic Radiosurgery (Technical Brief)
  - Heart Valve Replacement (Technical Brief)
  - Core NeedleBreast Biopsy
  - Lipid-modifying Agents
  - Radiofrequency Catheter Ablation for Atrial Fibrillation
  - Chemotherapy Agents in the Prevention of Breast Cancer
  - 9 Comparative Effectiveness Review Updates

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LATE-BREAKING CLINICAL TRIAL

Clinical Effectiveness of Coronary Stents in Elderly Persons

Results From 262,700 Medicare Patients in the American College of Cardiology-National Cardiovascular Data Registry

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Durham, North Carolina; Rockville, Maryland; Oakland, California; and Washington, DC

Objectives The aim of this study was to compare outcomes in older individuals receiving drug-eluting (DES) and bare-metal stents (BMS).

Background Comparative effectiveness of DES relative to BMS remains unclear.

Methods Outcomes were evaluated in 262,700 patients from 650 National Cardiovascular Data Registry sites during 2004 to 2006 with procedural registry data linked to Medicare claims for follow-up. Outcomes including death, myocardial infarction (MI), revascularization, major bleeding, stroke, death or MI, death or MI or revascularization, and death or MI or stroke were compared with estimated cumulative incidence rates with inverse probability weighted (IPW) estimators and Cox proportional hazards ratios.

Results The DES were implanted in 217,675 patients and BMS were implanted in 45,025. At 30 months, DES patients had lower unadjusted rates of death (12.9% vs. 17.9%), MI (7.3 of 100 patients vs. 10.0 of 100 patients), and revascularization (23.0 of 100 patients vs. 24.5 of 100 patients) with no difference in stroke or bleeding. After adjustment, DES patients had lower rates of death (13.5% vs. 16.5%, hazard ratio [HR]: 0.75, 95% confidence interval [CI]: 0.72 to 0.79, $p < 0.001$) and MI (7.5 of 100 patients vs. 8.9 of 100 patients, HR: 0.77, 95% CI: 0.72 to 0.81, $p < 0.001$), with minimal difference in revascularization (23.5 of 100 patients vs. 23.4 of 100 patients; HR: 0.91, 95% CI: 0.87 to 0.96), stroke (3.1 of 100 patients vs. 2.7 of 100 patients, HR: 0.97, 95% CI: 0.88 to 1.07), or bleeding (3.4 of 100 patients vs. 3.6 of 100 patients, HR: 0.91, 95% CI: 0.84 to 1.00). The DES survival benefit was observed in all subgroups analyzed and persisted throughout 30 months of follow-up.

Conclusions In this largest ever real-world study, patients receiving DES had significantly better clinical outcomes than their BMS counterparts, without an associated increase in bleeding or stroke, throughout 30 months of follow-up and across all pre-specified subgroups. (J Am Coll Cardiol 2009;53:000–000) © 2009 by the American College of Cardiology Foundation

The dramatic reductions in restenosis and repeat revascularization associated with coronary artery drug-eluting stents (DES) compared with their bare-metal stent (BMS) counterparts (1) prompted swift adoption into clinical practice (2). However, reports of late stent thrombosis (3,4) and higher mortality (5,6) resulted in release of 2 special Food

From the *Duke Clinical Research Institute, Duke University Medical Center, Durham, North Carolina; †Agency for Healthcare Quality and Research, Rockville, Maryland; ‡Kaiser Permanente, Oakland, California; and the §American College of Cardiology, Washington, DC. This project was sponsored by the Agency for Healthcare Research and Quality, U.S. Department of Health and Human Services, Rockville, Maryland, as part of the Cardiovascular Consortium and funded under Project ID: 24-EHC-1 and Work Assignment Number: HHSAA290-2005-0032-TO4-WA1 as part of the Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) program. The authors of this report are responsible for its content. Statements in the report should not be construed as endorsement by the Agency for Healthcare Research and Quality or the U.S. Department of Health and Human Services. Additional support was obtained from the National Cardiovascular Data Registry, American College of Cardiology, Washington, DC. Dr. Anstrom has received research support from AstraZeneca, Bristol-Myers Squibb, Eli Lilly and Co., and Medtronic. Dr. Anstrom has served as a consultant for Johnson & Johnson and Pfizer. Dr. Eisenstein has received research support from Medtronic Vascular and Eli Lilly and Co. Dr. Curtis has received research support from Allergan, Eli Lilly and Co., GlaxoSmithKline, Medtronic, Merck & Co., Johnson & Johnson (Ortho Biotech), Novartis, OSI Eyetech, and Sanofi-Aventis. Dr. Peterson has received research support from BMS/Sanofi and Merck/Schering.

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Clopidogrel Use and Long-term Clinical Outcomes After Drug-Eluting Stent Implantation

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Context Recent studies of drug-eluting intracoronary stents suggest that current antiplatelet regimens may not be sufficient to prevent late stent thrombosis.

Objective To assess the association between clopidogrel use and long-term clinical outcomes of patients receiving drug-eluting stents (DES) and bare-metal stents (BMS) for treatment of coronary artery disease.

Design, Setting, and Patients An observational study examining consecutive patients receiving intracoronary stents at Duke Heart Center, a tertiary care medical center in Durham, NC, between January 1, 2000, and July 31, 2005, with follow-up contact at 6, 12, and 24 months through September 7, 2006. Study population included 4666 patients undergoing initial percutaneous coronary intervention with BMS (n=3165) or DES (n=1501). Landmark analyses were performed among patients who were event-free (no death, myocardial infarction [MI], or revascularization) at 6- and 12-month follow-up. At these points, patients were divided into 4 groups based on stent type and self-reported clopidogrel use: DES with clopidogrel, DES without clopidogrel, BMS with clopidogrel, and BMS without clopidogrel.

Main Outcome Measures Death, nonfatal MI, and the composite of death or MI at 24-month follow-up.

Results Among patients with DES who were event-free at 6 months (637 with and 579 without clopidogrel), clopidogrel use was a significant predictor of lower adjusted rates of death (2.0% with vs 5.3% without; difference, −3.3%; 95% CI, −6.3% to −0.3%; $P=.03$) and death or MI (3.1% vs 7.2%; difference, −4.1%; 95% CI, −7.6% to −0.6%; $P=.02$) at 24 months. However, among patients with BMS (417 with and 1976 without clopidogrel), there were no differences in death (3.7% vs 4.5%; difference, −0.7%; 95% CI, −2.9% to 1.4%; $P=.50$) and death or MI (5.5% vs 6.0%; difference, −0.5%; 95% CI, −3.2% to 2.2%; $P=.70$). Among patients with DES who were event-free at 12 months (252 with and 276 without clopidogrel), clopidogrel use continued to predict lower rates of death (0% vs 3.5%; difference, −3.5%; 95% CI, −5.9% to −1.1%; $P=.004$) and death or MI (0% vs 4.5%; difference, −4.5%; 95% CI, −7.1% to −1.9%; $P<.001$) at 24 months. However, among patients with BMS (346 with and 1644 without clopidogrel), there continued to be no differences in death (3.3% vs 2.7%; difference, 0.6%; 95% CI, −1.5% to 2.8%; $P=.57$) and death or MI (4.7% vs 3.6%; difference, 1.0%; 95% CI, −1.6% to 3.6%; $P=.44$).

Conclusions The extended use of clopidogrel in patients with DES may be associated with a reduced risk for death and death or MI. However, the appropriate duration for clopidogrel administration can only be determined within the context of a large-scale randomized clinical trial.

JAMA. 2007;297:(doi:10.1001/jama.297.2.joc60179) www.jama.com

THE INCIDENCE OF EARLY VESSEL closure after coronary stent implantation was markedly reduced by the adoption of thienopyridine antiplatelet therapy.[1] The widespread adoption of dual antiplatelet therapy (aspirin and thienopyridines) has further reduced the risk of subacute thrombosis after bare-metal stent implantation to 0.5% to 1.9%.[1-3]

Instructions for the use of drug-eluting stents commercially available in the United States specify treatment with clopidogrel for at least 3 months (for sirolimus-coated stents) or 6 months (for paclitaxel-coated stents) after implantation. Premature discontinuation of this minimum antiplatelet therapy has been associated with stent thrombosis.[4,5] However, studies of late thrombosis events among patients with a drug-eluting stent have cast doubt on whether the recommended regimens are sufficient.[6,7] An observational analysis from BASKET-LATE (Basel Stent Kosten-Effektivitats Trial-Late Throm-

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CME Metrics Report

Comparative Effectiveness of Second-Generation Antidepressants in the Pharmacologic Treatment of Adult Depression: AHRQ Executive Summary

A Medscape Clinical Update

Agency for Healthcare Research and Quality

Supported by an educational grant from:
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November 29, 2007

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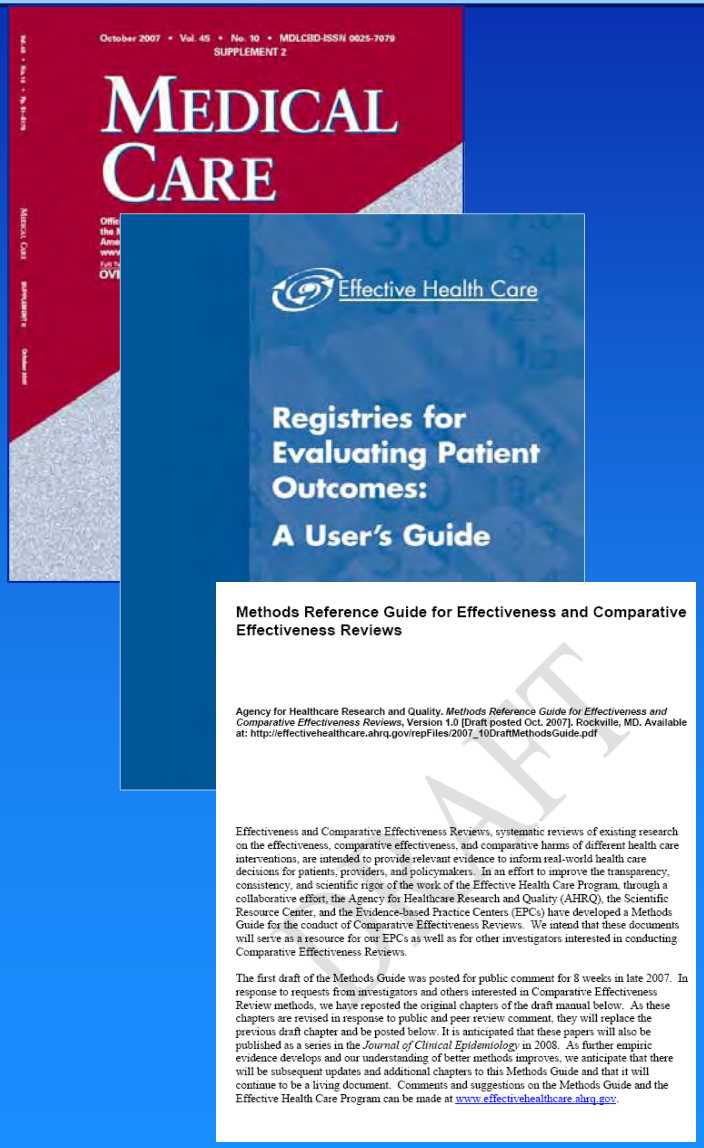
DARTNet: Distributed Ambulatory Research in Therapeutics Network

- Federated network of electronic health data from 9 physician organizations (over 500 clinicians and over 400,000 patients)
- Objectives of pilot study:
  - Create DARTNet and validate data and system integrity
  - Evaluate comparative effectiveness, safety and utilization of oral hypoglycemics:
- Assess factors affecting scale up (size and complexity) and sustainability of DARTNet

HMORN Distributed Research Network Prototype

- Developing the architecture for a multi-purpose, multi-institutional, distributed health data network.
- Intended to support secure data analyses on data that remains in the possession of the original data holder.
- Research study involves three large, integrated healthcare delivery systems that collectively care for over 4 million people.
  - Assessing the comparative effectiveness of two commonly used *second-line* antihypertensive agents: angiotensin-converting enzyme (ACE) inhibitors and beta-blockers.

Emerging Methods in Comparative Effectiveness & Safety



- Variation in methods among systematic reviews undercuts transparency
- Methods reduce the likelihood of scientific impartiality
- Methods help minimize misclassification of data
- Methods must continue to evolve and not remain stagnant
- AHRQ has and will continue to make investments in improving methods, esp. in understanding how treatments effect individuals differently, often referred to as “heterogeneity of treatment effects.”

Symposium on CER Research Methods

- June 1- 2, 2009 symposium will examine new & emerging methods for conducting comparative effectiveness research.
- The two main emphases:
  - Enhance the inclusion of clinically heterogeneous populations in comparative and clinical effectiveness studies.
  - Implement longitudinal investigations that capture longer term health outcomes, including patient-reported outcomes.
- 22 author presentations, with the proceedings published in peer-reviewed, open-access, journal supplement.
- Presentations will be concurrently broadcast on Internet using webinar format; invitations will be extended to each NAC member.

Plain Language Guides

Fast Facts

- ACEIs and ARBs are two of the many kinds of blood pressure pills.
- Both kinds of pills (ACEIs and ARBs) do a good job of lowering blood pressure.
- ACEIs and ARBs rarely cause serious problems. The main difference in side effects is that ACEIs are more likely than ARBs to cause a dry cough.
- ACEIs and ARBs do not affect cholesterol levels or blood sugar levels.
- Some ACEIs are available as generics, which cost less.

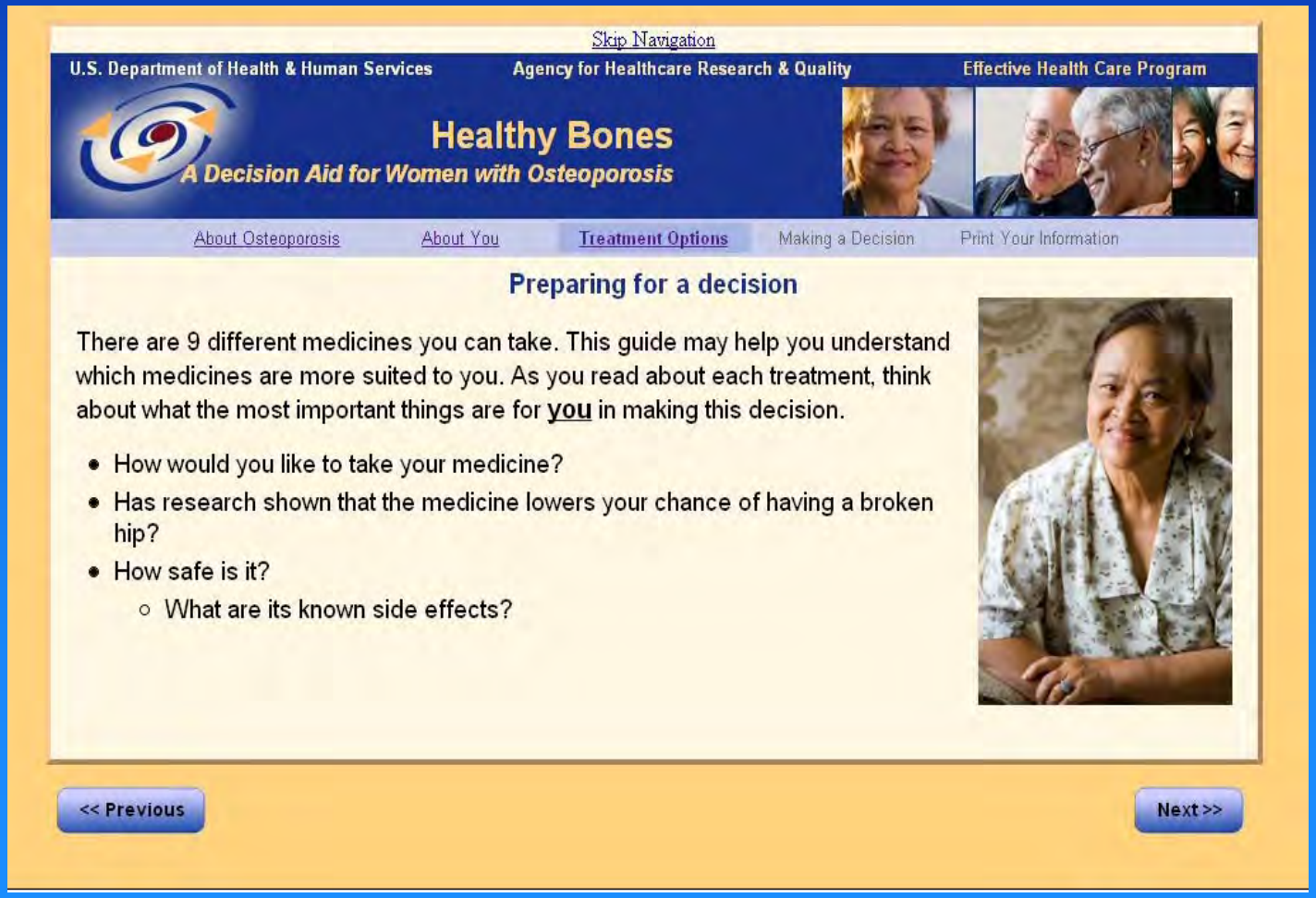
Coming soon…

- Summary guides
  - Osteoarthritis of the Knee (consumer and clinician)
  - Gestational diabetes (consumer and clinician)
  - Induction of labor (consumer and clinician)
  - Particle beam (policymaker)

- Spanish translations of 6 summary guides

Coming soon… (cont.)

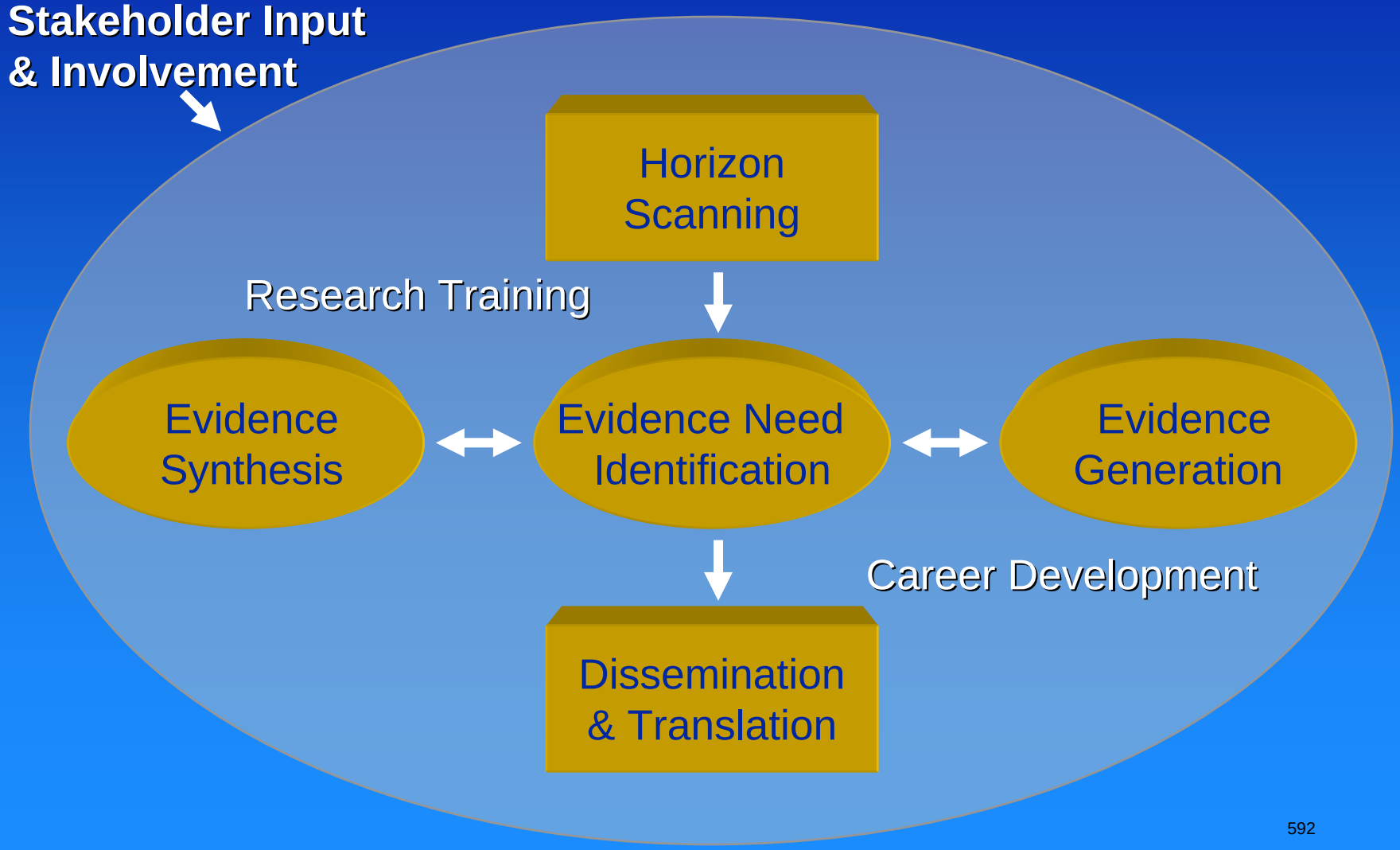
Interactive Decision Aids



New Resources – New Opportunities!

- Expanded infrastructure and capacity for Comparative Effectiveness Research
- Prospective studies that include under-represented populations
- Pushing forward on methods for Comparative Effectiveness Research (June 1-2 Symposium)
- Increasing investments in innovative broad dissemination and translation

Conceptual Framework



Horizon Scanning

- **Identification of New and Emerging Issues for Comparative Effectiveness (Horizon Scanning)**
  - identification of current or emerging interventions available to diagnose, treat, or otherwise manage a particular condition
  - vital for understanding the relevant healthcare context and landscape, as a basis for identifying and beginning to prioritize among research needs

Evidence Syntheses

- The review and synthesis of current medical research, to provide rigorous evaluation of what is known on the basis of existing research about the comparative effectiveness of alternative approaches to the given clinical problem
  - Includes methods needed to do comparative syntheses
  - Modeling and decision analytics
  - Vital for gap identification (next slide)

Evidence Gap Identification

- Systematic approach to identify areas where new research conducted within a comparative effectiveness framework would contribute to bridging the gap between existing medical research and clinical practice
  - Emphasis on identifying “key” gaps that will fill important areas for decision makers
    - Focus on under-represented populations
    - Value of information
    - Feasibility

Evidence Generation (1)

- The Clinical and Health Outcomes Initiative in Comparative Effectiveness (CHOICE) will represent the first coordinated national effort to establish a series of pragmatic clinical comparative effectiveness studies in the United States. This initiative will concentrate on under-represented populations (children, elderly, racial and ethnic minorities and other under studied populations).

Evidence Generation (2)

- Clinical Registries
  - AHRQ will make up to five awards for the establishment or enhancement of national patient registries that can be used for researching the longitudinal effects of different interventions and collect data on under-represented populations.

Evidence Generation (3)

- AHRQ will enhance its investments in establishing a learning health care system by funding the DEcIDE (Developing Evidence to Inform Decisions about Effectiveness) Network to expand developmental consortium in diabetes, cancer, cardiovascular disease, and distributed data network models utilizing clinically rich data from electronic health records.
  - AHRQ will use ARRA investment to funds to continue support for the development of a research framework that organizes the major methods topics and prioritizes critical areas for new research on methods, including validation.

Evidence Generation (4)

- AHRQ will use the ARRA investment to fund meritorious grant applications that were not funded in FY 09.

Dissemination and Translation

- The ARRA funds will primarily be used to support grantees in developing and implementing innovative approaches to integrating comparative effectiveness research findings into clinical practice and health care decision making.
- A smaller investment will strengthen the infrastructure supporting these activities, including the John M. Eisenberg Clinical Decisions and Communications Science Center whose workload will substantially increase.

Research training and Career Development

- AHRQ will provide institutional support to increase the intellectual and organizational capacity for larger scale programs in comparative effectiveness and allow fellowship training opportunities.
- Through grant mechanisms, funding will support the career development of clinicians and research doctorates focusing their research on the synthesis, generation and translation of new scientific evidence and analytic tools for comparative effectiveness research.

Citizen's Forum

- Formally engage stakeholders at the critical stages of identifying research needs, study design, interpretation of results, development of products, and research dissemination through a variety of mechanisms that are both inclusive and transparent
- Support programs in citizen awareness addressing the use of comparative effectiveness evidence in health care decision-making

The following individuals/organizations submitted written comments for consideration by the Federal Coordinating Council for Comparative Effectiveness Research

88 comments

Individuals

Theodore Chow, MD, FACC
Linda DeCarlo
Grace E. Jackson, MD
Amy Menefee
David Thomas Martella
Rachel Nosowsky
Cliff Shannon
Bill Wright

Senator Herb Kohl, Chairman, Senate Special Committee on Aging

Organizations

AdvaMed
American Academy of Hospice and Palliative Medicine
American Association for Clinical Chemistry
American Association of Naturopathic Physicians
American Association of Neurological Surgeons/Congress of Neurological Surgeons
American Board of Orthopaedic Surgery
American Clinical Laboratory Association
American College of Cardiology
American Heart Association - National Center
American Medical Group Association
American Osteopathic Association
American Psychological Association
American Society of Health-System Pharmacists
American Society of Clinical Oncology
American Urogynecologic Society
American Urological Association
Association of Clinical Research Organizations
AstraZeneca Pharmaceuticals

Bloomberg School of Public Health, Johns Hopkins University
Blue Cross Blue Shield Association

California Department of Public Health
California Office of Statewide Health Planning and Development
Center for Advancing Health
Center for Perioperative Research in Quality
Center for Pharmacoeconomic Research, University of Illinois at Chicago
Center for Policy Research and Analysis
Child and Family Policy Center
Children's Health Specialists
Citizens for Midwifery
Community Catalyst
Community Resources, LLC
Coverage Policy Unit

eHealth Initiative

Friends of SAMHSA
Frontier School of Midwifery and Family Nursing

George Washington University
Gundersen Lutheran Health System

Health Equity Associates, LLC

Independent Scientific Research Advocates

Journal of the American Medical Association

Lakes Inter-Tribal Epidemiology Center

Marshfield Clinic
Martin, Blanck & Associates, LLC
Medical College of Wisconsin
Medscape, LLC
Meharry Medical College
Mental Health America
Merck Childhood Asthma Network, Inc.
MGH Center for Child and Adolescent Health Policy
Morgan Stanley Children's Hospital of New York-Presbyterian
M2S, Inc.

National Alliance on Mental Illness
National Association of Children's Hospitals and Related Institutions
National Coalition of Mental Health Consumer/Survivor Org
National Hispanic Medical Association
National Initiative for Children's Healthcare Quality
National Patient Advocate Foundation
National Pharmaceutical Council
Nemours Health and Prevention Services
Nutricia North America

Oregon Health Sciences University

Partnership to Improve Patient Care
Parkinson Pipeline Project
Patient Safety Solutions, LLC
Powers, Pyles, Sutter & Verville PC
Prescription Policy Choices

RCHN Community Health Foundation

Samueli Institute
Shore Health System
Society of Thoracic Surgeons
SomaliCAN
SPAN USA
Standard Biologics, Inc

University of Colorado and Children's Hospital
University Hospitals of Cleveland
University of Illinois at Chicago
University of Pittsburgh
University of Wisconsin
United States Psychiatric Rehabilitation Association

Virtual Radiologic Corporation

Submitted by
Cynthia Crummey
American Board of Orthopaedic Surgery
ccrummey@abos.org

The American Board of Orthopaedic Surgery appreciates the opportunity to comment on the value of comparative effectiveness research. Founded in 1934, The American Board of Orthopaedic Surgery provides a 2 step process for initial certification of candidate orthopaedic surgeons and is responsible for the recertification process in orthopaedic surgery, and hand surgery and sports medicine subspecialties. As a part of our dual mission to serve the public and the medical profession we have ingrained specific elements of ethics, professionalism and evidence based practice in multiple areas of our process. To be eligible for the Board certification process, residents must satisfactorily complete an accredited 5 year training program in Orthopaedic surgery. For the initial certification process, we have developed a psychometrically valid examination that tests cognitive expertise in Orthopaedic Surgery (Part I). If successful in Part I, candidates may apply for an Oral Examination (Part 2) after approximately 2 years of independent practice. For the second part of the initial certification, candidates must successfully pass a 360 degree peer review evaluation process prior to sitting for the part II oral examination. Our peer review process employs a standard tool that represents the 6 core competencies, accrues input from multiple types of observers (colleagues, partners, administration and nursing), specifically assesses ethics and professionalism and is unique to our Orthopaedic surgery process. The part 2 examination is an oral examination for which the candidate must submit 6 months of operative cases in a standard format to our proprietary database. In the part 2 examinations, candidates present their own cases to 6 different Board certified volunteer examiners. Candidates are graded on a variety of skills including use of evidence based medicine, ethics and professionalism and systems based practice. Further, The ABOS owns and operates a recertification processes that includes a variety of recertification alternatives that are equally robust and are designed to both protect the public and meet the needs of busy practicing orthopaedic surgeons. Integral to the recertification process is the option of a recertification oral examination. Again for this examination, candidates that have been in practice for almost 10 years submit cases to our proprietary database providing another important evaluation of orthopaedic practice. We believe that this process developed by the American Board of Orthopaedic Surgery has high value to the diplomate and serves to protect the public good.

Though a small percentage of physicians, orthopaedic surgeons provide “cradle to grave” medical care to nearly 25% of the population. As such, orthopaedic care is a high priority to the public. The direct cost for musculoskeletal care and rehabilitation is on the order of 800 billion dollars. In 2004, HHS identified the care of arthritis and nontraumatic joint disorders as conditions as being of special significance and were among the first to be addressed by the Effective Health Care Program. The ABOS recognizes the need for effective health care programs in arthritis, nontraumatic joint disorders and also in the care of the injured patient. The ABOS believes continued evaluation of practice patterns of practicing orthopaedic surgeons gives it the unique opportunity to report on and improve certain aspects of care.

The ABOS is strongly supportive of comparative effectiveness research as an important tool in the fabric of medical decision making and its accumulation of information on orthopaedic practice, including procedures and outcomes, through the certification process provides ABOS a unique opportunity to conduct such research. As part of our commitment to this research, the ABOS has partnered with the Dartmouth Institute of Health Policy and Clinical practice to design, execute and publish a number of articles that are based on observed orthopaedic practices using our proprietary database. We have published or will soon publish articles that describe geographic variation in various orthopaedic practices, trends in practice patterns for the treatment of various conditions, the differential effectiveness of certain procedures in the treatment of various conditions and the effectiveness of educational programs in Orthopaedic surgery. These manuscripts, published in rigorous peer review journals can improve orthopaedic practice and are, therefore, useful to both our colleagues and the public.

Because we have useful data in our proprietary database that has been systematically collected over a number of years, the ABOS is in a unique position to continue to report on issues of effectiveness, device utilization, geographic practice variation and other important issues in Orthopaedic Surgery. Constructively employed, works of this type should be reported to give both physicians and patients a better understanding of the ramifications of personalized medical decisions. Hopefully, works of this type will stimulate innovation, further research and not stymie progress. We will continue our independent work in this area for the benefit of the profession and for the value to the public. Such initiatives will also have translational benefit in related fields of surgery and musculoskeletal health. We are eager to work with the Department of HHS in furthering the work that will lead to improved, clinically useful evidence-based guidelines and new educational formats that provide safe, effective treatment, while conserving the resources available for healthcare.

Novel projects that partner with the individual Boards that control the certification process for their diplomates in the various specialties could yield the next generation of performance improvement in the specialty of orthopaedic surgery and other specialties.

Respectfully submitted,

John Gray Seiler, III MD, President, American Board of Orthopaedic Surgery
Harry Herkowitz, MD, President–Elect, American Board of Orthopaedic Surgery
Shep Hurwitz, MD, Executive Director, American Board of Orthopaedic Surgery

For further information on the American Board of Orthopaedic Surgery: abos.org

Background References:

Clancy CM MD. AHRQ's Research Efforts in Comparative Effectiveness. Statement before the US House of Representatives Committee on Ways and Means, Subcommittee on Health. June 2007.
Ellis P. Research on the Comparative Effectiveness of Medical Treatments. A CBO paper. Congress of the United States. 2007

Garber AM, Tunis SR. Does Comparative –Effectiveness Research Threaten Personalized Medicine. New England Journal of Medicine. Vol. 360, 9, 1925-1927, 2009

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Koval KJ, Harrast JJ, Anglen JO, Weinstein JN. Fractures of the distal part of the radius. The evolution of practice over time. Where's the evidence. Journal of Bone and Joint Surgery. 90, 1855-1861, 2008.
Anglen JO, Weinstein JN and the ABOS Research Committee. Nail or Plate Fixation of Intertrochanteric Hip Fracture: Changing pattern of practice. A review of the American Board of Orthopaedic Surgery Database. Journal of Bone and Joint Surgery. 90, 700-7007, 2008.
Garrett We, Swiontkowski MF, Weinstein JN, Callaghan J, Rosier RN, Berry DJ, Harrast J, DeRosa GP and the research committee of the American Board of Orthopaedic Surgery. Journal of Bone and Joint Surgery. 88, 660-667, 2006.
Herkowitz HN, Weinstein JN, Callaghan JJ, DeRosa GP and the American Board of Orthopaedic Surgery. Spine Fellowship Education and its Association with the Part II Certification Examination. Journal of Bone and Joint Surgery. 88, 668-670, 2006.

Current ABOS Research Projects

1) Board Certified and Non Board Certified Orthopaedic Surgeons- differences in outcomes, cervical spine.
2) Arthroscopic shoulder surgery, changes in practice for subacromial decompression.
3) Shoulder surgery, changes in practice for rotator cuff conditions.

Submitted by
Kristin Hill, MSHSA
Lakes Inter-Tribal Epidemiology Center
KHill@glitc.org

I am responding to the invitation to provide comment to the Coordinating Council pertaining to CER (comparative effectiveness research). As the Director of the Great Lakes Inter-Tribal Epidemiology Center, one of twelve "epicenters" currently residing in Indian Health Service Areas, I would like to invite our involvement in the education and application of CER consistently when working in our Tribal communities. I am just now learning more about CER, and am understanding that CER may be (or has been) the standard process for clinical research and program evaluation as health care reform proceeds. The Tribal Epidemiology Centers began as a result of legislation in 1996 and have built trusting relationships with Tribal communities in order to increase data collection, analysis and use in health care decision making. We play a vital role in advocating for increasing representation of American Indians in data samples, data accuracy and translation of research in the community.

Actually, I would like to propose a conference call between the Coordinating Council and Directors of the 12 Tribal Epidemiology Centers (or face to face if possible) to explore how we can be involved and therefore, including the US indigenous population. I recommend that the meeting also include members of HRAC. Currently, American Indians carry a high burden of disease and are underserved. Unless we increase American Indian research, data and

representation, any health care system designed will continue to under serve this population. Please call me at your convenience and I will be pleased to coordinate a meeting.

Submitted by
Catherine DeAngelis, MD, MPH
Editor-in-Chief
Journal of the American Medical Association
cathy.deangelis@jama-archives.org

Comparative Effectiveness Research should comprise the following components:

1. A well thought out design to answer a specific, important question or questions. This design should include context, objective(s), populations to be studied, specific study design, expected and then actual results and conclusion(s).

2. Funding should be based on relative importance of the study to health of the public as compared to other proposed studies

3. A definite time line that can be completed realistically considering the population (numbers and types of individuals available) and other resources needed to complete the study.

4. Periodic reporting of results to determine the likelihood of completing the study. Scare funding should not be allocated to those studies that clearly cannot meet the time line indicated.

5. Peer review of the study results and publication with no further funding for that research if results do not meet expectations for that study

Submitted
Karen Lencoski, JD, MBA
Federal Manager, Government Relations & Advocacy
American Urological Association
Email: klencoski@auanet.org

The American Urological Association (AUA), the pre-eminent professional association for over 16,000 urologists worldwide and almost 10,000 in the U.S., greatly appreciates the opportunity to present our suggestions for comparative effectiveness research priorities to the Federal Coordinating Council. The long-standing mission of the AUA is to promote the highest standards of clinical urologic care through education, research, development of clinical guidelines, and the formulation of healthcare policy. The public health burden of urologic disease in the U.S. is large and growing, with an estimated annual impact of over $11 billion. Urologists are the specialists who most often diagnose and treat prostate cancer, the second leading cause of cancer deaths among men in the U.S. Urologists treat many other conditions common to Medicare beneficiaries, including urinary tract infections, benign prostatic hyperplasia (BPH), and urinary incontinence. Over fifty percent of the patients who see urologists are Medicare beneficiaries. We heartily endorse the Administration's focus on comparative effectiveness research (CER) as

an evidence-based, systematic way to identify the best treatments for specific patient populations and to generate the critical information that will help guide both physicians and patients through the complex decision process to select the most appropriate treatment. Medical specialty societies are uniquely positioned to identify evidence gaps in care, and where CER should be focused.

The AUA nominates three areas that warrant further investigation in a CER framework.

I. *Prostate cancer treatment*.

In 2008, an estimated 200,000 men were newly diagnosed with prostate cancer in the U.S., and about 29,000 men died from the disease. Prostate cancer is one of the most common cancers in men, and a significant health problem. Significant prostate cancer disparities exist between rural and urban populations and across racial and ethnic groups. Several studies have demonstrated that patients with prostate cancer in rural areas are more likely to be disadvantaged due to being diagnosed at later stages, receiving different management, being uninsured or underinsured, and having less desirable outcomes than their urban counterparts. African American men have prostate cancer mortality rates that are more than twice the rates observed in other racial and ethnic groups, and compared to white men are less likely, across all age groups, to receive treatment for prostate cancer, especially when diagnosed with more advanced cancer. Because prostate cancer is the second-leading cause of cancer deaths in men, these disparities are particularly significant. Appropriate PSA testing has helped with early detection of prostate cancer, but PSA is not equally available to the above noted populations.

Moreover, depending on whether the cancer is aggressive or slow growing, the range of options can vary significantly, and patient preference regarding treatment side effects and quality of life plays a particularly prominent role in prostate cancer treatment choice. In addition, prostate cancer is a focus of the Medicare program: it has been identified as one of eight high cost conditions selected for the Physician Resource Utilization Report pilot program authorized by Congress in the Medicare Improvements for Patients and Providers Act of 2009 (MIPPA). Yet, despite its prevalence, cost and complexity, there is a distinct lack of evidence comparing the treatment options for localized prostate cancer, These options include:

- Active surveillance (watchful waiting);
- Radical prostatectomy, which includes both conventional and robotic surgeries; and
- Radiotherapy, which includes brachytherapy, proton beam, and Intensity-Modulated Radiation Therapy.

The benefits and risks (e.g., incontinence, impaired bowel function, reduced sexual function) of each of these options must be carefully weighed by the patient and his family in consultation with his physician. CER studies should compare long term treatment option outcomes such as overall survival, quality of life and patient satisfaction for different patient populations (e.g., age, race, tumor characteristics, genetic profile, etc).

II. *Sustained public/private support for a robust quality infrastructure*.

All healthcare reform proposals are predicated on the presumption that a robust and well-developed quality infrastructure exists. However, this is not uniformly the case. The infrastructure varies substantially by provider type (hospital, dialysis facility, specialist

physicians) and condition (diabetes, cardiovascular disease, prostate cancer). There are several building blocks fundamental to the creation and maintenance of this essential infrastructure. First, basic clinical research provides the evidence needed to formulate clinical guidelines. In turn, evidence-based clinical guidelines are a prerequisite for the development of quality performance measures, and clinical registries provide the data to revise guidelines, test measures, provide comparative performance information and benchmarks, identify areas for quality improvement and highlight areas that require additional research.

We ask for support for the development and maintenance of the quality infrastructure through creation of a public/private partnership, under the auspices of AHRQ or NIH, both of which are positioned to accept private funds. This venue could be used to pool the resources of those public and private stakeholders, including government, business, private insurers, research entities, and medical specialty societies, all of whom have a vested interest in quality measurement and improvement and evidence based medicine.

III. –*Comparison of Imaging Modalities for Major Urologic Conditions*. A number of analyses conducted for MedPAC have established that at least some portion of the rapid rate of increase in physician ordered imaging services is attributable to duplicative or inappropriate imaging. Under the Deficit Reduction Act of 2005 (DRA), Congress reduced reimbursement for some imaging studies to exert control over this area. The AUA is embarking upon a comparative effectiveness study of imaging modalities for ureteral stones, with the intent of producing evidence-based guidance on the most effective and efficient imaging for this condition to disseminate to urologists, primary care practitioners and others who may treat this condition. The AUA requests that the Council consider the comparative, evidence-based study of imaging modalities for urologic and other conditions as an important focus for comparative effectiveness research. Such guidance across a range of conditions could go a long way to help the medical profession order only those imaging studies that are most effective and appropriate for the condition in question.

Submitted by
Linda DeCarlo
New York, NY
ldspeaking@aol.com

The comments below were submitted verbatim numerous time. Possibly initially sent to a large mass (for instance a list serv or other media) and encouraged interested parties to submit the written statement.

Comparative Effectiveness Research is essential to determine the etiologies of chronic illness and to determine those treatments that are effective versus those treatments that merely attempt to suppress symptoms while ignoring the underlying cause of the illness. In 2004, the Milken Institute (non-partisan, non-profit) issued its report, *An Unhealthy America: The Economic Burden of Chronic Disease* citing the most prevalent chronic illnesses in America as lung disease, heart disease, hype rtension, mental illness, cancer, diabetes, and stroke. ; Fifty percent of all Americans have at least one of these chronic illnesses. Each of these chronic illnesses has an environmental contribution in its etiology that rarely is identified or evaluated. Yet billions of dollars are spent---often wasted—by trying to suppress symptoms without identifying the cause

of the illness. The healthcare costs and dollars of lost productivity can be found, state by state, on their website www.milkeninstitute.org.
For years, there has been a debate whether certain chronic illnesses are psycho-somatic, or illnesses emanating from the body with psychiatric sequelae. Those who insist these illnesses are psychosomatic have never fully evaluated the patients to determine whether an underlying physical illness might be contributing to the psychological features. The phenomenon of a healthcare practitioner ascribing a psychiatric diagnosis to a real physical illness is eloquently penned by Harvard researcher Jerome Groopman , M. D. in his book *How Doctors Think*. Repeatedly, Dr. Groopman documents that serious and even life-threatening illnesses go undiagnosed by "well-trained" physicians who have=2 0a pre-conceived idea of what the patient should have, and are inexperienced in the patient's particular presenting diagnosis. Illnesses from celiac disease to Wilson's disease have been missed by physicians. In both of these cases, the patients would have died without the correct diagnosis which was later made by a more thorough and experienced doctor.

This issue of misdiagnosis due to inexperience raises grave questions about medical competency, medical training, patient outcome, and healthcare costs. In every instance of misdiagnosis, the patient is harmed and loses faith in a medical care system that should be wiser.
The Gold Standard research model in medicine which can clarify many issues of accurate diagnosis and effective treatment is the Environmental Control Unit (ECU). Consistent with President Obama's insistence on transparency and integrity in government, there is no area more important than healthcare where transparency and integrity are imperative. We need o bjective, accurate, non-lobbied data and outcome inform ation if we are to reverse the downward spiral of chronic disease and its enormous financial burden on U. S. citizens, personally and financially. Each of the above seven chronic diseases is on the rise. We must stop this trend by identifying the causes, eliminating them, and provide treatments that restore the patient's health rather than merely medicate the symptoms. An Environmental Control Unit is the research tool that can accomplish our best medical goals with complex medical conditions.

An ECU is a set of patient care rooms either on a wing of a medical facility, or located in a free-standing building. The air on the ECU is filtered to be free of any chemicals and the ECU is constructed with low- or no toxicity building materials. No fragrances are allowed on the Unit, and temperature and humidity are controlled. The specially-trained medical staff is instructed in the protocols necessary to identify and monitor the patient's symptoms, obtain baseline data on each patient, and monitor each patient as modifications to the patient's environment (air, food, water) are made. The 24-hour medical staff is available to document the impact of each carefully determined change for each patient. The test results are then assessed by the medical staff, and specific courses of treatment are identified for not only each patient, but also for symptom clusters that were previously considered unrelated. Japan has several ECU's.
This scientific approach is crucial for illnesses thought to be "purely" psychological (mental illness, chronic fatigue, fibromyalgia, etc.) as well as for patients with illness primarily somatic (COPD, cardiac disease, etc.). The data collected through an ECU will demonstrate the etiologies and treatments that are accurate and effective and end the needless and unproductive controversy between psycho-somatic and somatic illness for some well-described disease states.
Once delineated, psychological counseling and/or psychotropic medication may be required to stabilize the patient.

Of course there another, related perspective: Those patients with neurological illnesses which have an environmental component as a possible cause. *Harrison's Principles20of Internal Medicine* 16th Edition 2005 New York, McGraw Hill p 2408 , includes the following possible causes for Parkinson's Disease: Valproic acid, Fluoxetine, Lithium carbonate, alpha methyldopa, typical antipsychotic medications, manganese, methanol, carbon disulfide, carbon monoxide, anti-emetics, and possibly n-hexane to name a few. Yet these etiologies often go unexplored in patients with Parkinson's Disease.

Another related area for research funding is evaluating the human health consequences of chronic exposure to low levels of toxic chemicals. Cigarette smoke is a paradigm for many environmentally-triggered illnesses. Once thought to be harmless, with package labeling dispelling any health concerns in the 1960's, through observation and research we have come to learn that cigarette smoke causes multi-system disease that can lead to death. Lung cancer, heart disease, stroke, asthma, vasculitis, and low birth weight are all consequences of exposure to cigarette smoke, whether one smokes or whether one is exposed to second-hand smoke. This is not surprising considering cigarettes contain more than two hundred chemicals that were applied in the growing of tobacco through the production of the final pr oduct.
Other chemicals cause human illness as well, and increased incidence of illness has also been established between environmental chemicals and asthma, prostate cancer, and many other illnesses. These findings have been published in a wide variety of medical journals, and research into the environmental causes of disease is spread among many research institutes, therefore, the vast amount of information regarding the environmental impact on health is poorly centralized. We already have an unfortunate baseline describing chronic disease as reported in the Milken report. Funding is needed to conduct research that will identify the cause of illness and describe the best treatments. Funding an ECU will accomplish this and spawn an improvement in medical education so physicians will be better trained to diagnose and treat complex medical conditions.

Dr. Adrienne Sprouse presented this statement at the Listening Session in Washington, D. C. on April 14, 2009. I support this plan and ask the FCC to recommend its funding.

Submitted by
Naomi Aronson, PhD
Executive Director, Technology Evaluation Center
Blue Cross Blue Shield Association

Blue Cross and Blue Shield Association's Statement to the Federal Coordinating Council on Comparative Effectiveness Research Listening Session (May 13, 2009)*
I am Naomi Aronson, Executive Director of the Technology Evaluation Center, speaking for the Blue Cross and Blue Shield Association (BCBSA). Collectively, BCBS Plans provide healthcare coverage for 1 in 3 Americans. BCBSA strongly supports advancing comparative effectiveness.

Our priority recommendations are based on the potentially large populations affected; recognized "gaps" in the evidence; known disparities in outcomes; need for evidence specific to affected

subpopulations; and the need for evidence to inform patient preferences in selecting among treatment alternatives.

Our first priority is management of chronic stable angina. Coronary heart disease is the leading cause of death and a major cause of disability in the U.S.

Specifically, we call for research that compares:

- optimal medical management;
- percutaneous coronary interventions (including bare-metal versus drug-eluting stents); and
- coronary artery bypass grafting (including on-pump versus off-pump techniques).

We also want to know what works best in specific subpopulations: women; racial and ethnic patient groups; the elderly; diabetics; individuals with co-morbid conditions; and the exercise impaired.

Given the complexity of comparisons, the starting point should be a comprehensive systematic review. Identifying gaps in the evidence will set priorities for new studies.

Our second priority is treatment of localized prostate cancer.

One in 6 men in the U.S. will be diagnosed with prostate cancer. African-American men are disproportionately affected, having higher disease incidence and mortality; however, a recent Agency for Healthcare Research and Quality (AHRQ) report found no randomized, controlled trials that stratified patient outcomes by race.

Overall, there is a well-known lack of evidence comparing management strategies for localized prostate cancer, namely:

- active surveillance;
- radical prostatectomy (including the manner performed, i.e., robotic or conventional); or
- radiotherapy (conformal therapy, brachytherapy, proton beam, or intensity-modulated radiotherapy).

The outcomes should include overall survival, quality of life, adverse effects, and costs. Specific variables examined should include race, age and, of course, tumor characteristics. The recent AHRQ systematic review has demonstrated the dearth of comparative studies. Recognizing that launching randomized, controlled trials of this complexity would be a resource-intensive undertaking, we suggest that a thoughtfully designed observational study could be efficient and informative. Such an observational study could focus questions for subsequent trials.

Our third priority is cross cutting: how to translate knowledge of what works to care that will work.

What interventions can improve clinician and patient adoption and use of evidence-based care? When is it more effective to target change at the organizational level, the community level, or the individual level? What approaches and incentives to dissemination and adoption are most effective and under what circumstances? We must know how knowledge of what works can be translated to health care that will work.

The Blue Cross and Blue Shield Association appreciates the Council's efforts to set priorities for comparative effectiveness research. Thank you for the opportunity to present today.

Submitted by
Ron Manderscheid, PhD
Global Health and Civil Sector, SRA International, Inc.
and Department of Mental Health
Bloomberg School of Public Health, Johns Hopkins University
Ronald_Manderscheid@sra.com

Dr. Clancy and Other Council Members:
My name is Dr. Ron Manderscheid. I am the Director of Mental Health and Substance Use Programs at SRA International and Adjunct Professor in the Department of Mental Health at the Bloomberg School of Public Health at Johns Hopkins University. Previously, I held research and managerial appointments at the National Institute of Mental Health and at the Substance Abuse and Mental Health Services Administration.

I am here to speak on behalf of the 107 mental health and substance use prevention and treatment organizations that comprise the Whole Health Campaign. The Campaign seeks good universal health insurance coverage, good integrated care, and good prevention services for persons with mental or substance use conditions. We strongly support national health reform efforts and have prepared eight policy analyses that address key topics of reform.

I would like to make three very brief points today:

First, and most important, the federal comparative effectiveness research enterprise must be guided by consumer and family input. Consumers and families have the direct, lived experience of major health problems, as well as direct experience with successful and failed interventions. They can provide important and needed advice on priority setting and ongoing operations.

Second, the federal comparative effectiveness research enterprise must build infrastructure and train researchers. Currently, the mental health and substance use prevention and treatment fields lack the infrastructure and trained personnel to undertake comparative effectiveness research and bring needed innovation to the field. I know of only two comparative effectiveness studies done for either field. It is critical that the mental health and substance use fields not become backwaters in an ocean of progress: We do know that a person cannot have good health without also having good mental and addictive health. Hence, developing appropriate infrastructure will be important to the entire health enterprise.

Third, the federal comparative effectiveness research enterprise must be designed broadly. Specifically, it must span treatment, prevention, promotion, and health determinant interventions designed both for persons and for populations. To achieve better balance in expenditures on person and population interventions, we recommend that at least one-quarter of federal comparative effectiveness research expenditures be devoted to population interventions.

Also, comparative effectiveness research must span traditional research boundaries. For example, we need to be able to understand the joint effects of targeted depression and diabetes treatments, since these conditions very frequently co-occur together. Failure to consider such joint effects in the past has resulted in a situation where public mental health clients die 25 years prematurely.

In closing, the Whole Health Campaign looks forward to dramatic improvements in mental health and substance use interventions as a result of comparative effectiveness research. We want to support the Council in any way that we can because we recognize the vital importance of your work.

Thank you very much for the opportunity to testify today.

Submitted by
Grace-Marie Turner
President and founder
The Galen Institute – a nonprofit research organization dedicated to patient-centered health reform solutions
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To the members of the Federal Coordinating Council for Comparative Effectiveness Research:

Thank you for requesting comments and suggestions from the public about the role of comparative effectiveness research in American health care. It is widely agreed that doctors and patients need improved information about medicines and treatments to make the best decisions about patient care. As you know, many of us are concerned about how our country's program of comparative effectiveness research will be structured, what type of information will be gathered, and how it will be used. I would like to share some of our concerns with you now.

President Obama has repeatedly told us that we won't have to give up the health care we have if we're satisfied with it. In his Questions and Answers document during the campaign, he further stated that his **"plan will not tell you which doctors to see or what treatments to get. …No government bureaucrat will second-guess decisions about your care."**

Yet now the Federal Coordinating Council for Comparative Effectiveness Research, created through the economic stimulus bill, will have new and untested power over comparing medical treatments.

The real intent of the legislation came out in draft language from House Appropriations Chairman David Obey (D-Wis.), declaring that drugs and treatments "that are found to be less effective and in some cases, more expensive, will no longer be prescribed." This caused a political uproar, with some legislators urging that the council be strictly limited to "clinical" effectiveness studies and not allowed to embark on "cost-effectiveness" studies. We urge the council to focus exclusively on clinical effectiveness in directing research.

Many Americans are concerned about any federal policy that moves decisions away from patients and their doctors and puts them in the hands of federal authorities. I am concerned that the board could be susceptible to political influences and far removed from the unique medical needs of individual patients. Though the members of the council surely are well-intentioned, one body simply cannot judge what is best for all Americans.

The idea behind comparative effectiveness research is to make recommendations on the merits of competing medical treatments. While the idea of this "evidence-based medicine" sounds attractive – everyone wants to get better value for their health spending – giving a governmental or even quasi-governmental body authority over people's health choices is more likely to interfere with quality care than to improve it.

Experts from Europe and Canada, where comparative effectiveness agencies already are established, warn that this is far from the glide path to saving money and improving the quality of care that many political leaders believe.

Professor Michael Schlander, a well-respected German physician, medical researcher and economist, found that decisions by the National Institute for Health and Clinical Excellence in the U.K. have actually led to higher spending for the National Health Service, not the savings that had been expected.

The complexities of the clinical decision-making process are enormous, including the necessity of taking into account the needs of patients who may fall outside norms. Individual differences in responses to drugs and treatments are shoved aside, especially disadvantaging patients who do not respond well to standard care.

The central problem lies in having the federal government serve as the ultimate decision-maker in comparative effectiveness reviews. CER certainly has a place in the health care system in which multiple entities are analyzing and reviewing research, but one centralized government decision-making body simply cannot take into consideration the individual needs of multiple payers and 300 million Americans.

It is imperative that America use CER to provide the highest quality of information, not to dictate how doctors and patients use that information.

A centralized system conceived to compile information about the relative effectiveness of medical treatments would quickly turn into a tool to reward doctors who follow recommendations and punish those who don't. Doctors and hospitals would be directed to follow the recommendations, and their reimbursement – and risk of lawsuits – likely would depend on compliance. Comparative effectiveness boards, not doctors, would be making decisions about which treatments would be available, and the system would become more and more rigid as doctors fear going against the rules.

It is also evident that comparative effectiveness could stifle innovation.

If allowed to dictate the preferred practice of medicine, comparative effectiveness essentially replaces the experience, wisdom and knowledge of physicians with bureaucracies that reduce

decisions to formulas. In the name of protecting their bottom lines, public and private health care plans would likely refuse to cover treatments and procedures that didn't have the approval of this centralized agency.

Physicians and hospitals, fearing lawsuits, would also be much less likely to try treatments not yet analyzed and approved by the comparative effectiveness body – even if early evidence suggests a treatment might work for a particular ailment or set of patients.

And medical companies would be less likely to pursue research on new and potentially life-saving drugs, biologics, and medical devices when faced with another major bureaucratic hurdle to introducing their products to market. Ultimately, funds for new research would shrivel.

We cannot allow bureaucracy to replace innovation.

A centralized process of CER decision-making would slow the adoption of new medicines and other innovations in medical practice, including surgeries. The health sector would become more rigid and less open to innovation in the process. Federal standards simply cannot be flexible enough to accommodate the ever-changing and evolving nature of any science, including or perhaps especially medicine.

Those with experience in CER abroad say it is almost impossible to integrate clinical findings and cost estimates because they use different methods of evaluation. As a result, many subjective decisions are made in what is believed to be an objective scientific process.

A new study from the Institut économique Molinari in France says that approval processes in Europe are increasingly "tough, heavy-handed and costly … Despite the best intentions, the inevitable consequence of these regulations is to push up the cost of innovation substantially, to undervalue its benefits and to reduce the number of new products by making certain projects unprofitable."

I urge you to put patients first in all you do and in your coordination with federal agencies and Congress. Comparative effectiveness reviews must focus on creating quality information about medical treatments and then allow doctors and patients to make decisions, without limiting their freedom.

Thank you again for receiving my comments.

Submitted by
Marc H. Gorelick, MD, MSCE
Professor and Chief, Section of Pediatric Emergency Medicine
Medical College of Wisconsin
Milwaukee, WI

I am writing regarding the Federal Coordinating Council for Comparative Effectiveness Research, authorized by the ARRA. As a clinical effectiveness researcher with nearly 20 years of experience in pediatric emergency medicine, I have a strong interest in this extremely important initiative. I hope the Council will consider the following points as they proceed with their work:

-- Diagnostic and therapeutic interventions frequently differ in their effectiveness in children compared with adults. Moreover, the measures of effectiveness needed to evaluate services may be different. Any program of CER must account for those conditions and services that are relevant to children, and use methods (including outcome measures) that are appropriate to the population under study.

-- Emergency physicians, and emergency departments, have a unique role to play in CER for both adults and children. First, the ED is often the point of contact for many individuals with both acute and chronic conditions, especially among populations with limited access to regular sources of care. Second, the spectrum of illness and injury treated in the ED is broad. Acute illness and injury is the primary cause of morbidity and mortality for children. In addition, for many chronic conditions, such as asthma or diabetes mellitus, much of the morbidity is in the form of acute exacerbations requiring emergency care. Finally, the ED is frequently at the nexus of care, with interconnections across disciplines and settings.

-- Much comparative effectiveness research is conducted in single settings or systems of care, posing a threat in terms of power and generalizability. Indeed, many of the questions that remain unanswered relate to uncommon conditions or outcomes that have proven challenging to study. Multicenter research networks provide a promising means of addressing such pressing questions, and use of such networks is the best way to ensure that CER is adequately powered for important but uncommon conditions or outcomes. Examples of such problems include pediatric trauma, life-threatening medical illness in children (such as sepsis and severe asthma), and issues related to special populations (such as children with special health care needs).

Thank you for your consideration, and I look forward to seeing the work of the Council as it progresses.

Submitted by
Daniel L. Cohen MD, FRCPCH(UK), FAAP
Martin, Blanck & Associates, LLC
Clinical Quality, Medical Management and Patient Safety

I presume that the approach going forward will include, in addition to new traditional controlled studies or meta-analyses of currently published studies focusing on specific clinical interventions—procedures, medications, diagnostic studies, etc—the utility of programs such as disease management or case management which are now highly touted, but to date have shown mixed results. The way we provide therapy may likely be as important as the specific therapy provided regarding outcomes. The reason I mention this is because it is through partnerships between patients and providers that best outcomes may be achieved. For example treating hypertension may be more effective when anti-hypertensive medications are provided as part of a comprehensive hypertension management program, not simply as stand alone interventions. Of course the null hypothesis is that comprehensive DM programs result in little improvement per se, but that is why the universe has given us Chi Square.

Submitted by
Bill Wright
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Dear Members of the Council.

I have served as a public patient safety advocate for the past six years.

As a supporter of the President's healthcare agenda, I have been shocked repeatedly, when hearing the contents of this agenda, that the public issues surrounding the elimination of medical errors are missing.

Besides saving more that 200,000 lives annually, safer medical care could reduce the financial burden on American taxpayers by more than $4 Billion every year.

Let me encourage your support of the reinstatement of "Patient Safety" as a major item on the national agenda by your Council.

"In patient safety circles, I am also know as **"justapatient".**

Submitted by
Peter Dayan, MD, MSc
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To the Council:

An appropriately large proportion of the discussion on comparative effectiveness research has regarded specific interventions for specific disease states.

However, knowledge translation research must not be overlooked. The emergency setting provides a unique environment wherein strategies to bring knowledge to the bedside that have been studied in other settings (e.g. in-patient setting) may not be applicable or effective.
I hope that the discussants will consider the importance of performing research that compares the effectiveness of different implementation strategies, such as decision support, on patient outcome for an array of diagnostic tests and therapeutic interventions in the ED setting.
We are behind other countries in knowledge translation research; now is an opportune time to catch up.

Submitted by
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My name is Glen Schumock. I am Associate Professor in the Department of Pharmacy Practice at the University of Illinois at Chicago (UIC) and Director of the Center for Pharmacoeconomic Research. The Center for Pharmacoeconomic Research is part of the UIC College of Pharmacy and is affiliated with the UIC Institute of Health Research and Policy, and Center for Health Services Research. Importantly, the UIC Center for Pharmacoeconomic Research is the coordinating center for the Chicago-Area DEcIDE Center, one of 13 DEcIDE Center that are part of the Agency for Healthcare Research and Quality (AHRQ) Effective Health Care (EHC) Program.

As you know, the level of spending on health care in the US exceeds that of all other countries in the World. In 2007, health care expenditures in the US rose 6.1% to $2.2 trillion, or 16.2 percent of gross domestic product. By 2017, total expenditures are expected to reach $4.3 trillion. While the US spends more on health care and prescription drugs then other countries, data are mixed as to the value it gets for the money spent. For example, with a life expectancy of 77.5 years, the US ranks twenty-second among 30 OCED counties, with the average for OCED countries being 78.3 years. The US also has the lowest kidney transplant survival rate, the third highest rate of deaths from medical errors, and the third highest infant mortality rate among OCED countries.

Part of the problem in the US is that we do not have good information to make decisions when selecting among treatments for a specific indication. In particular, there is increasing awareness of the limited information provided by traditional randomized clinical trials to inform decisions about therapeutic alternatives as applied in actual practice. The randomized controlled trial (RCT) has long been recognized as the "gold standard" for evidence on the efficacy of innovative medical care interventions, particularly drugs, and RCTs are required by the FDA to market a new drug product. The pivotal Phase III RCT sponsored by a pharmaceutical company in the drug development process has a specific purpose – to establish the efficacy of the new drug (i.e., Can the drug work?). Yet it is now increasing understood that the traditional RCT does not provide the information necessary for practitioners to understand how the agent works in normal practice (Does the drug work?), and how it compares to existing agents.
These are the questions that CER answers. Thus, while traditional phase III RCTs have a clear purpose – that being to provide efficacy data to support market approval of a new drug, these trials do not typically provide the direct evidence required by decision-makers to make selections between therapeutic alternatives. Comparative effectiveness studies, on the other hand, are designed to generate such evidence and thus are more relevant to actual practice. Both types of studies are important and together complete the breadth of evidence needed for effectively evaluate therapeutic options.

I strongly support the work that AHRQ has conducted to-date in the area of comparative effectiveness research (CER). The Agency has developed a sound infrastructure that includes dozens of centers and hundreds of researchers from across the United States. These centers and researchers have produced important results and advanced the field of CER from a place of relative obscurity a few years ago to one that is now routinely discussed in the lay press. The EHC, which is comprised of the DEcIDE Centers, CERTS, and EPCs, among others, represent a wealth of experience and expertise; and these centers should remain the center point of the CER efforts going forward.

While the work that had been conducted under the EHC Program has been extremely successful, it is my opinion that more needs to be done to ensure that the findings of CER results in actual changes in practice. AHRQ should use funding provided under the ARRA legislation to ensure that physicians and other health care practitioners have access to results of CER and are able to translate the findings into their own practices. By doing so, AHRQ will better ensure that the American public is benefitting from the investments made in CER. I also believe that the Agency can expand the types of CER conducted, to include both retrospective observational studies and prospective clinical trials.

The ARRA funds present an unprecedented opportunity to both expand the important infrastructure already developed by AHRQ to conduct CER, but also to expand the scope of the work conducted.

Submitted by
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The following statement reflects the views of the University of Illinois at Chicago (UIC) Center for Pharmacoeconomic Research. The Center for Pharmacoeconomic Research is part of the UIC College of Pharmacy and is affiliated with the UIC Institute of Health Research and Policy through the Center for Health Services Research. Importantly, the UIC Center for Pharmacoeconomic Research is the coordinating center for the Chicago-Area DEcIDE Center, and has been actively involved in comparative effectiveness research (CER) for several years.

Researchers associated with the UIC Center for Pharmacoeconomic Research conduct studies evaluating the comparative effectiveness and cost effectiveness of medications and other medical technologies. Too often new drugs are approved and marketed in the United States (US) without sufficient evidence available to understand the relative benefits and risks compared to existing agents. Pharmaceutical companies rarely have incentives to compare their agents to reasonable therapeutic alternatives. Yet this is the type of study necessary for patients and healthcare providers to make informed treatment decisions when considering treatment alternatives. Given its unique position to facilitate research for the public good, it is imperative that the federal government take the leading role in supporting the generation of new evidence that directly compares relevant treatment alternatives – just as the Agency for Healthcare Research and Quality (AHRQ) has done through the Effective Health Care (EHC) Program.

While the work conducted by AHRQ to-date in the arena of comparative effectiveness has been noteworthy, we feel that there should be greater consideration of economic factors in future CER. Incorporation of costs is an integral part of informed decision-making, and as such understanding of the cost implications of therapies should be a central element of comparative effectiveness research. The relevance of costs highlighted by the current budget pressures faced by US healthcare payers and likely will have to become one of the explicit criterion by which policy is guided as US healthcare, as currently structured, becomes unsustainable. Information about costs allows us to understand not only the direct differences in terms of clinical outcomes but also the value of interventions and whether or not they represent an efficient use of resources.

We would note that AHRQ EHC Program is well conceptualized and executed, and provides an existing infrastructure on which to build for the conduct of CER. Further, the DEcIDE Centers, CERTS, and EPCs represent a wealth of experience and expertise. With appropriate funding, this resource has the capacity to do much more than it has in the past.

We also feel that CER should continue to use a variety of study designs to generate evidence about the comparative effectiveness, comparative safety, and cost effectiveness of medical interventions.

Observational studies, pragmatic clinical trials, and other study designs offer complementary information with varying resource requirements and we believe it is important to continue to take

advantage of a broad array of study designs as well as to examine new ways to collect and organize the data and conduct these types of analyses.

Finally, we would support a broad agenda for future CER that focuses on the evaluation of important new medical technologies, interventions and programs. Not only do we need to understand comparative effectiveness of treatments for a particular disease, but we also need to understand the value and impact of preventative health care interventions, programs for medication therapy management, diagnostic technologies, and disease management programs. Not only is the generation of this evidence important, but its uptake into practice is critical, and new approaches for dissemination and translation of CER will be essential to inform and change practice in healthcare that balances the interests of all stakeholders.

The ARRA funds present an unprecedented opportunity to expand the important infrastructure already developed by AHRQ to conduct CER. In doing so, we can usher a new era of evidence-based decision making into the US healthcare system that will benefit all Americans. The UIC Center for Pharmacoeconomic Research stands ready to continue to assist in this endeavor.

Submitted by
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The Pittsburgh Regional Health Initiative (PRHI – www.prhi.org) is among the country's first multi-stakeholder, regional healthcare improvement coalitions and was a founder, and now fiduciary agent, of the Network for Regional Healthcare Improvement (NRHI). PRHI's board of directors includes representatives from the region's hospitals, its physician community, major private and public healthcare purchasers, consumer advocates, and insurers. Our stakeholders believe strongly in the power of such regional collaborations to test and disseminate quality and efficiency improvements that will have the greatest effect on patients and cost.
We at PRHI associate ourselves with the recommendations you have received from NRHI. In addition, we want to bring to your attention unique PRHI projects that could define and shape comparative effectiveness research (CER) by building on experience from regional projects and demonstrating the value of a leadership role for regional quality coalitions like PRHI to advance CER.

While many regional healthcare coalitions concentrate on encouraging quality improvement through public reporting, PRHI has focused on initiating clinical quality improvement projects, and on measuring results on the basis of patient outcomes. Because PRHI has been able to tap into the singularly comprehensive clinical database of the Pennsylvania Health Care Cost Containment Council (www.phc4.org), most projects are measured in terms of unequivocal patient outcomes: e.g., risk-adjusted rates of in-hospital mortality, complications and 30-day readmissions; incidence of hospital-acquired infections.
When PCH4 data is not available, we work with our project partners to develop data and measures that track patient outcomes. For instance: (a) partnering with the Centers for Disease Control and Prevention and the region's hospitals on central line-associated bloodstream (CLAB) infection project that resulted in a 68% reduction in CLAB's; (b) a regional cardiac

arterial bypass graft (CABG) improvement project that showed initial, highly variable outcomes among cardiac centers and individual surgeons, and then engaged the surgeons in creation of a regional cardiac registry that was followed by lower mortality and complication rates.
Current PRHI-sponsored quality improvement projects that we believe respond to CER priorities are described below very briefly. Please note that these projects are organized both to demonstrate effective quality improvement approaches and disseminate them within local (outpatient and inpatient) care networks and the entire region. We believe that further regional demonstrations could build on these projects to advance CE knowledge.

- Chronic Obstructive Pulmonary Disease (COPD)/Readmissions Project. This is an ongoing project that engages two local hospitals and several affiliated primary care practices that admit COPD patients to them. Readmission rates among chronically ill individuals are a significant problem, and the 30-day COPD readmission rates at the participating hospitals exceeded 25%. As a result of improved transitions of care from hospital to community, and augmentation of primary care with deployment of a care manager (including home visits), significant reductions in readmissions are being achieved. Interim success is sufficiently dramatic and unequivocal that the region's largest commercial insurer has already made performance improvement payments to the participating hospitals. There is an excellent opportunity for implementing the COPD project model across the region and adapting it for other chronic diseases with high readmission rates. This could be designed and replicated by comparing hospital experiences when different hospitals with demographically similar service areas are designated for intervention/no intervention.

- Integrating Treatment in Primary Care Project. This is a recently initiated, privately-funded project that engages five community health centers. The goal is to demonstrate patient-level, practice-level and system-level outcomes through evidence-based practice for patients with chronic disease and co-morbid mental health/substance use conditions. Many chronically ill patients have co-morbid medical problems, which are often exacerbated by co-occurring behavioral health problems, including depression and hazardous or addictive use of legal and illegal substances. These problems correlate with high hospital admission and readmission rates, and much higher cost. The project goal is to demonstrate significant reductions in 30- and 90-day readmission rates through training of primary care staff in effective screening methods for depression (IMPACT depression intervention) and substance use (SBIRT - Screening, Brief Intervention, Referral and Treatment). If successful in proving better patient outcomes and return-on-investment, PRHI will seek to disseminate and reimburse for these interventions throughout the regional healthcare system. A meaningful project would compare FQHCs' patient outcomes when SBIRT and IMPACT are implemented, as contrasted with outcomes in non-participating FQHCs or outcomes within the experimental sites, pre- and post-interventions.

- Polypharmacy and Medication Reconciliation. This is a recently completed series of demonstrations, the results of which made such a compelling case for widespread action that a follow-up project is contemplated. Through its "Healthcare Champions" program, PRHI recruits teams of caregivers to conduct explorations of important patient care issues. The most recent iteration, organized as "Pharmacy Agents for Change," engaged

8 teams of clinical pharmacists in related studies of polypharmacy and chronically ill patients. The teams documented medication error frequency, lapses in care coordination and transitions of care, and patient falls among chronically ill patients. Design and implementation of specific interventions demonstrated the benefits of physicians, clinical pharmacists, and nurses collaborating on systematic medication documentation and reconciliation (med rec). The project also raised awareness of polypharmacy issues among providers and changed prescribing practices. Results point to the potential for reduced medication cost, lower utilization of high-risk polypharmacy combinations, and fewer adverse drug reactions and patient injuries, that could be proven through a rigorous RCT. Such research could demonstrate the ROI for med rec in reducing hospital admission and readmission rates and average length-of-stay, and improving patient quality of life and functionality.

- Improving Chronic Disease Care by Small Primary Care Practices and Safety Net Providers. This is a series of recently initiated and impending PRHI projects. PRHI's overarching goal is to prove the feasibility of developing formal or informal local accountable care networks, through which community hospitals and affiliated/aligned primary care practices would share electronic patient information, enable primary care provider direct access to hospital specialists, and coordinate care in both the inpatient and outpatient settings. The goal is show that through such local accountable care networks patient outcomes can be improved, particularly among high-risk patients. All three projects promise financial rewards for delivering more effective care, which could be measured by savings accrued through reduced hospitalizations.

Our unifying premise is that trends in healthcare reimbursement and outcomes-based performance measurement will require that many/most community hospitals and primary care practices re-invent themselves and their relationships. Community hospitals are already under significant financial stress, and inevitable changes in healthcare payments make it untenable for most of them to rely financially on filling beds. The small practices that admit 90+% of patients to community hospitals are also under significant financial stress, lack resources to make needed changes, but will be penalized if they do not adopt EHR's, etc. These hospitals and the practices that admit to them, however, have an opportunity to transform themselves into high-quality, efficient networks for both primary care and acute episodes of care. We propose that any of the following projects be enhanced, tracked and standardized to prove the relative effectiveness of aligning payment with desired outcomes (reduced ER visits, hospitalizations and re-hospitalizations):

1. CMS EHR Demonstration Project, for which southwestern Pennsylvania is one of four sites, and through which 140 small primary care practices will be able to earn substantial amounts for EHR implementation and attainment of CMS chronic care quality measures. Because EHR costs are only part of the challenge for small practices, PRHI and Highmark Blue Cross Blue Shield have formed a strategic

partnership to provide $1 million in customized staff training, technical help and business management assistance for participating practices.

2. Regional Chronic Care Demonstration Project, sponsored by the Pennsylvania Governor's Office of Health Care Reform, through which two dozen small primary care practices and safety net clinics will receive financial incentives for participating in PRHI-coordinated practice coaching, collaborative learning, and patient registries which will enable participants to implement the Chronic Care Model.
3. Transforming Safety Net Clinics into Patient-Centered Medical Homes, a collaboration with the Commonwealth Fund, through which PRHI will work with 12 federally qualified health centers to transform them into Patient-Centered Medical Homes (PCMHs).

In conclusion, we believe that research and dissemination of research findings at the regional level is unequivocally essential to realizing the quality improvements and returns-on-investment that are integral to CER success. As described above, PRHI projects are continuing to bring about relevant, measurable advances at the regional, state, and national level. We hope that significant funds will be set aside for regional research and dissemination projects and look forward to working with you and others.

Submitted by
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American Society of Health-System Pharmacists
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Good afternoon. My name is Cynthia Reilly and I am the Director of the Practice Development Division at the American Society of Health-System Pharmacists. ASHP represents 35,000 pharmacists, pharmacy students, and pharmacy technicians who practice in hospitals and health systems. Pharmacists' expertise in medication use ensures that drug therapies are used safely, effectively, and in a cost-conscious manner. I appreciate the opportunity to present the Society's perspective on comparative effectiveness research, or CER, to the distinguished members of the Federal Coordinating Council for Comparative Effectiveness.

ASHP is a member of the Alliance for Better Health Care, which advocated for inclusion of comparative effectiveness research funding within the American Recovery and Reinvestment Act of 2009. The Society is also publisher of *AHFS DI*, a comprehensive, independent reference on the clinical use of medications, which is recognized through federal legislation under Medicare Part B, Medicaid, and Medicare Part D as an official compendium. For over 50 years, *AHFS DI* has followed sound and high-quality editorial processes to synthesize complex evidence for dissemination and use by a broad range of stakeholders, including prescribers, pharmacists, individuals who make health-policy and coverage decision, patients, and others. ASHP believes there is significant need to compare the effectiveness and safety of specific drug therapies within pharmacologic classes, drug therapies within different classes, and drug

therapies with other treatment modalities. *AHFS DI* can serve as a foundation for medication information to support CER and ASHP looks forward to participating in this research. Today, ASHP requests that the Council consider three CER recommendations related to health care delivery systems that represent critical information needs to improve patient outcomes:

- Optimal practice models for delivery of patient care,
- Strategies for using IT-enabled decision support for delivery of CER, and
- Best practices for disseminating and implementing CER.

As described by the Dartmouth Atlas, the quality and cost of care is inconsistent across geographic regions, with much of this inconsistency attributed to variation in the care setting and the health care professional that provides the service. Under Medicare Part D, pharmacists provide medication therapy management services that include formulating medication treatment plans; monitoring and evaluating patients' response to therapy; performing medication reviews to identify, resolve, and prevent medication-related problems; and coordinating and integrating MTM services within the broader health care services provided to patients. Pharmacists also participate in chronic disease management and prevention activities under collaborative practice agreements with physicians. MTM programs and published research have demonstrated that pharmacist management of disease and drug therapy significantly improves patient outcomes, while reducing overall health care costs. However, there is limited research that directly compares this practice model to models in which care is provided by other health care professionals or interdisciplinary teams. ASHP believes such research would demonstrate best practices and strongly recommends models of care as a research priority for CER.

A critical element of CER is ensuring that research findings reach the point of care where clinicians, together with patients, can use the information to make informed treatment decisions. Electronic health records and other technologies are expected to provide point-of-care information; however use of these technologies is currently limited, as described in a recent *New England Journal of Medicine* study that found less than 2% of hospitals have fully implemented an electronic health record with clinical documentation, test and imaging results, CPOE, and decision support. Decision support technology has great potential to deliver CER findings, but strategies for creating and integrating these programs within other technologies, as well as barriers to implementation, are not well-studied. ASHP encourages the Council to support research that compares approaches for using clinical decision support and other technologies in the translation and implementation of CER.

In addition to technology, other effective dissemination and translation techniques are needed to ensure that CER findings are used to make informed decisions that improve patient outcomes. However, there are significant challenges in these activities. It has been estimated that there is a 17-year lag time between evidence generation and its widespread implementation. Many strategies have been used to enhance the rate and extent of adoption of evidence-based best practices, including clinical guidelines, continuing education for health care professionals, patient education tools, and most recently, academic detailing. However, these approaches are not well studied and results are variable. ASHP would encourage the Council to support research that compares the benefits and limitations of each approach in order to determine the strategy, or combination of strategies, that facilitates use of CER by each audience, including clinicians, patients, and payers.

Finally, based on ASHP experience as a drug information publisher, we encourage the Council to consider that, similar to drug information, CER research and dissemination efforts are not single events, but rather an ongoing process that requires sustainable and ongoing effort to ensure the currency and usefulness of the information as evidence evolves.

ASHP appreciates this opportunity to provide recommendations for CER. Along with our members, we look forward to collaborating with the Council and others to ensure that CER is not only useful, but also disseminated to clinicians, payers, and patients and subsequently translated into practice.

Submitted by
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Please support the building of a universal health information network (uhin). In order to benefit from medical and health experience we should have uhin to quickly and efficiently evaluate health threats and health interventions. The uhin may also be used for administering the financing of health care and cost/effectiveness evaluations in a global context. The Kaiser Family Foundation surveys now show the majority of the public acceptable to health information networks and the possible privacy risks.
http://www.npr.org/templates/story/story.php?storyId=103458129 .

To develop the uhin efficiently the Federal and State governments with International cooperation should develop a publicly endorsed monopoly or authority to direct vendors and health care providers in enforceable acceptable standards and pay vendors for use of their expertise. The uhin may also be used as a single virtual payor with all payors having access for an administrative fee.

For more please see my web site http://pages.prodigy.net/pcgioia and book **The Way: Knowledge Balances Territory and Compassion** available at www.Amazon.com or available as a 800kb document file on request.

Submitted by
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Dear Council Members,
Comparative effectiveness research should include estimates of population level effectiveness as well as patient-level effectiveness. A growing body of research offers evidence that the same biologically efficacious treatment may be associated with increased or decreased disparities and/or increased or decreased mortality rates in different geographic areas. From the older literature, there is evidence that introduction of Sabin vaccine transformed poliomyelitis from a disease that predominantly affected whites to one that predominantly affected blacks (1,2). A similar problem, not so clearly related to access to care, led to intra-city disparities upon release of measles immunization in the divided community of Texarcana. Because of structural community inadequacies, the Texas side of the city was unprepared to translate research on measles immunization into practice while the Arkansas side of the city was ready. If "effectiveness" studies had been done only on the Texas side (or in communities like the Texas side), there might have been concern that the vaccine, though efficacious, was not effective. In fact, this is one reason the Texarcana study was done. Landrigan's research showed that biological efficacy or even effectiveness did not predict public health effectiveness (3). More recently, it has become clear that Medicare's decision to reimburse providers for screening mammography (4) as well as the introduction of HAART for HIV (5) and surfactant for neonatal respiratory distress (6) were all associated with increased disparities in most, but not all communities. Preliminary studies on breast cancer using Medicare claims data suggests that communities that appear to be successful in delivering mammography to both black and white elderly cannot be characterized by county-level estimates of the availability of medical resources or socioeconomic status. In summary, comparative effectiveness research that ignores public health success or failure, and public health comparative effectiveness research that ignores variation in community capability for translating innovation into practice could both lead to alpha- and beta- errors in effectiveness estimates.

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Submitted by
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Dear Sirs,

The disease specific focus of much of the proposed Comparative Effectiveness health services research to date risks a serious deficiency in both the impact and generalizability of the resulting work. In particular, those of us doing research in patient care quality and safety are concerned that the proposed clinical domain/disease focus will hamper major advances in our efforts to reduce medical error, enhance the design and impact of healthcare information technology, and improve quality. Moreover, the targeting of specific clinical topics means that other domains/topics will be excluded from priority consideration yet some of these may be superior laboratories for the initial evaluation of quality/safety/informatics interventions that ultimately will have broad-reaching impact.

I implore you to consider adding comparative effectiveness priority research areas to include critical cross-cutting research questions (e.g., clinical decision making, human-technology partnership, team coordination and continuity of care) and evaluation of general intervention/improvement methodologies (e.g., simulation-based training and assessment, computer-based clinical decision support).

I would be happy to discuss these concerns with you further.

Submitted by
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Independent Drug Education and Outreach
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The Problem

Currently, pharmaceutical sales representatives are one of the most common ways doctors receive information about the latest drugs on the market. However, most of the information they provide is designed to market their company's products, rather than serve as an unbiased source of data about the range of pharmaceutical treatments available to patients. With evidence showing that interaction with pharmaceutical sales representatives can impact doctors' prescribing patterns, it is important to ensure that physicians have access to independent information that is well-researched, comprehensive, and objective.

The Solution

Academic detailing programs send trained pharmacists, nurses, and other health care professionals into doctors' offices to disseminate independent data about the benefits, risks, costs, and comparative effectiveness of the full array of pharmaceutical options for patient treatment, including low-cost generic alternatives.

- **Fund grants or contracts to develop educational materials.**
  - The grantee or contractor would develop educational materials showing the relative safety, effectiveness, and cost of prescription drugs, including generic and over the counter alternatives and non-drug treatments for selected conditions. These materials would include brochures, handouts, and electronic information accessible to both patients and doctors.
  - Entities that can demonstrate clinical expertise in pharmaceutical research, such as medical and pharmacy schools and academic medical centers, would be eligible to apply.
  - Applicants may not receive financial support from any manufacturer of the drugs being reviewed.
  - AHRQ will review and approve the accuracy and effectiveness of the materials on a bi-yearly basis.

- **Fund ten grants or contracts through AHRQ to dispatch trained medical professionals into physicians' offices to discuss and disseminate the unbiased educational materials.**
  - Public entities and nonprofit groups would be eligible to apply for the grant or contract, as would other entities that demonstrate the capacity to train and deploy the medical professionals to disseminate and discuss the materials.
  - Applicants may not receive financial support from any manufacturer of the products being discussed.

- The grant or contract recipients would hire and train appropriate staff, identify health care providers to be the recipients of the outreach, and evaluate the effectiveness of the program on both cost and prescribing behavior.
- Regulations would also be in place to ensure the accuracy and timeliness of the information being distributed, to prevent conflicts of interest, and to promote the effectiveness of the program.

The Cost

There is documented cost savings in the states that already have academic detailing programs in place. Analysis of a program in Pennsylvania found that drug expenditures for a single class of drugs were reduced by roughly $120 per doctor per month for patients in the state program. Among the heaviest prescribers, the reduction was $378 per doctor per month for just one class of drugs. A study in the New England Journal of Medicine projected that for every dollar spent on academic detailing, two dollars can be saved in drug costs. This kind of academic detailing program would likely pay for itself and create additional cost savings for the federal government, private insurers, and patients. Most importantly, it will help ensure that patients receive the most appropriate, highest quality treatment.

Written statement of the Association of periOperative Nurses (AORN)
Patrick Voight RN BSN MSA CNOR; President
Linda Groah RN MSN CNOR FAAN; CEO

Thank you for giving AORN the opportunity to address the Council on the very timely and important issue of comparative effectiveness. We want to emphasize three points:

1. Data collection from **nursing sources** is a critical element to inform **effectiveness** decisions;

2. **Standardized data** available from the point of patient care is essential to inform **comparative** analysis.

3. **Syntegrity** creates an opportunity for a national surgical data repository that could be used to measure quality outcomes and potentially be used for comparative effectiveness studies to decrease cost and improve quality.

Since the 1980's the Association of periOperative Registered Nurses (AORN) has been a pioneer in developing and promoting the Perioperative Nursing Data Set (PNDS), a standardized language for documentation and evaluation of the care provided in the operating rooms of our nation's hospitals and ambulatory surgery centers. As a 501(c)(6) association based in Denver, AORN represents over 43,000 registered nurses and has a history of patient centered safety and quality activities.

The guiding premise of the PNDS effort was to assist perioperative nurses in documenting the care they gave before, during and after the surgical procedure, while providing a foundation for examining and evaluating the quality and effectiveness of that care.
While effectiveness within a hospital or surgery center was informed by this data, the ability for comparison between providers was rarely available because each facility customized the PNDS to their environment.

In early 2008, AORN initiated the development of an electronic and standardized perioperative framework referred to as Syntegrity.

This standardized perioperative framework is not intended to replace current information and documentation systems, but is designed to be incorporated into existing software. Most hospital surgical IT vendors already license AORN's PNDS but again, this is often customized for a specific facility. With the emphasis on electronic and standardized data collection coming from the federal government, the Syntegrity framework is poised to fill that emergent need. AORN is acknowledged nationally as the association that establishes evidence based standards and recommendations for care of the surgical patient. Syntegrity incorporates these standards and recommended practices into the database. Thus, Syntegrity creates an opportunity for a national surgical data repository that could be used to measure quality outcomes and potentially be used for comparative effectiveness studies to decrease cost and improve quality.

Here is a concrete example of how Syntegrity could be helpful for infection prevention: Syntegrity includes current CMS requirements for documentation on infection prevention processes. This "pop up box" requires the nurse to document from the choices in the electronic field or provide an explanation for any exception. This standardized, electronic data capture now becomes available for a repository from which specific analysis may be performed to measure the effectiveness of the CMS requirement. It is important to emphasize that the data collection by the nurse is already part of most surgical processes – what Syntegrity does is STANDARDIZE the data collected in an ELECTRONIC format . This creates an opportunity for a national surgical data repository that could be used to measure quality outcomes and potentially be used for comparative effectiveness studies to decrease cost and improve quality.

As further guidance to the Coordinating Council on the important priorities of health reform that AORN believes are impacted by the consideration of comparative effectiveness, we provide the following information and specific AORN resources that are meaningful to this discussion.
Quality improvement must play an essential role in health care reform efforts ensuring reform not only expands coverage, but also improves the care patients receive.

- AORN standards and recommended practices are a key resource. AORN is acknowledged nationally as the association that establishes evidence based standards and recommendations for care of the surgical patient. Syntegrity incorporates these standards and recommended practices into the database.
- AORN's Perioperative Nursing Data Set (PNDS) is a standardized language that facilitates the documentation and evaluation of the care provided by perioperative nurses.
- AORN has embarked on an initiative to create a standardized perioperative framework (Syntegrity) that will be integrated into hospital and surgery center electronic perioperative information systems.
- The essential role of the RN as Circulator and the value added of the registered nurse first assistant underscore the role of perioperative nurses in achieving quality and may be informed with comparative effectiveness analysis arising from data made available through Syntegrity data repository.

Performance measurement is a core building block to provide high quality affordable care. Information that is grounded in good evidence will support quality improvement, payment

reform, and enable better clinical and consumer decision-making. This information can tell us which care is leading to better outcomes and which treatment options are more cost effective.

- AORN supports the National Priorities Partnership convened by NQF
- AORN supports Stand for Quality

Investment in **health information technology** should be linked to improving care. Health information technology (HIT) represents an important means of advancing quality measurement and improvement. But HIT can only help improve the quality of care if it is designed to more effectively collect performance information.

- AORN Syntegrity provides a standardized perioperative framework (SPF) that will be integrated into hospital and surgery center electronic perioperative information systems to provide evidence for quality improvement in the operating room.
- Syntegrity creates an opportunity for a national surgical data repository that could be used to measure quality outcomes and potentially be used for comparative effectiveness studies to decrease cost and improve quality.

Performance measurement must be dramatically expanded, but measurement is not enough. Expanded efforts on all fronts to foster greater use of **performance information to support clinical improvements** and the delivery of more cost effective care, expand public reporting, and expand the use of performance information to promote changes in payment to promote value.
In conclusion, we want to emphasize three points:

- Data collection from **nursing sources** is a critical element to inform **effectiveness** decisions.

- **Standardized data** available from the point of patient care is essential to inform **comparative** analysis.

- **Syntegrity** creates an opportunity for a national surgical data repository that could be used to measure quality outcomes and potentially be used for comparative effectiveness studies to decrease cost and improve quality.

For further information from AORN or to set up an information briefing, contact Craig Jeffries, AORN Public Policy Consultant at CJeffries@AORN.org.

Submitted by
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Since our founding in 1992, the work of the Center for Advancing Health (www.cfah.org) has been guided by three principles:

- That scientific **evidence**, while always evolving, offers the best guide for positive changes in the health of the individuals and the nation.
- That individuals, sick or well, will only benefit from the expertise of health professionals and available technologies if they have the knowledge, skills, judgment and willingness to **engage** in their health and health care over time.
- That minimizing the social and behavioral barriers to people's engagement in their health and health care will contribute to **equity** of opportunity for health for all.

Determining priorities for comparative effectiveness research (CER) comes at a time in our nation of great uncertainty, but also a time of enhanced scientific and political opportunity. At the CFAH, we acknowledge the complexity and significant challenges that this coordinating council faces at this critical time in our history.

Rather than nominating one condition over another (which is outside the core expertise of the CFAH), we simply offer that priorities for CER should be on high volume and/or high cost conditions for which there exist significant variations in practice AND multiple treatment or diagnostic options. Research priorities and methodology should also factor in any systematic variations in disease prevalence or treatment response across different populations, as well as consider known health disparities in treatment provision.

It is our belief that substantial efforts must be expended to build public interest in and support for CER. These efforts should be focused on communicating the value of and application of CER for everyone's health and health care. It is, therefore, our primary recommendation that your effort to advance public understanding of CER and even more critically, develop TRUST in the value and output of an institute(s)/body(ies) devoted to CER, be as important as a debate about "who's on first" with regard to selecting priority areas of study.

At the CFAH we recognize that as a society we have often been lulled into believing that new scientific discoveries, that "wonder pills" and technology are the keys to living well. And that more treatment and/or more costly treatments are frequently equated with quality. This expectation and preference for the latest, often "high-price" option means that apparent advances in the number and variety of treatment options creates both a solution AND a problem. However, we also know that advances in scientific knowledge can only increase health and quality of life IF people are able to make informed decisions about their health care. In addition, people must be willing to change life-long habits and manage complicated medical regimens.

The success or failure of modern medicine is increasingly dependent on an individual's ability to engage more fully in their own health.

For example, here are some observations of our president and founder, Jessie Gruman, when she conducted over 200 interviews with patients and their families about their experience with health care for her book *AfterShock, What to do When the Doctor Gives You - or Someone You Love a Devastating Diagnosis*. She learned that the vast majority of them were surprised at what they were expected to know and do, and overwhelmed when they grasped (however dimly) that their actions and their choices could make the difference between receiving good care and bad and could even contribute substantially to the quality and even the length of their lives. She also learned that they often felt abandoned in their attempts to find the right care and administer it for themselves or their loved one. Surprised, overwhelmed and abandoned…not exactly criteria associated with making sound decisions. If CER is to meet its potential, we must address the realities and concerns of an already compromised patient.

So we would like to offer five potential strategies to engage the public:

1. Align early and often with trusted public advocacy groups and spokespersons to disseminate basic information about the need for and the value of CER.
2. Institutionalize the participation of consumers and patient advocates in the reviews and dissemination of findings.
3. Be fully transparent about the selection and study process for treatment reviews.
4. Make all findings directly available to the public in accessible formats.
5. Share potential outcomes and/or consequences of CER reports in various "real-life" scenarios for the average patient and physician (downside and upside from each perspective).

Lastly, it is of great concern to the CFAH that opponents of CER have grabbed rhetorical high ground with negatively framed language specifically designed to frighten people. Thoughtful perspectives from a variety of trusted sources are urgently needed to provide the public with a more balanced understanding.

Thank you again for this opportunity to add input to your important process.

Submitted by
David Thomas Martella
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Our medical system has failed. This problem doesn't touch one person, or ten, or one hundred, this problem touches every citizen of The United States of America. This problem has probably touched all citizens in one way or another, either as a victim, or friend or family member of a victim. The hospitals and the doctors they employ have either killed or maimed someone the reader of this letter has known. That is how far the government, insurance companies, and "we" the citizens, including all employees of the medical system, have let this happen right in front of our eyes without any action, and with what little action already taken, not the best results.

I have had the same doctor who told me they are sorry I couldn't get justice also tell me that doctors won't testify against each other.

There are bills being passed in the "billions of dollars" range to help with our medical system. My broken left leg has already cost the government almost fifty thousand dollars and will continue to cost the government through my upcoming amputation. The amputation is due to an infected rod (pseudomonas aeruginosa, coagulase negative staphylococcus) that was placed into my lower left leg, tib-fib break that never broke the skin. The only time my skin was open was in the operating room at Skyridge Medical Center in Cleveland, TN. The operation was done by Dr. Rickey Hutcheson. I filed several complaints with The TN Department of Health, (case #'s TN00021037, 200802582, 200802547), with no action taken so far. The hospital has already been let off the hook by a "surprise attack investigation" which never included me, you're welcome to get a copy of that review at the East TN Regional Office, the last two complaints mentioned have been handed over to another office, the Office of Health Care Facilities, for whatever action they deem appropriate. This means no action will be taken. If you multiply the fifty thousand dollars already spent on me times just one percent of our population on the same insurance I'm on (Medicare) the cost to the government is now around one hundred and fifty billion. Why can't these doctors be held accountable for that money? Why is the government gladly and generously paying for their mistakes?

If someone doesn't pay their child support the government threatens to take away their drivers license and put them in jail. If someone has a dog and it bites another person, the government arrests the dog owner. Where's the involuntary manslaughter or reckless endangerment charge for the medical field. The government of The United States of America has made "murder" a common word for the common people to have to deal with when it comes to the problems with hospitals or doctors. The government of The United States of America has made it legal for hospitals and doctors to get away with murder and reckless endangerment cases pertaining to their patients. Where's the justice in that? I'll answer that for the government, since they aren't brave enough to stand up and do the right thing. There is no justice in these situations, but you, the government, doesn't care. Do you think the Founding Fathers of this country would have stood for this? Remember, "No Taxation Without Representation". Do you think Abraham Lincoln would have stood by and let this happen? He thought to much of every citizen alike to let this happen to any of his people, but now, you answer these questions because you, the government of The United States of America, deep down in your heart and soul know the answer, whether you say it out loud or not. The lawyers of this great country are doing more right now for the rights of all citizens than the government. The lawyers know there can and should be no caps on pain, suffering, and death, and we appreciate that.

The Malpractice Laws are designed for monetary compensation from the hospitals and doctors to the patient for the services the patient received when something went wrong. It's to late for an apology and a set amount of money at this stage. We're tired of the way the government allows and the doctors and hospitals insurance companies having the power to control the "buy out system" the hospitals and doctors go through for their unprofesionalism. Where is the retribution in that system? There are enough doctors going to school that would be glad to take over the offices on Main Street of the unprofessional and unethical doctors running the medical field. Even politicians seem to get into more trouble than doctors. Why?

The doctors try to fix problems, but most of them really don't "help" anyone when a large percentage of patients are leaving sicker than when they came to the hospital. Then, most get a "second opinion doctor" who tells the patient what the other doctor did wrong and has to spend their valuable time fixing botched up work from another doctor. That's how it was worded in my case. Well, the United States citizens will no longer stand by and let this happen. Instead of caps and apologies, I think it would be better to assure the population as a whole that the hospitals and doctors WILL be held accountable for their mistakes, instead of patients dying or being maimed by our medical system. Also, it costs the common people (in my case, one thousand dollars for a medical review of my file from a specialist in a contingence state) money that a large percentage doesn't have. I had to get a loan to start a medical malpractice lawsuit. That is unfair, and the government knows this is true.

Justice is what the common people long for, the government of The United States of America stands for, and what the politicians on Capitol Hill are withholding. What went wrong? Maybe, no one in Washington D.C. is looking at this problem from the right angle. The solution to this problem is not to condemn the hospitals or doctors of this great country, the solution to this problem should give all citizens alike the confidence of their medical system and to give the medical system back its reputation in which it has strayed. I don't want our future generations to go through what we have HAD to go through. Should they "inherit" this problem?

This is corruption at its best. The insurance companies of the hospitals and doctors are controlling this situation, and you (the government) know it. Is that what they're talking about when they say "monopolize"? Well, if so, the government of The United States of America is allowing this to happen. Is it like this in other countries, let's say, with "socialism"?

Stand by me, as two warriors should, and help the hurt, infected, dying, and families of the dead, etc., citizens of The United States of America not be the only ones being held accountable for the infected rods, bad joint replacements, etc. We were just the ones putting our trust in hospitals and doctors who are fouling up, the patients were just innocent bystanders laying on the hospital beds while the doctors were performing the operations.

Thank you for your time and attention.

Submitted by
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Good afternoon. My name is Gary Persinger, Vice President for Health Services Research at the National Pharmaceutical Council (NPC). On behalf of NPC, I would like to thank you for providing this opportunity to comment on comparative effectiveness research (CER) and the activities of the Coordinating Council. This is a critical discussion focused on providing health care decision makers with timely, balanced, and high quality clinical evidence to help inform their decisions and improve patient health outcomes.

About the National Pharmaceutical Council
Briefly, the National Pharmaceutical Council sponsors and conducts scientific analyses on the appropriate use of pharmaceuticals and the clinical and economic value of improved health outcomes through pharmaceutical innovation. CER and its foundation of high quality scientific evidence are important areas of focus for NPC. It is our goal to ensure that sound evidence is recognized by independent experts, considered appropriately by private and public payers, reflected adequately in benefit designs, and incorporated into clinical practice. NPC was established in 1953 and is supported by the nation's major research-based pharmaceutical companies.

Chronic Diseases Afford Greatest Impact
It is clear today that health care costs are rising at an unsustainable rate, making it reasonable for CER priorities to focus on medical conditions with the greatest impact on morbidity and cost. These include chronic conditions such as cardiovascular disease, chronic respiratory diseases, cancer, diabetes, arthritis, and serious mental health conditions. CER should not be limited to the drugs used to treat those conditions, but rather, it should be extended to all relevant health care services including medical and surgical procedures, diagnostics, and medical devices.
In addition, this research should include alternative health care delivery methods and insurance benefit designs. The proposed prioritization of research topics and studies in these areas of medicine, their associated research time frames, final study outcomes, and related information should be made transparent to all stakeholders and should be disseminated in a timely manner.

Key Additional Factors for Consideration
It also will be important for the Federal Coordinating Council to consider several key questions as it assesses the CER-related research conducted by Federal agencies and departments and develops recommendations for selection of the highest priority research.

- First, what strategies can be employed to ensure the continuous evaluation of new evidence related to specific health care technologies – for example, how best to determine when a health technology assessment should be revised based on new clinical information?

- Second, how can CER be employed optimally in a manner that preserves incentives for the continuous innovation of health care technologies in areas of unmet need?

- Third, how can CER at a broad population level be balanced with the goals and rapid scientific advancements in the area of personalized and stratified medicine in order to encourage the development of targeted therapies for subpopulations?

- Fourth, what research should be conducted to define rigorous, high quality, and validated CER methodologies that are focused on providing timely, accurate and balanced information in order to assist clinical decision making?

    - This research should include, but not be limited to, defining how best to address the full range of health effects of a new technology, including quality of life, functionality, and productivity, as well as how best to appropriately characterize the strengths, weaknesses, and limitations of various underlying health technology assessment analytic techniques.

- Lastly, what support is required for the development of new CER methodologies, such as analysis of non-randomized studies of treatment effects using secondary databases, practice-based clinical practice improvement studies, more accurate modeling and simulation techniques, and methodologies that ensure optimal interpretation and application of CER in a variety of patient care settings?

The National Pharmaceutical Council welcomes the opportunity to be a part of this critical dialogue and stands ready to assist the Coordinating Council as it moves forward in developing recommendations to ensure coordination and best use of resources for CER.

Thank you.

Submitted by
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BACKGROUND

Consumers spend millions of health care dollars annually on Complementary and Alternative Medicine (CAM) practices and therapies that have limited or no solid medical evidence base and which may interact adversely with existing treatments or even exacerbate existing medical conditions. No widely-accepted systematic and rigorous process exists for collecting safety and efficacy data on these untested CAM practices. The Scientific Evaluation and Review of Claims in Healing (SEaRCH) program addresses this national need through a well-documented,

transparent process for evaluating CAM practices and therapies. SEaRCH performs a systematic assessment of the methodologies, techniques, and outcomes reported for CAM practices and compares these to existing medical practices and methodologies. SEaRCH provides a rapid, cost-effective screening before time and money are spent on future research or evaluation of untested practices.

SEaRCH has grown through public and private partnerships over the last decade. The original SEaRCH concept began in 1996 with a mandate from Congress to document and evaluate CAM therapies and practices. In 1997, through collaboration with the Centers for Disease Control and Prevention (CDC), the Office of Alternative Medicine (OAM) at the National Institutes of Health (NIH) developed the Field Investigation and Practice Assessment (FIPA) program and conducted several large-scale evaluations of CAM practices. The FIPA program was extended in 2003 under the Congressionally-mandated CAM Research for Military Operations and Healthcare (MIL-CAM) program and was further developed as the Epidemiological Documentation Service (EDS) through a subcontract to the National Foundation of Alternative Medicine (NFAM). The EDS program was transferred to the Samueli Institute in 2008 where it was further developed and renamed SEaRCH.

The Samueli Institute is currently refining and expanding SEaRCH to enable more rapid throughput and assessment of CAM practices. SEaRCH fits well into the existing Samueli Institute research portfolio, which includes the Prospective Outcomes Documentation System (PODS) for conducting clinical observational studies, the Institute's Systematic Review program, and its capacity for conducting rigorous pre-clinical research through its network of laboratory partners.

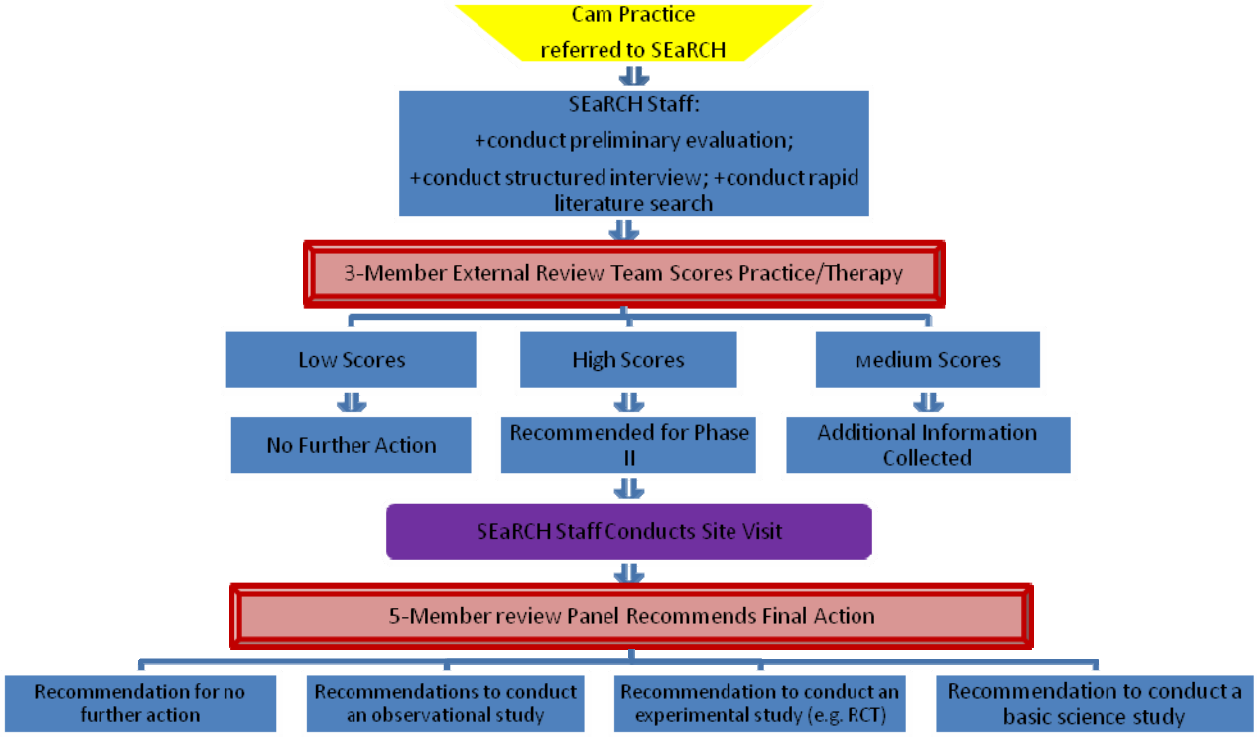
GOALS AND OBJECTIVES

In the highly competitive and resource-limited world of medical research, many potentially valuable CAM therapies and practices are often left uninvestigated. The **goal** of the SEaRCH program is to systematically and rigorously evaluate CAM practices, therapies, and claims worldwide to determine if they warrant further scientific investigation and research. The **long-term is goal** is to identify safe, effective, and affordable CAM treatments that warrant the time and effort for further testing and comparison against existing therapies. The specific **objectives of the expansion** of SEaRCH are to: further develop the methodology and toolkit for evaluating healing claims, build a team of scientific reviewers and partners, systematically identify candidate CAM practices, employ the methodology to prioritize and make recommendations to perform comparisons of CAM practices and therapies with existing therapies, and to communicate the initial findings from these evaluations to the public. SEaRCH methodology will be available for use for CAM therapy, practice and claims by CAM practitioners, the National Center for CAM (NCCAM), the office on Cancer CAM at the NCI, the CDC and the Department of Veterans Affairs - all of who have expressed a need for such a methodology and approach.

SEaRCH PROCESS

SEaRCH employs a rigorous two phase evaluation process: Phase I: Practice/Therapy data compilation and screening; Phase II: Therapy or Practice Site Visit and direct data collection and Final Review and Recommendations (See Exhibit).

When a therapy or practice is referred to SEaRCH for evaluation, Phase I begins with compilation of basic information and documentation. SEaRCH staff does a preliminary evaluation through contacting the principal investigator/therapist and identifies any missing data elements. SEaRCH staff then conducts a rapid literature search to amass data on comparison practices. The SEaRCH staff also conducts a structured interview with the principal investigator/therapist to obtain additional specific data on the history and current breadth of the practice reach. Phase I culminates with three external scientists/practitioners reviewing the amassed data using a rigorous scoring approach which supports the range of CAM therapies, practices, and procedures. Low scores in the Phase I screen lead to constructive reviews sent to the principal investigator/therapist and a recommendation of no further action. Mid-range scores lead to requests for additional information from the principal investigator/therapist and/or additional SEaRCH staff background research. Based on this new information, the practice/therapy may be reviewed again or recommended for no further action. High scores in screening are recommended for Phase II. In Phase II the SEaRCH staff performs a highly structured site visit. The data collected on the site visit is added to the practice/therapy file with the staff recommendation for action. The entire file is then evaluated by the same external team who conducted the screening review plus two new members. The review team is expected to reach consensus and write a comprehensive report to recommend a specific research protocol involving the practice/therapy or recommend no further action.



SUMMARY

SEaRCH is a systematic, rigorous methodology for evaluating unusual and novel claims for improving health. Through its iterative protocol driven process SEaRCH can critically evaluate and triage the multitude of claims for healing, and select the ones with the most capacity and promise to make a difference for community and global public health. The consumer medical and health services communities will benefit from SEaRCH because resources and efforts can be focused on the CAM practices with the greatest validity and most promise. An expanded rapid through put capacity will enable SEaRCH to widely serve the heath care community and become the standard approach for evaluation of CAM therapies.

Submitted by
John Lewis
Vice President of Public Affairs,
Association of Clinical Research Organizations
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Members of the Council, thank you for the opportunity to speak with you today.
My name is John Lewis. I am Vice President of Public Affairs for the Association of Clinical Research Organizations, ACRO. Our members have more than 70,000 employees who are involved in research in more than 60 countries around the world. Working primarily for pharmaceutical and biotechnology companies that sponsor clinical trials, ACRO companies perform a wide range of activities, from providing assistance with study design through regulatory submission, across the spectrum of clinical trials, from phase I first-in-human studies through phase IV post-market evaluations. In addition to clinical trials, our members' expertise includes: health services research; patient registries; safety surveillance and other public health activities; data management, analysis and reporting, biostatistics; and the topic at hand today, comparative effectiveness research.

I would like to make three points:

First, to generate the maximum impact from the research dollars allocated by the American Recovery and Reinvestment Act (ARRA), we should use as much currently available data as possible as the basis for comparisons between alternative treatments. This includes Phase IIIb and Phase IV studies that use active comparators, and are reported to the FDA today, as well as a wide range of other data sources; from electronic health record systems to health care claims databases, and databases of various government health plans such as CMS and Veterans Affairs. Special attention should be paid to the methods and standards used to aggregate, analyze and report this data. In allocating ARRA funds to this endeavor, priority should be given to organizations with a successful and demonstrable track record of working with large of amounts of data from disparate sources.

Second, when meta-analysis of existing data is an insufficient method to reach the desired research endpoint, new clinical trial designs are needed. As research organizations that specialize in complex trial design, we would be pleased to participate in any effort to establish the

methodologies and standards by which these trials are conducted and to carry out these important and complex trials.

Third, because expertise on CER resides in both public and private entities, every effort should be made to encourage public-private collaboration in the design, conduct, analysis, and reporting of CER. We believe such collaboration should extend to include research sponsors, patients, providers and other stakeholders. We realize that the composition of this Council was established by legislation and did not provide for the inclusion of industry representation. Nevertheless we urge the Council to seek the required expertise to carry out this research whether in private, government or academic settings.

ACRO currently participates in the FDA's Clinical Trials Transformation Initiative, the NIH Biomarkers Consortium and several other similar collaborations and we could envision a similar collaborative process working in this regard. In the Biomarkers Consortium, for instance, one of our members, Quintiles Transnational, is playing a lead role in statistical and data analysis on a project involving the review of existing clinical trials data for a specific metabolic disorder.

As global leaders in clinical research, ACRO members are well suited to aid in the design, conduct and analysis of CER. We stand ready to work with all the stakeholders – pharmaceutical and biotechnology companies, academic and other researchers, patient groups, prescribers, payers, and government agencies – in shaping and executing a CER portfolio that will promote continued innovation in drug development rather than limit it.
Thank you.

About ACRO

The Association of Clinical Research Organizations (ACRO) is the professional organization of companies whose focus is clinical research. The association provides an active voice for the CRO industry, which provides specialized services that are integral to the development of drugs, biologics and medical devices. ACRO helps its members improve the quality, efficiency and safety of biomedical research. ACRO member companies employ more than 70,000 professionals worldwide. For more information, please visit www.acrohealth.org

Submitted by
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I would like to thank all the members of the Council for providing me with the opportunity to address you today. As a physician-attorney who has devoted his career to improving patient safety and healthcare quality, I would like to discuss briefly the potential medical legal implications of the scientific findings deriving from comparative clinical effectiveness research.

The Potential Liability and Risk Management Implications Deriving from the Results of Comparative Clinical Effectiveness Research

Many physicians may find it controversial enough that the results of comparative clinical effectiveness research may impact their future reimbursement. This controversy could intensify if the results of these studies could be taken into account in liability actions, also.

What potential legal weight might the results of these studies have in liability actions? What would these studies need to have taken into account in arriving at their conclusions for a physician to be able to use adherence to them as a shield? What would need to be taken into account to permit the other side to use them as a sword against physicians for not following them (or even for following them resulting in a bad outcome)?

A close cousin to comparative clinical effectiveness research (controlled clinical trials) has carried varying degrees of weight in medical liability actions in the past. As the standard of care has slowly evolved from local determinations of "what would a reasonable physician have done under similar circumstances" to what is the recognized best evidence based practice(s) to employ, the results of these studies have acquired more heft in liability actions.

Comparative clinical effectiveness research will likely be somewhat similar in its evolution and probable applications in medical liability actions. What may well turn out to be different about comparative clinical effectiveness research are at least two things:

- Reimbursement (in whole or in part) may turn in the future upon proof of following the recommendations deriving from this research; and

- To get their results into the mainstream more quickly and cost effectively than the very expensive and time-consuming controlled clinical trials), comparative clinical effectiveness research may be conducted through the analysis of large electronic databases to link certain practice patterns with superior value clinical outcomes.

Especially in the case of studies whose results are produced by the analysis of large clinical databases (often derived from the use of electronic medical records), there would need to be certain safeguards built in to ensure fairness and accuracy in using the fact of adherence or non-adherence with best practices as a basis for liability or exoneration. Some of the factors that would need to be taken into account during these studies when applied to demonstrate potential negligence (or lack thereof) for not following their care recommendations would be:

- **Pre-treatment Severity of Illness**: Did the plaintiff in a particular action exhibit a comparable clinical condition and severity of illness to those in the study whose results are being introduced either as a sword by the plaintiff or a shield by the defense?

- **Statistically Significant Conclusions:** Were there enough patients in the samples comparing interventions to be able to conclude with an acceptable level of statistical significance that the variations in clinical outcomes were due to the differences in the interventions and not due to chance?

- **Impact of Other Clinical and Non-Clinical Factors:** Were there other countervailing factors occurring during the comparative effectiveness studies other than the interventions being evaluated (e.g. preventive measures, lifestyle changes, environmental influences, etc.) which were not sufficiently "teased out" of the analysis of the results so as to open to question the conclusions for recommending one specific intervention over another?*

As these comparative effectiveness studies begin to be conducted and their results disseminated, it will be important that physicians know the extent to which they may be able to rely upon them as an affirmative defense in medical liability actions. It will also be important for these studies to be carried out with the above considerations in mind to ensure that their results may be judged as fair and accurate to provide assurance that following their care recommendations will meet or exceed the standard of care.
I thank again all members of the Council for providing me the opportunity to make this statement to you on this historic day for those of us who have devoted our careers to improving the quality and safety of healthcare.
***The foregoing three bullet points have been excerpted from an upcoming online article to appear in the April, 2009 edition of "Risk Review" copyrighted by the Princeton Insurance Company (http://www.princetoninsurance.com)**

Submitted by
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Introduction and Summary
The primary message of this statement is the importance of including addiction and mental disorders in the scope of work supported by comparative effectiveness research. The basic comparative effectiveness question for these conditions is to understand the cost and quality implications to the overall health system of continuing to under treat both conditions in systems that are siloed and distinct from mainstream health and health care.

The role of comparative effectiveness research in reforming our health and health care systems depends in part on the scope of the research that is undertaken. This statement supports that the Council consider a broad scope of comparative effectiveness research. Specifically, the Council should consider the relative effectiveness of research on policy, the organization, financing and delivery as well as the prevention, treatment, and recovery of addiction and mental disorders. Including both addiction and mental illness in the scope of your consideration is essential to achieving the goals of health reform for two reasons. First, the prevalence of addiction and

mental disorders suggests that other health conditions and illnesses coexist in large segments of the population. Experience as well as empirical evidence suggests that positive outcomes for general medical and other chronic illness requires in part simultaneous treatment of addictive disorders. Second, the cost of untreated addiction for our health care system is significantly more than the cost of extending treatment for addiction disorders and the same holds for mental disorders.

Policy Research

While there are many public policies that impact addiction disorders, most are not health policies but found in such areas as criminal justice, housing, transportation, etc. The basic policy question for comparative effectiveness research is the impact of recognizing addiction disorders as preventable and treatable health conditions. Can we weigh the relative cost and impact of extending treatment to the 90% of 23.6 million people affected who do not receive any treatment, but represent more than 70% of people incarcerated in correctional facilities at state and county levels? For the 6% of adults with serious mental illness, is community based comprehensive care a more efficient, humane, and effective form of care than warehousing in county corrections facilities that result for example in Los Angeles County jail being the largest 'mental institution' in the US today. In this case, before we can look at the relative effectiveness of one treatment versus another it is essential to understand the relative effectiveness of investing in making treatment available through insurance and or public mechanism versus the effect and cost of leaving 90% of those with addiction disorders untreated.

Organization, Financing, and Delivery of Addiction Prevention, Treatment and Recovery Services

Others have noted the gap in health care that exists between what we know that works, and what is actually used to promote well being. Experience and some research suggests, that the latest evidence based interventions are underused, less often because of a lack of technical knowledge, and more often because the organization, delivery and financing of these interventions represent barriers to the adoption and use of proven practices. Understanding optimal designs for the organization, delivery and financing systems that promote the use of what science tells us works, is critical. For example, The Network for the Improvement of Addiction Treatment (NIATX) represents an empirically established approach to improving efficiency, eliminating redundancy, and streamlining systems that provide access to and delivery of effective interventions. The Council should consider the relative effectiveness of approaches to delivery system improvement and change as part of the scope of your work.

Preventing, Treating and Supporting Recovery

Empirical based interventions are available to prevent, treat, and support recovery from addiction disorders. These standards, backed by controlled and peer reviewed research, unfortunately compete with a wide range of practices, beliefs, traditions and philosophies used in many contexts to prevent, and treat addiction. The NQF standards should be a foundation from which analysis of addiction treatment interventions are examined. In addition, greater understanding is required for the relative prevalence and applicability of 'natural or self directed' recovery processes. Finally, while pharmacology and behavioral therapies together offer great promise for managing addiction disorders, their development and market use is hampered by perceptions of

'poor market potential' at this time. The comparative effect of accelerating the development of these and other new interventions is a critical area of investigation.

Submitted by
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National Hispanic Medical Association
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Chairman and Committee Members, I am Dr. Elena Rios, President & CEO, the National Hispanic Medical Association, a non profit association in Washington, DC representing Hispanic physicians. The NHMA mission is to improve the health of Hispanics and other underserved. The NHMA supports policies that will reform public health and medical services to decrease health care disparities and improve health status of Hispanics and other vulnerable groups.
The Unequal Treatment Report highlights the recommendations needed to decrease health care disparities – educate the leadership about health care disparities, diversify the health care workforce, expand cultural competence training, expand data collection with racial/ethnic and language variables and support research on the system's responsiveness to minority populations – access, utilization patterns, performance measures, innovation, and "collecting data on race, ethnicity, and language of preference is a quality of care as well as a civil rights issue."
Evidenced-based public health and medicine strategies are necessary to decrease variation of service delivery that impacts and rations care to Latinos, especially in our poor neighborhoods. We know from the annual AHRQ National Health Care Disparities Reports that our community has the worst access and quality care compared to non-Hispanics in the nation.
The U.S. is facing a tremendous growth of the diversity in the population. According to the Census Bureau, by 2042, one out of four Americans will be of Hispanic origin. We support the Obama Administration deliberations to help shift health care delivery based on increased services for acute and chronic disease to a systemic approach with integrated care in a region that is responsive to its population and focuses on prevention first. Medical treatment should be based on comparative effectiveness value of treatment strategies that produce the greatest benefit for the Hispanic community at the lowest cost.
We recognize that comparative effectiveness research is about value in health care. According to the report, HHS in the 21st Century, "assessments of value should include measures of both individual and societal costs and benefits that result from research on prevention and treatment as well as methods of organizing, delivering and paying for services."
Yet, despite this research being supported by the federal government over the past several years, we recognize there have been alarms sounded - the Congressional Black Caucus says beware of producing information for ' a one size fits all' approach that could decrease access to treatments for minority patients; and Amgen and Johnson and Johnson in the HHS in the 21st Century report cautioned that cost comparisons could lead to increased costs and rationing care.
However, we believe that comparative effectiveness research will add to the body of knowledge for reducing health disparities for 1) physicians to use to improve quality care for patients; as well 2) for administrators to use to improve health systems of delivery in the following priority areas:

1. Cultural competence and health literacy research in order to ultimately change behaviors and improve lifestyle in our communities

2. Effective ways of communicating with Hispanic patients and their families
3. Knowledge about health disparities interventions between hospital systems and clinics that have longstanding experience with Hispanic physicians and their patients
4. Innovative research targeted to Hispanic patients and their families
5. Integrated care that is outcomes based – and with mental health and oral health as well as physical health

Besides supporting comparative effectiveness research studies, there is a need to develop the mechanisms to share the research results with our minority provider community. HHS could lead the development of public private partnerships with Hispanic health care professionals and community based leaders about rewarding caregivers or showcasing providers who deliver high value care to Hispanics and to increase Hispanic physicians and others to participate in focus groups to develop mechanisms for information dissemination to providers in our communities. HHS should take this opportunity to develop Hispanic health professional researchers by targeting the untapped pool of Hispanic health professional students and residents and graduate students interested in serving in their communities.
Lastly, given the growth of the Hispanic population, there is a need to create regional areas for Hispanic health research and follow the health care decision-making in the health systems.
For more information, contact NHMA – 202-628-5895

Submitted by
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Who is proposing this research project?

The Frontier School of Midwifery and Family Nursing (FSMFN), American Association of Birth Centers (AABC), Duke Clinical Research Institute (DCRI) and Orases Consulting Corporation (OCC) have collaborated to develop a methodology for a study of optimal birth and an online registry of obstetrically uncomplicated pregnancy, labor, birth and postpartum/newborn care outcomes and the practices applied in achieving those outcomes. FSMFN is dedicated to educating nurse-midwives according to the best available evidence for collaborative practice in all birth settings. AABC is dedicated to developing a seamless experience for mothers within a system in which the institutions and care providers effectively collaborate to offer high quality, evidence-based maternity care. DCRI, consultants for the project, plan to establish the first registry on “normal” birth for comparative research by any researcher seeking such a data set. OCC, specialists in computer systems design, provide programming for the web-based data collection and reporting, system maintenance and technical support. The work to date has been conducted by volunteers with minimal funding from the AABC and FNS foundations for computer services.

Who will participate in this study?

All types of providers of maternity care services practicing in all settings are invited to participate in the proposal for a study of optimal birth. To date, 68 clinical sites that include midwife and physician collaboration in home, birth center and hospital sites have agreed to participate and over 22,000 childbearing women have been entered into the data base. When fully implemented, it is estimated that 200 sites will be participating.

The study instrument includes a profile of the practice and site, demographics of the mother, a description of the care that was provided, the outcomes of that care and the level of the mother's satisfaction with her care. It is available for review at www.birthcenter.org. Over the past four years the instrument has been developed and beta tested. A pilot study of five sites by a doctoral student at the FSMFN is currently under way to further test reliability and validity of the data. Funding for the full implementation of the project is being requested.

Why is this study important?

This study is needed to provide current information for policy decisions, removal of barriers to implementation of the midwifery model of care, and the importance of educating for collaboration within the professions and institutions providing that care. Although the outcomes and cost benefits of nurse-midwifery care in a variety of settings have been reported over the years, a current, comprehensive, prospective study of comparative practices that led to improved outcomes is lacking. This study is designed to provide current information on what constitutes optimal birth and how it may be best achieved.

The hypothesis is that optimal birth is best achieved when the mother makes an informed choice based on the best available evidence about her birth environment, primary care provider and family or other support personnel; participates in the decisions made about her care and medical intervention; and when midwives, obstetricians and nurses work collaboratively to focus on the individual needs of each woman and family they serve.

Background

Eighty years ago the Frontier Nursing Service (FNS) demonstrated that nurse-midwives could provide a model of care in a remotely rural, underserved area that was safe, satisfying and cost effective. In fact the care provided by FNS nurses resulted in such a dramatic improvement in maternal and newborn outcomes that the Metropolitan Life Insurance Company on analysis of the data recommended that the FNS model of care be adopted nation-wide. For the past eight decades, nurse-midwives, as primary care providers referring to obstetrical specialists as medically indicated, have continued to document improved maternal newborn outcomes at lower cost but midwifery has not yet been fully integrated into our health care system.
Now, well into the first decade of the twenty-first century The Cochrane Review of reliable research on maternity care world-wide, has recently reported that all women should have access to, and be encouraged to use, midwifery led units. Midwifery is the gold standard of primary care in the 29 countries with better infant mortality rates than seen in the United States.

Therefore it is relevant in this time of change to ask – why, when there is so much evidence supporting the health and cost benefits of midwifery care do we continue to educate so many surgeons and minimally support midwifery education while expressing concerns about rising Cesarean Section rates that increase costs without any significant improvement in birth outcomes? Why alternative measures for pain relief are not available to all laboring women such as hydrotherapy, choice of positions, or self administered Nitrous Oxide which is a proven safe, affordable and effective method of pain relief in labor that is being used in all settings in other countries across the globe but only available in the United States in a few teaching centers? It could be said that we have marginalized the basic needs of the majority of healthy women anticipating an uncomplicated childbirth experience by exposing them to routines designed for women with complications of labor and birth who need acute care services.

Policy implications for activating change

Workforce development

To educate a nurse-midwifery workforce in the United States we have relied on an adequate supply of nurses. Although enrollment in these education programs is up, we would benefit from offering to our unemployed young people a program like the Cadet Nurse Corp that was introduced during WW II under "the Bolton Act". It would serve to more rapidly prepare to meet projected demands not only for childbearing women for an aging population.

We should teach evidence based midwifery instead of obstetrics in all nursing and medical schools so that students receive an evidence-based orientation to the care of the majority of women rather than a disease and fear-based orientation to the minority of women experiencing this essentially normal life event. This is a better foundation for basic students making career choices for specialization.

It has become clear that nursing may not be the only pathway to midwifery education. To expand the midwifery workforce that this study will indicate is needed, education, certification and licensure for direct entry to the profession needs to evaluated, standardized and barriers removed.

Removal of existing barriers

We cannot produce midwives if we do not have access to clinical training sites. It is a strange paradox that we import foreign trained physicians to fill obstetric residency programs here to focus on the pathology and surgical intervention of child birth while midwifery students increasingly are sent to foreign lands for clinical experience. To bring the ratio of midwives to obstetricians in balance with the ratio of the estimated 80 percent of healthy women giving birth to the 20 percent needing medical or surgical intervention and to meet the staffing needs of clinical teaching sites, we must look at directing tax dollars to fund midwifery and obstetric residency programs to match the women served, residencies that include instructing these two very different professional groups to work together to bring their individual talents to the care of each woman served.

Autonomy in practice and payment for services

Autonomy in practice and equitable payment for services must be assured. To pay a midwife 65 percent of the payment afforded physicians for the time and education intensive care that

improves maternal and newborn outcomes is discriminatory. Midwives must be paid as any other licensed primary care provider and birth centers must be paid like any other health care facility. Midwifery-led units like birth centers should be included in the formation of the "health care homes". Midwifery and advanced practice nursing units established as a seamless part of a collaborative health care system would greatly improve access to quality care in both rural and urban underserved areas.

Conclusion

Although this represents a paradigm shift, there is no better place for beginning reform for efficient utilization of the health care system than providing midwifery time and education-intensive care to expecting mothers when they are most open to learning family health improvement measures. Mothers provide much of the primary health care to their families and are the major decision makers for when, where and to whom they will entrust their family's medical care needs. The cost of birth continues to rise. The current gap between maternity care practices and the available evidence is widening. Evidence-based collaborative midwifery and obstetrical care is an important part of the solution for reducing costs, expanding access to quality care and improving the birth outcomes. It must continue to be evaluated as it develops. A normal birth registry and a study of optimal birth is a step in the right direction for achieving that end. Without this paradigm shift, it is doubtful that the United States will ever reach its goal of optimal health outcomes for all mothers and babies.

Submitted by
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The Consortium for Citizens with Disabilities (CCD) believes that comparative effectiveness research is, and should continue to be, an important tool in helping patients and providers distinguish between the effectiveness of both existing and emerging treatment options. Having better evidence to support the clinical effectiveness of a wide range of health care interventions has the potential to lead to improvements in the quality of care and could potentially maximize the impact of the health care dollars spent in this country.

CCD, a coalition of national disability-related organizations, urges caution, however, to pursue comparative effectiveness research in a manner that does not lead to inappropriate restrictions in coverage of and access to assistive devices, therapies, treatments, medications, and long term services and supports for people with disabilities and chronic illnesses.

Many of the assistive devices, technologies, and therapies used by persons with disabilities to be functional and live independent and fulfilling lives have widespread application and are generally accepted by physicians and other health care professionals. Because many of these devices and technologies do not have to undergo the rigors of FDA review, the formal evidence base for such treatments may be less developed than other areas of health care.

With respect to all devices, therapies, and medications, it is important to recognize that disability conditions vary widely in severity and complexity. There are often multiple comorbid conditions in play and many disabilities are low prevalence, making specific and meaningful clinical effectiveness studies challenging to pursue. Ethical questions and other factors often make double-blind clinical trials in this area non-viable. Even well-grounded research on the general population can be easily misapplied to the disability and chronic illness populations, especially persons with intellectual, behavioral and cognitive disabilities.

In fact there are many potential applications of comparative effectiveness research to areas other than acute care medicine such as long term services and supports for these populations. It is critical that the outcomes of such research are not misapplied or used to broadly establish coverage rules that trump an individual's circumstances and specific needs.

For these reasons, the CCD believes that comparative effectiveness research is not a substitute for the clinical judgment of the physician or health care professional in consultation with the patient. In the absence of double-blind clinical studies, due consideration should be given to reliable observational studies and consensus medical opinion, along with the clinical judgment of the health care professional. This is particularly important for people with complex, disabling, or chronic conditions.

Comparative effectiveness research is very important and has the potential to bring down health care costs over time by improving the quality of care. CCD supports the pursuit of comparative effectiveness research as long as there are meaningful protections to prevent inappropriate restrictions in coverage of and access to health care and long term services and supports for people with disabilities and chronic conditions.

Submitted by
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AstraZeneca Pharmaceuticals LP (AstraZeneca) is pleased to submit the following comments on comparative effectiveness research priorities in the American Recovery and Reinvestment Act to the Federal Coordinating Council (FCC) for Comparative Effectiveness Research (CER) in advance of the public listening session on Tuesday, April 14, 2009.

AstraZeneca is a leading global healthcare company dedicated to the research and development of new medicines in therapeutic areas including cardiovascular, gastrointestinal, oncology, respiratory, and neuroscience. AstraZeneca is committed to the discovery of drugs that will allow patients to lead longer, healthier and more productive lives, and to supporting scientifically robust research that improves the delivery of effective, high-quality care to patients. AstraZeneca is also committed to patient health; including helping ensure that patients have access to the most appropriate therapies in the most appropriate setting.

Healthcare reform is a top priority for our nation's policy makers. AstraZeneca believes that today's discussions – whether about healthcare coverage or healthcare costs - are important steps toward enhancing patient health and improving the quality of healthcare in the United States.

AstraZeneca believes CER, the comparison of one diagnostic or treatment option to one or more others, is an important component of healthcare reform. The goal of CER is to conduct or support research to evaluate and compare the clinical outcomes, effectiveness, risk and benefits of two or more medical treatments and services that address a particular medical condition. CER offers the promise of improving healthcare quality and outcomes by making it easier for patients and their doctors to choose the best treatment or treatments through evidence-based decisions. Yet, given today's financial challenges and political dynamics, AstraZeneca is concerned that CER could be used to deny coverage or reduce payments for interventions, thus limiting patient access to treatment options.

At AstraZeneca, we are committed to ensuring that patients and their health care providers have the best information available to support their decisions regarding treatment. In support of that commitment, we have been, and continue to be, engaged in CER activities. To ensure patients and their health care providers receive the most value from CER, AstraZeneca is proud to share the principles that guide our discussions related to this issue:

Outcomes First

Focus on improving individual patient outcomes rather than short-term, population-based cost control.

Drive, Not Limit Innovation

Encourage the development of innovative healthcare interventions and not be utilized as the sole rationale for product approval, coverage, pricing, or reimbursement decisions.

Research Across All Interventions

Employ rigorous, transparent research methodologies applied across the range of healthcare interventions and treatment modalities.

In addition, AstraZeneca believes:

- The current decentralized approach to conducting CER in public and private settings has yielded highly useful information in an efficient manner. If CER is centralized, it should be a public/private partnership, funded from both public and private sources, focus on the patient, and be distinct from any organization making coverage and policy decisions.

- Funds applied to CER should focus on diseases where there is a significant burden to the patient and the health system. For example, the impact of providing better care to patients with chronic diseases such as diabetes, asthma and heart disease, could be

profound if the breadth of research includes comparative benefit designs, prevention programs, delivery systems and medical and behavioral interventions.

- CER offers the promise of improving healthcare quality and outcomes by making it easier for patients and their doctors to choose the best treatments.

- CER, when focused on clinical-effectiveness and not short-term cost-effectiveness, will encourage the development of innovative interventions.

AstraZeneca thanks you for the opportunity to comment on CER. We look forward to continuing to engage in a thoughtful dialogue around this important component of the future of our healthcare delivery system in the US. If you have any questions, please do not hesitate to contact Christie Bloomquist at (202) 350-5545 or Christie.Bloomquist@astrazeneca.com or Brian Maloney at (202) 350-5542 or Brian.Maloney@astrazeneca.com.

Submitted by
Winifred S. Hayes, RN, MS, PhD
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Dear Sir or Madam:

The following is being submitted on behalf of Dr. Winifred S. Hayes, President and CEO of Hayes, Inc. Hayes, Inc. is a leading provider of evidence-based health technology assessments that serves health plans, government agencies, hospitals, health systems, and consumers. Hayes also provide consulting services to help these same constituents put our research into action to improve patient care and outcomes. Today, Hayes clients serve over 200 million consumers and patients.

Statement of Dr. Winifred S. Hayes:

To the Federal Council for Comparative Effectiveness Research:

You asked the American public to share potential solutions for our current healthcare crisis. Thank you for opening this dialog and your willingness to include all citizens to help improve America's healthcare system.

I have worked in health care for nearly 40 years, as a nurse, an educator, a researcher, and in the healthcare business sector. Twenty years ago, I founded Hayes, Inc., a health technology assessment company that provides evidence-based research reports on new, emerging, and controversial health technologies to health plans, government agencies, hospitals, health systems, and consumers. We also provide consulting services to help these same constituents put our

research into action to improve patient care and outcomes. Today, our clients serve over 200 million consumers and patients. In the interest of transparency and disclosure, I have a vested interest in the synthesis, dissemination, and application of the expanded base of clinical evidence that would be created under Stimulus Act funding provisions. As such, I am fully committed to improving the safety and quality of healthcare through decisions grounded in evidence. This is my passion, this is my mission, and I believe this is the cornerstone of any meaningful healthcare reform efforts.

With my front-row seat in healthcare, I have thought a lot about why our health care system lags other developed nations. The factors that follow, I believe, are among the most important:

- Failure to effectively and efficiently integrate scientific evidence into healthcare decision-making, resulting in overuse, misuse, and even (in some instances) underuse of health technologies.
- Insufficient, and in many cases poorly designed and executed, comparative effectiveness and cost effectiveness research.
- Adoption of newly-approved technologies before evidence supports it, which contributes to spiraling healthcare costs without commensurate improvement in patient care.
- Perverse financial incentives that drive premature dissemination of new medical technologies even when they do not improve patient care or outcomes.
- Lack of efficient and effective methods to keep clinical practitioners up-to-date on "best evidence" and "best practice" at the time of need.
- A consumer population that is largely overwhelmed with navigating the health care system and finding the right evidence-based resources to make the best health care decisions.
- Uneven and untimely health care access and quality.
- Continued erosion in employer-sponsored health insurance.
- Insufficient focus and allocation of resources to health promotion and disease prevention.

I believe that the comparative effectiveness funding provided for in the Recovery Act (ARRA) will help to stem the tide of rising healthcare costs and will provide research that is sorely needed for payers, providers, and consumers to make better healthcare decisions. To maximize the impact of this funding, I believe the council must focus on primary comparative effectiveness research. There are a number of private sector entities, including Blue Cross Blue Shield Association's TEC Program, Federally-funded Evidence-based Practice Centers, and my firm, Hayes, Inc. that are already positioned to help payers, providers, and consumers synthesize existing and new evidence and integrate the resulting conclusions into the healthcare decision making process. If the Federal Government ultimately assumes the primary role for synthesis of evidence, it will short-circuit these private sector initiatives, add a layer of bureaucracy to the system, and politicize the process.

With this as a backdrop, the following are my recommendations to make best use of the new Federal funding for comparative effectiveness research and improve acceptance and buy-in from the healthcare community at large:

- Coordination and prioritization efforts of the Federal Coordinating Council for Comparative Effectiveness Research must include the private sector in setting the research agenda. Questions about which treatments, technologies, medicines, and

procedures to compare are best posed by the hospitals, universities, manufacturers, consumers, providers, and private research organizations that are at the front lines of healthcare.

- Let the private sector determine how to put the resultant clinical evidence into practice. As noted above, the private sector has already made progress in accomplishing this and I encourage you to build on this base. Federal involvement in putting research results into practice will be seen as a form of rationing.
- Create an environment where providers and consumers have access to the best available clinical evidence. Federal support of the dissemination of comparative effectiveness and cost effectiveness research findings, including digital and user-friendly ways to do so, is needed.
- Allow the private sector to respond to the improved evidence base in making healthcare decisions and avoid an expanded federal policy role that determines coverage and reimbursement beyond the current CMS structure. This approach will allow for public dialogue and solutions and will be less politicized and ultimately better accepted in the healthcare community.
- Encourage all healthcare-accrediting organizations to create standards that require the consideration of scientific evidence in healthcare decision-making. If a technology is adopted in spite of weak evidence, the standards should address a mechanism to evaluate the technology's impact on patient safety, clinical outcomes and comparative value, specifying patient indications and contraindications.
- Fund the creation of patient registries by the private sector. Consider incentivizing industry participation. Universal industry participation is essential if patient registries are to be successful.
- Facilitate public discourse around the meaning, role and importance of clinical evidence, clinical significance, utility, comparative effectiveness and cost-effectiveness as these terms relate to healthcare services, costs, benefits and, ultimately, the choices we make.

Implemented in this manner, the additional funding for comparative effectiveness research provided in the Stimulus Act will eliminate many of the healthcare industry issues I articulated at the start of this letter. I would welcome an opportunity to discuss these and other ideas and recommendations further and to support our healthcare reform efforts.

Submitted by
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There is a wide variation in the human xenobiotic (foreign chemical) metabolism pathways that is conserved across most advanced life forms on earth. These polymorphous pathways include the Cytochrome P-450s and N-acetylation, but, at least in humans, also lesser known pathways such as glucuronidation, methylation, sulfoxidation, sulfation, glycination, and esterases such as paraoxonase and pseudocholinesterase.

This genetic diversity in the metabolism of xenobiotic chemicals would not exist if it was not evolutionarily advantageous to species or populations as a whole.

A key point is that some metabolism pathways will not only detoxify toxicants, but they can also activate carcinogens. Thus, while some individuals may have a slow xenobiotic metabolism pathway--and may be more prone to toxicity buildup rather than carcinogen activation, others individuals have a fast xenobiotic metabolism pathway, which may reduce toxicity buildup but may tend to activate carcinogens and thus increase their risk of cancer. This wide diversity in individual metabolism guarantees that no matter what the toxic exposure, some individual or another in the population is likely to survive in order to preserve the genome.

In fact, this diversity is so great that if on were to add up all the "sensitive" subpopulations, they would probably include the majority of the population. To quote a former director of the U.S. National Institutes of Health (NIH): "Everyone is sensitive to something".

In short, the genetic diversity in individual metabolism of toxics by humans and animals provides robust protection for species and populations in the face of the ever changing environment which has existed on earth over the course of billions of years.

Given these genomics, it is "contrary to nature" to adversely select against individuals who bear genetics which benefit the species.

To paraphrase Sheldon Samuels of the AFL-CIO, failure to protect sensitive subpopulations is social, as opposed to natural, selection against those individuals. But in the case of those polymorphous xenobiotic metabolism pathways with a very high frequency in the genome, such social selection is also contrary to long term species survivability.

In the near future, technology will allow the inexpensive determination of individualized detoxicogenomic profiles, such as by using gene-chips. The National Institute of Environmental Health Sciences (NIEHS) Environmental Genome Project (EGP) has been studying the genetics of the xenobiotic metabolism pathways since 1992 http://www.niehs.nih.gov/research/supported/programs/egp/ and comparative toxicogenomic databases are now being created. http://ctd.mdibl.org/ This may soon allow personal biochemical analysis and individualized medical treatment to be provided at low cost.

We implore the Federal Coordinating Council (FCC) for Comparative Effectiveness Research (CER) to not use simplistic models in designing their research studies, that may provide results on the purported effectiveness of simplistic treatments that under a single-payer or socialized medicine economy instituted under health care "reform" in the U.S. may be used to severely limit choice of treatment to only those previously deemed effective on large groups, including the treatment choice of those with chronic illness that consequently face medical indigency.

Examples of some of the economic and political forces presently at work to constrain treatment choice include:

We have seen complaints about the British National Institute for Health and Clinical Excellence (NICE) report on Chronic Fatigue Syndrome (CFS) and Myalgic Encephalomyelitis (ME) being used to limit the treatments for CFS/ME patients in Britain to Cognitive Behavioral Therapy (CBT), Graded Exercise Therapy (GET), and psychiatric drugs. CFS and ME are in fact symptomatic aggregates of conditions with a diversity of causes, and the few approved treatments in Britain either do not work or are counter-productive in many cases. When combined with a single-payer health care system, this effectly results in denial of individualized treatment for at least medical indigents.

Similarly, in the U.S., we have seen the American Psychiatric Association (APA) re-writing the DSM-V psychiatric manual to try to enlarge the category of purported "somatoform disorders" to include about 15% of primary care patients which they label as having "functional somatic syndromes" such as CFS/ME and Fibromyalgia (FM), and to impute a psychological mechanism of "somatization" to their genesis, in order to try to apply "psycho-social" and psycho-pharmaceutical treatments. Further, the DSM is intended to be "harmonized" with the World Health Organization (WHO) ICD-11 medical coding manual, so that this expansion into medicine by American psychiatry may be promulgated worldwide.

We believe that these regressive efforts to psychiatrize the patients of numerous medical specialties and those who use alternative medical treatments are counter-productive, and are contrary to the progressive possibilities of personalized genetic and biochemical analysis and individualized medical treatment which may soon become possible through modern technology.

We urge the FCC-CER to lead the future towards increasing medical knowledge and improving individualized medicine, rather than to allow its research to be used to regressively promote the rationing of treatment choices for the masses in the name of purported cost savings.

Thank you.

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Submitted by
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My name is Amalia Punzo, MD. I am unable to attend Tuesday's session on Effectiveness Research and would like to contribute a written request prior to the session.

I am a physician trained originally in Internal Medicine. I subsequently gained further training in Integrative Medicine and Homeopathy as a way to address my patient's chronic disease conditions and to practice more holistically with an eye towards safety and disease prevention.

I would like the Council to include homeopathic treatment for both acute and chronic disease states in their research considerations. There are many studies which validate the effectiveness of homeopathic treatment for a wide variety of acute and chronic ailments including otitis media, diarrhea, fibromyalgia, flu, allergic rhinitis, rheumatic disease, mild traumatic brain injury, respiratory conditions, etc...Recently the UK has conducted a large scale public health project looking at the cost effectiveness of homeopathic treatment in primary care, since in Europe homeopathic medicine is much more widely prescribed and accepted. This study reflected favorably on homeopathic treatment and also showed marked cost-savings. In countries where socialized medicine is practiced, it is important to show efficacy as well as cost savings. Our medical educational system focuses almost exclusively (excepting in cases where surgery is indicated) on pharmaceutical approaches to diseases to the exclusion of homeopathic or naturopathic medicine. It was my patients who initially shared with me their experiences using complementary/alternative approaches and for that I am extremely grateful. I subsequently took it upon myself to learn more about these therapies, since there was little to no CAM education in the world of conventional medicine. As a primary care practitioner I was eventually able to treat most diseases more effectively resulting in far less toxicity than when I practiced purely pharmaceutical medicine. It is my sincere hope that we will see well- designed and coordinated clinical outcomes research projects performed in the upcoming years which include homeopathic medicine. The public is more aware than their providers in many cases and wonder why it is that their physicians are not more educated about complementary, alternative, and integrative approaches to disease.

In the last several years I have been trained as an Improvement Advisor by the IHI and am intimately involved in Quality Improvement at my organization. I would like someday to put these QI/Outcomes research skills to good use in conducting such clinical outcomes trials using practice-based research networks that include homeopathic medical treatment. I am hopeful that this administration will pave the way to finance high quality clinical and community outcomes research using a variety of non-pharmaceutical approaches to both acute and chronic disease. I would be happy to provide references to the research studies that I referred to at the beginning of my email.

Submitted by
Daniel B. Fisher,MD,PhD
Steering Committee
National Coalition of MH Consumer/Survivor Org.
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The National Coalition of MH Consumer/survivor Org. would like to see NIMH research funds available for:
1. determining the comparative effectiveness of peer-run and peer-assisted crisis respite services as an alternative to psychiatric hospitalization.
2. research into the degree to which psychosocial, recovery-oriented community supports can reduce the use and cost of of medication.
3. Degree to which peer-run warmlines can reduce the use of emergency room and hospitalization by psychiatric consumers
4. Major psychosocial, and peer related factors involved in persons recovery from mental illnesses when used with medication compared to medication to medication alone as is being practiced in most locales now

Submitted by
Barbara Goldsmith, PhD
President, American Association for Clinical Chemistry

The American Association for Clinical Chemistry appreciates the opportunity to provide comments to the Federal Coordinating Council for Comparative Effectiveness Research in regards to how the Department of Human Services should allocate the $400 million in comparative effectiveness research funding it received under the American Recovery and Reinvestment Act of 2009. We believe this research is critical to gathering the evidence-based data on the utility of health services needed by health care providers to improve the quality of patient care.

AACC strongly supports the purpose of this Council, namely to coordinate the comparative effectiveness research (CER) activities of the federal agencies and to assist the Department in prioritizing future research projects. We believe this approach can reduce overlapping initiatives among the agencies and lead to a more cohesive research agenda. Further, AACC applauds the Council's efforts to engage the health care community and public in this deliberative process. We believe this is important to ensuring that the selected research studies meet the needs of caregivers and relevant best practices organizations.

Although there are many worthy areas for research, AACC recommends that the Coordinating Council include Acute Coronary Syndrome, Heart Failure, Diabetes Mellitus and Cancer, particularly as they as apply to laboratory medicine, among its national priority areas. We think cardiac care, diabetes and cancer need to be addressed given the prevalence of the diseases and their impact on patients, families and the health care delivery system. Also, we recommend that the scope of CER include the evaluation of approaches to health care delivery and care management that foster effective application of personalized medicine.

AACC strongly believes that the Council must maintain the focus of CER on gathering and disseminating knowledge for improving clinical decision-making and patient outcomes rather than emphasizing its cost effectiveness aspects. AACC believes CER must not be used to restrict medical decision-making, hinder technological innovation or, most importantly, limit patient access to effective treatment options. We appreciate the opportunity to provide this input to the Council and look forward to working with you as this process moves forward.

AACC is the principal association of professional laboratory scientists--including MDs, PhDs and medical technologists--and is the leading laboratory association in the realm of evidence-based medicine. AACC's members develop and use chemical concepts, procedures, techniques and instrumentation in health-related investigations and work in hospitals, independent laboratories and the diagnostics industry worldwide. The AACC provides international leadership in advancing the practice and profession of clinical laboratory science and its application to health care. If you have any questions, please call me at (617) 879-0267, or Vince Stine, PhD, Director, Government Affairs, at (202) 835-8721.

Submitted by
Dominic Hodgkin, Ph.D.
Associate Professor
Institute for Behavioral Health
Heller School of Social Policy and Management Brandeis University, MS 035
Waltham MA

To: The Federal Coordinating Council for Comparative Effectiveness Research
From: The Workgroup on Comparative Effectiveness in Behavioral Health, Institute for Behavioral Health, Heller School for Social Policy and Management, Brandeis University
We welcome the new administration's initiative to expand funding for comparative effectiveness research. This initiative has particular relevance to behavioral health care, the area where our own research is focused. It is relevant because behavioral health care (which includes mental health and alcohol and drug abuse treatment) has been particularly prone to some of the problems that comparative effectiveness research is intended to address. These problems include:

- Rapid provider adoption of costly new psychotropic medications, often in the absence of any head-to-head trials demonstrating their superior effectiveness over existing, less costly medications.
- Slow dissemination of certain other effective medications that are mainly used in public sector settings, e.g. naltrexone for alcoholism.
- Under-utilization of approaches with a strong evidence base, such as cognitive behavioral therapies.
- Persistence of non-evidence based practices, such as sub-therapeutic dosing of methadone.

We therefore encourage the Coordinating Council to make sure that some of the new federal funding is directed toward comparative effectiveness research in behavioral health care.
At the same time, we note that these studies will need to go beyond merely measuring 'average' effectiveness of a medication or treatment across the whole population treated. Previous studies

have found that a given medication can have widely different effects across patients, which might be masked by an average effect. We are pleased to note that the enabling legislation takes account of this and calls for studies of subpopulations.
Targeting federal funding for comparative effectiveness research also offers the chance to compare treatments that have been relatively less studied, for example some psychotherapies that are less easily standardized across providers. In some cases this might involve comparing different ways of delivering a given treatment, e.g. web-based treatment versus traditional treatment with counselors.

Many behavioral health care purchasers and providers are frustrated with the current lack of knowledge, and eager to learn more about what works to help patients. They would be likely to act upon the findings that would result from future comparative effectiveness research, for example by disseminating information, removing institutional barriers, and redesigning incentives. In conclusion, behavioral health care may be an especially fruitful area for finding results with policy implications.

Members of the Workgroup on Comparative Effectiveness in Behavioral Health:
Dominic Hodgkin, Associate Professor (chair),
Constance Horgan, Professor and Director, Institute for Behavioral Health
Elizabeth Merrick, Senior Scientist
Gail Strickler, Senior Research Associate
Eve Wittenberg, Senior Scientist

Submitted by
Tony Coelho
Chairman
Partnership to Improve Patient Care

Dear Federal Coordinating Council Members:
As you convene the first of three meetings this afternoon to hear from the public concerning their views on the implementation of comparative effectiveness research (CER) under the American Reinvestment and Recovery Act of 2009 (ARRA), I would like to express support of CER that is transparent, patient-centered and considers the broader body of evidence, the patient's individual needs and preferences, and the physician's clinical expertise.
The Partnership to Improve Patient Care (PIPC) was formed in November 2008 to support new comparative effectiveness research proposals that are centered on patient and provider needs, raise awareness about the value of well-designed CER and promote the important role of continued medical innovation as part of the solution to cost and quality challenges in health care.
PIPC members, representing a diverse, broad-based group of health care stakeholders, are dedicated to working together to promote CER that protects patient access to innovative treatment options; supports the ability of patients, doctors and other health care professionals to choose the care that best meets the individual needs of the patient; and, fosters continued medical innovation. Comparative effectiveness research can be a valuable tool to "learn what works in health care" and support good clinical decision-making. At the same time, such research can be misapplied in ways that restrict patient access to optimal care, undermine physician/patient

decision-making, and discourage continued medical progress. Below is a list of PIPC Supporting Principles that we believe must be met to ensure that patients and providers interests remain paramount:

- Define CER as a tool to improve patient care;
- Enhance information about treatment options and about how to close the gap between care known to be effective and the care patients receive;
- Focus on communicating research results to patients, providers and other decision-makers, not making centralized coverage and payment decisions or recommendations;
- Provide information on clinical value and patient health outcomes, not cost-effectiveness assessments;
- Design studies that reflect the diversity, including racial and ethnic diversity, of patient populations and communicate results in ways that reflect the differences in individual patient needs;
- Assure that studies are technically excellent and appropriate;
- Require open and transparent processes where all stakeholders have input into research priorities and design and have an equal voice in governance of a CER entity;
- Examine all aspects of health care including care management, medical interventions, benefit design, and processes of care for all patients;
- Support continued medical advances, including personalized medicine and other advances that can help improve patient care and control health care costs;
- Recognize the unique nature and value of targeted therapies that benefit specific groups of patients with rare and orphan diseases.

CER has the potential to transform healthcare and better inform patient and provider decision-making. Focusing on improved quality is the best way to achieve a more sustainable and affordable healthcare system, and comparative clinical research can help us reach this goal. It is important to distinguish comparative clinical effectiveness research which focuses on health outcomes from cost-effectiveness research, which can be misused in ways that deny individuals access to the medical care that is best for them based on arbitrary cost thresholds. Working with PIPC, I will strive to make sure CER is centered on patient needs and does not become a basis for denying patients access to the care they need.

On behalf of PIPC's member organizations, I look forward to working with you to advance CER that improves the lives of all Americans.

Thank you for your consideration.

Submitted by
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The American Urogynecologic Society (AUGS) appreciates the opportunity to provide comment to the Federal Coordinating Council for Comparative Effectiveness. AUGS is a health care organization comprised of clinicians and scientists dedicated to advancing research, education and patient care in the area of female pelvic medicine and reconstructive surgery. AUGS believes significant investment from the $1.1 billion in funds for comparative effectiveness research provided by the American Recovery and Reinvestment Act of 2009 should be granted to areas focused on pelvic floor disorders. AUGS is committed to ensuring that these investments would be well spent and would yield better patient outcomes.

Although pelvic floor disorders (PFD), including disorders of urinary and/or bowel control, and pelvic organ prolapse are common and costly, these conditions are relatively understudied. Of these disorders, urinary incontinence is the most common.

A recent report from the Pelvic Floor Disorders Network, funded by NICHD, and published in the *Journal of the American Medical Association* (JAMA) reports that pelvic floor disorders are common and serious conditions impacting women in America. This report sited that of the women who reported any symptoms of a pelvic floor disorder, 16% experienced urinary incontinence, 9% reported fecal incontinence and almost 3% had symptoms of pelvic organ prolapse. These numbers increased with age with half of all women over 80 being affected. One out of 11 women will undergo surgical treatment for pelvic organ prolapse and urinary incontinence in her lifetime. In February another study was published in the *New England Journal of Medicine* that demonstrated that weight loss resulted in a 47% drop in weekly incontinence episodes. These findings confirm that weight loss can be considered a first-line treatment for women with incontinence. These two articles demonstrate that there are so many things we are still discovering about these conditions and additional research funds need to be allocated to encourage further investigation and education of these disorders that are associated with depression, isolation, and decreased quality of life.

There are three key areas where we believe comparative effectiveness research could identify treatments that would yield better outcomes and care for women who suffer from pelvic floor disorders.

Stress Urinary Incontinence

Improvements in surgical treatment of urinary incontinence will improve the quality of life for American women and reduce associated health care costs. Surgery for treatment of stress urinary incontinence (SUI) is common and increasing, with 135,000 surgical procedures done in the US, an approximately 45% increase from 1988[1]. Ongoing efforts to select the most appropriate initial surgery and comparative trials to compare surgical therapies to non-surgical treatments, including pelvic floor exercises are needed.

Approximately 10-40% of women have recurrent or persistent SUI after a continence procedure and therefore re-operation rates after surgery for urinary incontinence are high, with at least one third of women undergoing repeat surgery during her lifetime [2,3]. Few data are available to guide surgical treatment of recurrent or persistent SUI, although it is commonly accepted that repeat continence procedures are associated with higher failure rates and that failure rates increase over time[4-6]. Comparative effectiveness trials are urgently needed to guide the care of women with persistent or recurrent SUI.

The NIH has invested in comparative effectiveness trials for women with uncomplicated SUI. The NIDDK Urinary Incontinence Treatment Network recently reported in the SISTEr Trial that cure rates after continence surgery are considerably lower than previously reported[7]. Unfortunately, only a small minority of women in this trial had undergone a prior continence surgery: 13% in the sling group and 15% in the Burch group. A second comparative effectiveness trial has just completed enrollment, but given the similarity in inclusion/exclusion criteria, it is likely that the network will enroll a similar percentage of women with recurrent or persistent SUI after a prior continence procedure.

Therefore, despite the high incontinence prevalence rates and high re-operation rates, there are no adequately powered randomized trials investigating the optimal method for treating SUI in this population of women. Without advanced understanding of the consequences and optimal surgical strategies for SUI, treatment in this important area of women's health is advancing slowly.

Prolapse Surgery

Three to six percent of women will develop pelvic organ prolapse during their lifetime, with half reporting significant impact on her quality of life secondary to the prolapse[8]. Surgical therapy is the gold standard for the treatment of pelvic organ prolapse. Pelvic organ prolapse is the main indication for hysterectomy in women over the age of 50. In 1997, approximately 225,000 surgeries were performed for pelvic organ prolapse in the United States with a direct cost of $1.12 billion dollars[9].

While numerous surgical options are available, relatively few comparison studies have been performed. In a recent Cochrane review, only 22 studies of significant quality and follow-up could be included in the review. Astonishingly, these 22 studies included less than 3,000 patients to compare the effectiveness of different surgeries for pelvic organ prolapse[10]. Many more studies with adequate power are needed to determine the best surgical procedures. Randomized trials with long term follow-up to access cure, risks for failure, and complications are drastically needed. Without comparative effectiveness studies, women and their surgeons do not know the best procedures to perform to help relieve women of this common health problem.

Randomized Trials and Mesh Registry

In an attempt to improve surgical repairs of pelvic organ prolapse, many surgeons have begun the use of vaginally placed mesh to strengthen the native tissues. Currently very little data exists to support this treatment option, yet its use is growing in popularity. The use of mesh adds cost and potential complications to the procedure, with little data to support improvement in outcomes or reduction of surgical failures. We are very interested in accessing comparative

effectiveness research dollars to support randomized surgical trials and/or for the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data.

The medical community would benefit greatly through the creation of a patient registry that tracks the use and effectiveness of surgical mesh for the treatment of pelvic organ prolapse and stress urinary incontinence. The collection of pre- and post-market data is key to understanding why mesh erosions occur and what can be done to eliminate the risk to patients.

Establishing a national registry is a large project and one which can not be done by one organization alone. AUGS would like to work with NIH and partner with the FDA and CMS to create a mesh registry that improves outcomes while saving patients from medical complications and the need to undergo multiple costly surgeries.

Conclusion

AUGS sincerely appreciates the support our members have received and the work we have been able to accomplish to date through government supported grants. Millions of women are impacted by pelvic floor disorders. Comparative effectiveness studies are needed to determine best practices and therapeutic options. Thank you for your attention to our requests and this important area of research in women's health.

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Submitted by
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AdvaMed
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My name is Teresa Lee, and I am here on behalf of AdvaMed, the Advanced Medical Technology Association. AdvaMed represents the medical device and diagnostics products industry, and our members produce nearly 90 percent of the health care technology purchased annually in the United States.

We greatly appreciate the opportunity to comment today on HHS's implementation of comparative effectiveness research funds allocated to AHRQ, NIH and the Secretary in the American Recovery and Reinvestment Act of 2009. We have comments on three subjects today: (1) transparency and stakeholder input; (2) selection of the comparative effectiveness research priorities; and (3) content of the research topics.

Transparency and Stakeholder Input

First, regarding transparency and stakeholder input, we have been pleased to hear of the Obama Administration's strong commitment to "openness and transparency," particularly in relation to operations around comparative effectiveness research and that the Council "looks forward to hearing from all parties as it moves ahead." AdvaMed values the Council's conduct of this listening session today as a major first step in considering stakeholder input. In keeping with the Administration's emphasis on openness and transparency, we urge the Council to make public all of its meetings in order to keep the public informed of the priority-setting process, and to enable on-going input. Furthermore, we urge the Council to make its draft findings and recommendations available for public comment before they are finalized and submitted to the President and Congress. This approach will enhance the credibility of the Council's findings and recommendations by enabling fully informed decision-making with input from patients, clinicians, health care providers, and scientific and clinical experts employed by manufacturers who have firsthand experience with how various topics would be relevant for clinical practice.

Selection of Comparative Effectiveness Research Priorities

Second, regarding comparative effectiveness research priorities, AdvaMed recommends that HHS first focus on efforts to improve the infrastructure for comparative effectiveness research. Using the Recovery Act funds to improve health services research workforce training would be one good way to lay the groundwork for any longer term efforts to bolster comparative effectiveness research. In particular, we think that sponsorship of interactive forums where comparative effectiveness grantees can work shoulder-to-shoulder with private sector researchers would be valuable so that these clinical and technical experts can learn from one another. Another priority area should be the development of improved pathways to translate research into practice. As you know, one study found that it takes 17 years on average for clinical research results to enter into mainstream practice. To ensure that comparative effectiveness research

successfully advances practice, we should invest in developing better methods and tools to make sure that clinicians and patients understand and integrate research findings.

With respect to specific research subject areas, AdvaMed recommends that the comparative effectiveness research agenda be prioritized with a focus on areas that have major clinical significance and that will have the greatest impact on delivery of health care to patients in the United States. By way of example, chronic disease management and hospital-acquired infections and conditions represent significant comparative effectiveness research opportunities. Selecting comparative effectiveness research priorities along these lines would be consistent with the recommendations set forth by the IOM in its 2003 report "Priority Areas for National Action: Transforming Health Care Quality." From a public health standpoint, it makes sense to follow a broad-based approach that is not purely disease-based, but rather includes preventive care and behavioral health as means to improve quality.

We also recommend that the Council be inclusive in the research priority areas selected, so that the process of care, including the services involved, clinician capability, and other factors are taken into account in assessing comparative effectiveness. This is particularly important in assessment of medical devices.

Content of the Research Topics

Third, AdvaMed supports using comparative effectiveness research to inform medical decisions. The purpose of comparative effectiveness research should be to provide better evidence for physicians and patients to use in making individual clinical decisions. It should enhance, not hinder or preclude, a physician's ability to exercise independent professional medical judgment in providing care to patients, so that patients have access to the interventions that best meet their individual needs and circumstances. That is why it is essential that clear, comprehensible study results be disseminated to physicians and the patients whom they treat. It is why comparative effectiveness findings should not result in one-size-fits-all coverage recommendations.

Finally, we believe that the comparative effectiveness research pursued should focus on clinical effectiveness, not cost effectiveness. As stated in the Conference Report to the Recovery Act, the purpose of the comparative effectiveness funding is for the conduct or support of research on "*clinical* outcomes, effectiveness, risk and benefits" (emphasis added). We note that on March 4th, NIH announced its new initiative called the "NIH Challenge Grants in Health and Science Research," which may use funding designated by the Recovery Act specifically for comparative effectiveness research. The NIH listed several topics that would include cost effectiveness analysis. To use the Recovery Act's comparative effectiveness funds in this manner would run contrary to the statement in the Conference Report language and for this reason, we recommend against this application of the funds. Moreover, using this research to deny access to appropriate treatments for individual patients with individual medical histories and individual needs should not be the objective. AdvaMed supports the conduct of clinical comparative effectiveness research and believes that such research will ultimately improve quality of care and have a favorable impact on overall efficiency in the health care system.
Thank you for your time today.

Submitted by
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American Academy of Hospice and Palliative Medicine
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Dear Dr. Haddix and members of the Federal Coordinating Council,
We are writing to provide input into how to most effectively allocate the new CER resources.

Several reports from the National Institute on Health[1] and the Institute of Medicine[2] have called for substantial investments in palliative care research. While the growth of our field has been remarkable, the knowledge base to support basic elements of clinical practice still remains small. The need to evaluate efficient, patient-centered care delivery systems has grown even more critical. CER activities that address critical research questions in palliative care will help align CER with the critical areas identified by the National Quality Forum Priority Partners as fruitful areas to achieve systemic improvement in health care. [3]

The ***American Academy of Hospice and Palliative Medicine*** recommends the following strategies be integrated into the CER priorities:

1) Integrate palliative care outcomes into a broad range of CER.
Along with studying the comparative impact of interventions, pharmaceuticals and devices on the outcomes of survival and function, all CER in the seriously ill patient population should look at palliative care outcomes related to the burden of disease. These outcomes include:

- Quality of life including disease-related burden of pain and other symptoms
- Patient and family experiences with care

When comparing program interventions, additional outcomes to be measured should also include:

- Quality of care including the quality of communication and alignment of care and its outcomes with patient preferences, and care transitions

2) Target CER efforts to develop the evidence base for the clinical interventions that best ameliorate pain and other symptoms.
Ample evidence demonstrates that patients with serious illness experience a multitude of profoundly distressing symptoms. A conservative estimate suggests that of the 1 million persons who died in a U.S. hospital in 2001, 324,000 had fatigue, 280,000 had loss of appetite, 244,000

[1] National Institutes of Health, **National Institutes of Health State-of-the-Science Conference Statement on Improving End-of-Life Care** State-of-the-Science Conference Statement. December 6–8, 2004. http://consensus.nih.gov/2004/2004EndOfLifeCareSOS024html.htm

[2] Marilyn J. Field and Christine K. Cassel, Editors; Committee on Care at the End of Life, Institute of Medicine**. Approaching Death: Improving Care at the End of Life. 1997** www.iom.edu/CMS/3809/12687.aspx

[3] Palliative and end-of-life care is one of six priority areas identified by the National Quality Forum as critical for improving our national health care delivery. http://www.nationalprioritiespartnership.org/PriorityDetails.aspx?id=608

had shortness of breath, 232,000 had dry mouth and oral ulcers, 208,000 had cough, 196,000 had pain, 148,000 had confusion, 148,000 had depression, 140,000 had nausea, 92,000 had difficulty sleeping, and 88,000 had vomiting. The reasons for this distressing state of affairs are many but almost all stem from an approach to medical research that has often viewed symptoms and suffering as interesting primarily insofar as they guide the physician to a correct diagnosis.[4] The prevailing philosophy dictates that once the diagnosis is made (e.g., cancer) and the disease is treated (e.g., chemotherapy), the symptoms will dissipate. What should be done to relieve suffering during treatment or when a disease can't be treated or cured is rarely discussed, and certainly has not been adequately researched. It is not surprising, therefore, that a recent NIH-supported comprehensive review of research revealed that data that should guide the treatment of late life symptoms are not only inadequate but in many instances completely absent. High quality evidence informing symptomatic management is glaringly lacking for even the most common, highest impact symptoms such as dyspnea. Concerted application of CER to routinely used clinical interventions could yield important advances in improving treatment of common, distressing symptoms.

3) Compare palliative care delivery models.

The last year of life consumes almost 30% of lifetime Medicare expenditures[5], and evidence for the positive impact of both hospice and palliative care programs on both quality of care and cost of care is accumulating.[6] However, the knowledge base is sorely lacking in methodologically rigorous studies that illuminate which processes of care and specific program interventions and models are the most effective. Although we understand that comparisons of service delivery models have not typically been an object of study via CER, we believe that applying CER to palliative care models would yield important and actionable information that is critical for informing efficient, higher quality late life care. In particular, the palliative care field needs studies to illuminate best care models for difficult populations, such as minorities and nursing home patients, and to reduce health outcome disparities and inform policy debates about shaping reimbursement policies.

4) Create the infrastructure needed to carry out CER in patient populations using palliative care.

The NIH State of the Science Consensus Panel noted that research in palliative care is hampered by an under-funded, under-resourced research sector specific to palliative care. The NIH panel recommended*:*

> *"Create a network of end-of-life investigators and well-defined cohorts of patients to facilitate coordinated interdisciplinary, multi-site studies. This should include establishing new networks of end-of-life investigators as well as expanding existing networks (such as the National Clinical Trials Cooperative Groups) so they have a critical mass of end-of-life investigators and appropriate study populations. These*

[4] on Gunten CF. Interventions to manage symptoms at the end of life. J Palliat Med. 2005;8 Suppl 1:588-94.
[5] Hogan C, Lunney J, Gabel J, Lynn J Medicare beneficiaries' costs of care in the last year of life. Health Aff. 2001 Jul-Aug;20(4):188-95.

[6] Dartmouth Medical School. Center for the Evaluative Clinical Sciences. The Dartmouth Atlas of Health Care, 1998. Chicago, IL : American Hospital Publishing, 1998. http://www.dartmouthatlas.org

networks should enhance training of a new generation of interdisciplinary scientists (through funding mechanisms, such as K-awards, T32s, and R25s).”

This directly supports an existing priority of the National Institute on Aging: 05-AG-101* Data Infrastructure for Post-Marketing Comparative Effectiveness Studies.

“*The challenge is to create the data infrastructure that will enable comparisons of particular therapies, prescribing patterns, and benefit designs on health outcomes. Problems with currently available studies include omission of key patient groups (such as the elderly in nursing homes), lack of information on adherence and outcomes in polypharmacy, lack of information on outcomes across different insurance benefit designs, and lack of information on actual prescribing patterns and outcomes across regions and over time. “*

In summary, we believe that comparative effectiveness research in hospice and palliative medicine can yield many benefits for patients, providers, and our healthcare system. In the coming decades more Americans than ever will be facing their later years of life, and we must therefore ensure that these priorities are integrated within CER.
Thank you for this opportunity to participate in this inquiry. We would be happy to provide more information for clarification.

Submitted by
Grace E. Jackson, MD
Wilmington NC
gracejackson@ec.rr.com

Dear Federal Coordinating Council:

This statement is written with respect to the allocation of money (1.1 billion dollars) from the 2009 Recovery Act fund for the purpose of achieving Comparative Effectiveness Research.

Recommendation #1 Prioritize the End of Corporate Fraud

Research into the comparative effectiveness of medical treatments is a laudable goal, but only if it does not repeat the same errors of the past. When, in the early 1990s, medical journals, medical schools, residency and postgraduate training curricula, and health care facilities came under the influence of Evidenced Based Medicine (or EBM), the favored treatments in American medicine came to reflect the following values and priorities:

1) symptom suppression (rather than elimination of root cause of illness)
2) short-term studies (e.g., Randomized, Placebo Controlled Trials)
3) fraudulent research designs (e.g., placebo washout/lead-in)
4) concealment of data unfavorable to the interests of the drug industry
5) academic censorship (e.g., non-disclosure and confidentiality agreements)
6) distortions in the medical literature (ghostwriting, file drawer effect)
7) treatment by consensus (rather than treatment based upon science)

Each of these developments contributed to the hegemony of sham standards of care.

Effectiveness Research will be meaningless if it repeats these errors of the past.

Recommendation #2 Focus Upon Basic Science and Biology

The current system for approving new medications and medical devices emphasizes proof of efficacy in principle, *rather than proof of effectiveness in fact*. This system has given rise to the introduction and widespread use of one, after another, copycat therapies based upon dubious definitions of benefit (e.g., checklists of subjective symptoms in psychiatry; measurements of "risk factors" and surrogate endpoints as substitutes for real progress in ameliorating the symptoms of chronic disease). Most critically, the past 20 years of American medicine have diverted attention away from the study of basic physiology and the mechanisms of disease.

Unless and until the treatment paradigm in American medicine returns to an emphasis upon root causes of illness and disease, and upon the eradication or amelioration of those causes, the system of healthcare will continue to reflect interventions which are largely futile for patients.

What might be done:

1) identify environmental sources of illness and map the epidemiology of risk factors and diseases related to same
[e.g., the U.S.A. needs a national equivalent of Green Cross International]

2) re-evaluate national healthcare policy with respect to HPDP
[Health Promotion / Disease Prevention]

-- verify or refute high cholesterol as the necessary and sufficient cause of heart disease

-- verify or refute the existence of cumulative safety thresholds for diagnostic radiology

-- verify or refute the long-term harmfulness of the current immunization schedule (particularly, with respect to autoimmune dysfunction, diabetes, asthma, obesity, and neurobehavioral syndromes)

-- verify or refute the long-term harmfulness of fetal ultrasound

-- verify or refute the long-term hazards of fluoridation

-- verify or refute the validity of Gallo's work, positing HIV as the cause of AIDS

[see Nortin Hadler's books: *The Last Well Person* and Worried Sick]

Recommendation #3 Prevent and Mitigate Iatrogenic Harm

The allopathic model of medicine is failing America because authorities will not acknowledge the unnecessary harmfulness of synthetic chemicals. All of the existing training programs, textbooks, Board Certifications, and treatment algorithms emphasize the use of pharmaceuticals that are based upon short-term drug trials, and short-term studies in lab animals. Yet, human subjects (at least, in the U.S.A.) are increasingly encouraged to consume multiple medications for life. This philosophy of lifetime, prescription drug dependence ignores the scientific realities of what happens to patients under the influence of chronic medication.

Unless and until health care providers, policy makers, and regulators recognize the problems of *allostatic load* (the body's adaptations to therapy which ultimately result in diminishing benefits or worsening disease) and prioritize the avoidance, amelioration, and/or reversal of *target organ toxicity*, no amount of "effectiveness research" will be meaningful.

What might be done:

1) effectiveness research must involve considerations of treatment UTILITY (Benefits *and* Hazards)

2) effectiveness research must include considerations of **Target Organ Toxicity** [e.g., how various treatments harm the diseased organ] and **allostatic load** [e.g., how various treatments induce changes in gene expression which may result in delayed but potentially long-lasting effects]

3) effectiveness research must include considerations of treatment effects upon the **environment** (e.g., xenobiotic diffusion via sewage; air pollution from hospital incinerators) and environmental effects upon treatment (proximity of treatment facilities and patients to radon, radioactive waste, Superfund or other toxic waste sites, petrochemicals, etc)

Recommendation #4 Recruit the Best Treatments from Around the World

The federal government gives lip service to the importance of research in the areas of complementary and alternative systems of health care. However, the U.S.A. has become a pharmaceutical oligarchy which permits no challenge or rival to allopathic medicine. No amount of effectiveness research will be meaningful unless and until the yoke of pharmaceutical authoritianism is broken. Ideally, effectiveness research will incorporate the "best treatments" (herbs, diet and lifestyle modification, environmental modification) from around the world.

Recommendation #5 Protect the Privacy of Patients and Physicians

Given the pervasiveness of corporate fraud and the denigration of integrity within the American health care system – particularly, as these have progressed in the era of Evidence Based Medicine -- patients and physicians require protection from harmful practices. Treatment facilities, insurance companies, and State Medical Boards mandate compliance with corporately shaped, corporately biased Group Think.
Clinicians have lost the right to practice medicine by using their best clinical judgment, informed by an understanding of basic science, direct observation, and the consideration of the unique circumstances of each and every patient.

It is extremely unlikely that America's pharmaceutical oligarchy will ever be displaced or transformed into the kind of system which serves mankind, rather than profit and power. This being so, the ***results of effectiveness research must not be allowed to infringe or violate the privacy of patient***s, nor the rights of clinicians who desire the freedom to honor the ethical principles of patient autonomy, physician beneficence, and physician non-maleficence.

What might be done:

1) patients and providers must be allowed to opt out of electronic medical records system, health care registries, and other databases where biological and social information can -- and most likely will -- be used to ration health care, restrict employment or travel, or reduce entitlements

2) patients must be protected from medical tyranny (i.e., medical blackmail --- the allocation of therapies or benefits based upon compliance with dubious and potentially harmful treatments)

3) health care providers must be protected from medical tyranny (i.e., medical blackmail in the form of Pay-for-Performance programs, "Consensus" Statements, Sham Peer Review/Disruptive Physician proceedings, etc).

Summary

Effectiveness research is a laudable goal, but only if it is conducted in a way which avoids the tragic errors of the past (i.e., the corporate medical fraud which has gained traction under the influence of EBM, the Daubert decision of 1993, the Prescription Drug User Fee Act, the Bayh-Dole Act, Direct-to-Consumer Advertising, etc), and only if it anticipates new challenges of the future.

Particularly in the context of emerging technologies (brain mapping, gene mapping, high-speed information exchange), there will be ever more opportunities for the leaders of allopathic medicine -- and for the leaders in government -- to enslave, rather than to serve, the providers and consumers of health care.

Ultimately, effective health care must also be ethical health care. This will require a return of integrity in the conduct of American medical research. It will also require a health care system which prioritizes the delivery of services that are consistent with fundamental human rights, and with the human species' duty to protect (rather than to plunder) the planet's biosphere.

Thank you for the opportunity to contribute these ideas and opinions.

Submitted by
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I write to day to express my concern about the impending termination of the Partnership Program of the Cancer Information Service as of January 2010. I am especially concerned given the current stimulus package opportunity to maintain the community infrastructure and employment of a cadre of highly trained cancer control specialists. The stimulus package provides a unique opportunity to maintain this effective and high quality program in the community during your planning process to determine the future initiatives to translate science into practice and address health disparities.

Given the historical mistrust of researchers and federal government by minority communities, and the fact that these same communities bear a disproportionate share of the cancer burden, we simply cannot afford to purposely cause a break in the relationship between community organizations and the National Cancer Institute. The current plan to "pause" and reconsider the paradigm is high on risk and has no reward when taken in the context of community partners.

The translation of science into practice is the logical end-point for bench science. It is also the beginning point for public health practice. Both researchers and practitioners must engage in this natural shared responsibility. Community partners – whether they are churches, clinics, health departments, or local cancer control coalitions – need the expert, unbiased guidance and support of the Partnership Program in order to target audiences, select or adapt programs, implement and evaluate evidence-based programs, and plan strategically for the future. This is difficult work, and work that cannot be done solely via electronic media or avatar.

I understand that other Partnership Program models could potentially be more effective or efficient but the truth now is that NCI is the current sole custodian today of the trust between minority communities and NCI. And although NCI supports a number of projects via grants, none provide ongoing, comprehensive coverage throughout the United States. As such, NCI needs to maintain an ongoing presence and some level of face-to-face, personal engagement with minority and rural communities until a new paradigm can be designed, procured and implemented.

Specifically, I strongly urge you to implement these policy actions:

1. Implement a transition strategy where current Centers are funded through stimulus dollars at a level sufficient to maintain these core functions:
   a. link NCI to community organizations
   b. link communities to NCI
   c. build capacity of community organizations to interpret, adapt and implement evidence-based programs
   d. communicate future plans of NCI in such a way as to promote the community's acceptance and trust
   e. facilitate a smooth transition to new model(s)

2. Incorporate local community organization leaders into the redesign process, not just representatives from national organizations (e.g., community health workers, community development corporation leaders)

3. Accelerate the planning process as much as possible so a new procurement process can avoid missed opportunities to innovate in the field.

To be certain, community trust is the prime concern here. Yet, there is one other concern I believe to be important.

The current workforce of the Partnership Program is a unique and valuable part of our country's infrastructure. In these times when local and state health departments, as well as voluntary associations and other non-profit organizations, all across the country have hiring freezes or some combination of furloughs and lay-offs, we simply cannot and should not weaken our already fragile public health workforce. I believe my suggestion of funding Centers so that core functions may be maintained until the next iteration of the Partnership Program is in place will serve to minimize the short-range harmful impact on the workforce.

In addition to my practical, low-cost policy suggestions, I have several important questions:

(1) I would like to have a copy of the full evaluation results of the Partnership Program evaluation that was conducted about a year ago and included community collaborator feedback;

(2) I would like to have concrete action steps that demonstrate NCI's commitment to "community trust" that go beyond the study group

(3) I would like to know why NCI thinks a gap in service to community and the loss of 80-100 jobs is acceptable when one looks at the effort and investment in saving and creating jobs through the Recovery Act.

In closing, given the recent historic investment in Economic Recovery Act, NCI has the mandate to uphold its responsibility to community partners and make preservation of this part of the public health workforce a high priority. You can do this by taking the steps I have suggested in this correspondence.

This is an important leadership opportunity.

I look forward to your response and action to implement these recommendations.

Submitted by
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Thank you for the opportunity to comment. I am Secretary of the Community Health Worker (CHW) interest group of the American Public Health Association. I was also a principal author of HRSA's CHW National Workforce Study (2007) and convened an invitational conference in 2007 to draft a national research agenda on CHWs.

I strongly recommend that your plans include significant studies of the cost-effectiveness of CHW interventions. Past studies suggest a great potential for CHWs to reduce total costs for programs such as Medicaid, Medicare and SCHIP but the data are limited. There has been increasing interest in CHWs but policy has been slow to respond due to an inconclusive evidence base, largely due to methodological weaknesses and inconsistencies in past research. A group of leading researchers is planning a follow-up conference to recommend common metrics for CHW effectiveness and cost-effectiveness early in 2010.

CMS is currently testing CHW approaches to interventions such as excessive Emergency Room utilization (Medicaid) and diabetes self-management (Medicare) but neither initiative is rigorously studying cost-effectiveness of CHW methods. Minnesota has recently (February 2008) authorized hourly reimbursement for CHW services under Medicaid, the first State to do so. Various reports such as the IoM's "Unequal Treatment" (2002) have recommended greater roles for CHWs. The 2009 SCHIP reauthorization specifically includes mention of CHW roles in outreach and education. The recent NIH Challenge Grants announcement includes three Topic Areas which specifically mention CHWs: 05-MD-102* (Prevention of Chronic Diseases in Disparity Populations), 05-MD-105* (Health Literacy) and 09-MD-101 (Creating Transformational Approaches to Address Rural Health Disparities).

It would be helpful to all these agencies to have a coherent, coordinated interagency approach to understanding the full potential of this workforce. HRSA's Maternal and Child Health Bureau obtained private commitments of some $2 million for a large scale CHW cost-effectiveness study in 2000-2001 but the project was cancelled by the incoming Administration. The present economic recovery appropriation is an ideal vehicle for such an investment.

I would be happy to provide further information and contacts.

Submitted by
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Good evening:
On behalf of Bayer HealthCare Pharmaceuticals Inc., we are writing to notify the Council and the Office of the Secretary that we intend to submit a written statement for consideration regarding comparative effectiveness research.

We understand from the Federal Register notice published on April 10, 2009, that interested persons registering for the public meeting on April 14th notify the Office of the Secretary of their intent to submit a written statement for consideration by the Council. Although we are not submitting our comments at this time, consistent with the information in the Federal Register notice, we write today to notify the Council and the Office of the Secretary of our intent to do so in the near future.

Please let us know if you have any questions in the interim.

Submitted by

David M. Carlisle, M.D., Ph.D.
Director
California Office of Statewide Health Planning and Development

Mark B. Horton, M.D., M.S.P.H.
Director
California Department of Public Health

States and local jurisdictions, with Medicaid, SCHIP, public health and a variety of other programs, will directly benefit from the results of Comparative Effectiveness Research.

Given these potential benefits, it is critical that the priorities of state and local jurisdictions be given consideration in evaluating various Comparative Effectiveness Research strategies. For example, many jurisdictions, such as California's Medi-Cal program, have on-going

investigative agendas designed to improve program effectiveness that can be considerably amplified by Federal support. Such efforts would extend beyond purely clinical protocols to include the evaluation of public health, community-based, and behavioral strategies that may enhance the effectiveness of public programs.

States and local jurisdictions also have strengths and advantages that compliment Federal Comparative Effectiveness Research programs. These strengths and advantages include

functional relationships with academic partners, access to a variety of special demographic, cultural, and linguistic populations, and a direct knowledge of how potential interventions may work in their local environments.

Submitted by
Susan Hodges, President
Citizens for Midwifery
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Maternity care, one of the largest sectors of the health care system, clearly needs the Council's attention and needs Comparative Effectiveness Research (CER) funds directed to it.

Over 4 million women give birth to over 4 million babies each year in the US. Research tells us that around 80% of those women go into labor relatively healthy, but nearly all are subjected to inappropriate practices and unnecessary medical interventions that result in high rates of complications and morbidity. Multiple organizations have compiled extensive systematic reviews of the research about many aspects of maternity care, and the evidence clearly demonstrates that much of what is done in US maternity care is unnecessary and often harmful, and very costly, resulting in poor outcomes.

We urge the Council to recognize the enormous importance of maternity care, both for the economy and for the lifelong outcomes for babies, mothers and families, and to make use of the large body of available research comparing outcomes of typical obstetric practices and protocols with the supportive care of midwives, in and out of the hospital.

The recently released report, *Evidence-Based Maternity-Care: What It Is and What It Can Do*, from Childbirth Connection in New York City (http://www.childbirthconnection.org/article.asp?ck=10575) , is the most recent and most complete analysis of the evidence relevant to birth practices. This report states that "Effective maternity care with least harm is optimal for childbearing women and newborns." It is also optimal for taxpayers, who directly pay for nearly 40% of births through Medicaid, but also for those who will need to pay for the harms that result from ineffective care, sometimes for a lifetime. The report also examines the many barriers to optimal care.

Despite spending far more on maternity care than any other country, the US has dismal outcomes relative to other developed countries high infant mortality rates, stagnant or rising maternal mortality rates, high maternal morbidity rates, high rates of preterm birth, high rates of low birth weight babies, and relatively low rates of breastfeeding success. The other industrialized countries, who spend less and get better outcomes, use midwives as primary health care providers for pregnant women, because midwives are trained to support normal (physiological, undisturbed) birth, and only refer to obstetricians those women who actually need their skills.

One example of a problem that needs attention is the record high rate of cesarean section, which cost us much more than normal vaginal births. Over 1 in 3 women now give birth by major abdominal surgery, although research shows that the benefits for mothers and babies break even

at a rate of 10 to 15%. Overuse of cesarean section is having short and long term impacts on the health of mothers and babies and will likely impact the whole health care system for years to come. Indeed, cesarean section and induction of labor are cited as factors in the continuing rise in late preterm birth, which greatly increases the risks for long term health and educational difficulties for the babies involved. Furthermore, more than 800 hospitals in the US now have policies to not allow women to attempt vaginal delivery for a pregnancy following a cesarean section, even though there is ample evidence that vaginal birth after cesarean (VBAC) is a healthier choice for the majority of such mothers and their babies.

Women can find out more about a used car than they can find out about their obstetrician or their local hospital, because only two states are required to publicly report birth outcomes and interventions for hospitals, and there is NO reporting for individual obstetricians. Combined with the lack of transparency and accountability, few women and their families are aware that most maternity care is not based on research evidence, or that where and with whom they choose to give birth are independent risk factors for whether they have induced labor, cesarean section, or episiotomy (another overused surgical procedure) and a myriad of other practices and interventions. This lack of transparency about how their providers practice and what happens in their local facilities makes finding appropriate care very difficult for women and families.

We hope CER will not only look at Comparative Effectiveness Research for maternity care, but also consider how government agencies can help the public understand the need for evidence-based practice that is so desperately needed in maternity care.

We are asking CER to look at the extensive existing evidence comparing the care practices used by midwives, who have excellent outcomes in and out of the hospital, with the care practices used by obstetricians in hospitals that result in abnormally high rates of interventions and complications. Obstetricians are extremely skilled specialists in the pathologies of pregnancy and childbirth. However, unlike nearly every other sector of health care, most pregnant and birthing women and their babies are healthy and do not need the skills these expensive specialists possess. In no other area of health care do we expect (and pay for) physicians to provide interventive and invasive “care” that is not medically indicated.

Midwives on the other hand are trained in the normal processes of pregnancy and childbirth, with an understanding about when greater intervention is needed. Most of the other developed countries with better outcomes and lower costs use midwives to attend most women and babies. Indeed, the Midwives Model of Care (see below), which provides respectful, individualized care that includes education, support, and appropriate use of technology, has been shown to not only improve outcomes overall, but to also help reduce disparities among women at risk for poor outcomes due to race, ethnicity, income-level, and other demographic factors, while reducing costs.

We urge the Council to examine the current research and work with all stakeholders, including consumer advocacy organizations and midwives, to direct CER funds toward maternity care. We also need to develop an appropriate national data set of criteria for optimal maternity care. This will allow providers, facilities, payers, and most importantly, women and their families to determine the appropriate facilities and providers that will offer them optimal care and give their

babies the best start possible.

Thank you for your attention to these very important concerns and for your consideration of the need to use Comparative Effectiveness Research to bring about better and more cost-effective care and outcomes for all mothers and babies in the US.
Sincerely,

Citizens for Midwifery Board of Directors
Susan Hodges, President
Arielle Greenberg
Carolyn Keefe
Nasima Pfaffl
Willa Powell

The Midwives Model of Care
The Midwives Model of Care is based on the fact that pregnancy and birth are normal life processes.
The Midwives Model of Care includes:

- Monitoring the physical, psychological, and social well-being of the mother throughout the childbearing cycle
- Providing the mother with individualized education, counseling, and prenatal care, continuous hands-on assistance during labor and delivery, and postpartum support
- Minimizing technological interventions
- Identifying and referring women who require obstetrical attention

The application of this woman-centered model of care has been proven to reduce the incidence of birth injury, trauma, and cesarean section.

Submitted by
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Members of the Federal Coordinating Council for Comparative Effectiveness Research, I would like to thank you for the opportunity to provide public comment on CER and the Council's activities.

My name is Hazel Moran, and I am from Mental Health America, America's oldest and largest advocacy organization concerned with all aspects of America's mental health. In fact we celebrate our Centennial this year. Given our longstanding interest in the public's health we are the founder of the National Working Group on Evidence-Based Health Care (The Working Group), which is a collaboration of approximately 40 patient and consumer organizations, professional societies, providers and other interested stakeholders that want to help shape the initiatives in evidence-based healthcare (EBH) in order to close the gap between our knowledge

and ordinary practice. As such, I am pleased to speak on behalf of consumers and patients in support of comparative effectiveness research.

Since 2005, we have worked to broaden participation of patient groups in all aspects of EBH to improve the usefulness of this information for consumers and clinicians as well as to assure that the locus of decision making remain with the patient and his/her clinician. We focused principally on comparative effectiveness reviews and evidence dissemination as two key components of the EBH movement.

Through information exchange and engagement in public dialogue, the Working Group has emphasized the importance of balancing an understanding of scientific research with real-world clinician expertise, each individual patient's needs and history as well as their perspectives and preferences. The Working Group strives to empower patients and consumers by involving them in designing and prioritizing research, as well as reviewing evidence and contributing to its translation, dissemination and implementation. For example, Mental Health America participated in the review and dissemination of several publications including AHRQ's Summary Guides on the Off-Label Use of Atypical Antipsychotic medication and the treatment of Depression. Our involvement has been viewed as constructive and helpful we would like to continue to engage in the growing federal CER effort by helping to deepen patient involvement in CER. To generate the balanced and practical clinical evidence that the legislation envisions, it will be critical for the Council to ensure that all those conducting federal CER incorporate the patient and clinician perspective into the direction and design of CER.

The creation of new evidence and new decision support techniques to inform patient's treatment decisions offer great potential to improve care. To help realize this progress, patients and consumers can provide important support to federal CER research in three principal ways.

First, they can advise on topic selection. Second, by specifying the information they need to make decisions about their care, patients can help determine what data should be captured in CER. Third, patients can provide valuable public support for the CER dissemination and implementation.

In the Federal Register Notice for this meeting, you specifically asked for individuals making comments to respond to six questions. We would like to respond to those questions with three recommendations:

- **Create a national citizens' advisory board to help HHS** better understand the perspectives and values of the general public when designing and disseminating CER. This is critically important to make sure that the full continuum of issues are considered when making policy recommendations regarding the implementation of CER findings. A good model for this is the Citizens Council that the UK's NICE convenes.[2] NICE charges the Council with conveying the views of the public both to the Institute and to the groups that NICE commissions for appraisals and clinical guidelines.
- **Establish an explicit channel for patients to advise HHS on CER.** The FDA's Patient Representative Program is an excellent model for this kind of participation.[1] The program educates patients to be effective advisors, and then includes them on advisory boards that

correspond to their particular medical experience. HHS could similarly inform patients on some basic scientific and methodological concerns of CER and then solicit their input. Additionally, these patients must be trained and supported to effectively engage in this process. HHS should, as part of the patient advisory function, design and implement a systematic program to educate and support the patients so that they can effectively engage in these technical discussions. The establishment of an HHS Office of Patient Values and Inclusion can ensure that patients assist in all aspects of developing the systems to deliver therapies for the major medical problems we face as a nation, managing chronic disease in an era of discovery. More recognition of these patient assets is needed along with support for training and development. The establishment of such an Office can assure this.

- **Sponsor research that is designed to improve clinical decision making by both clinicians and patients.** Research that considers the individuality of health conditions and factors such as family history, individual experience in treatment and patient values and preferences is needed. Designing CER trials that mine the heterogeneity of response for identifiable sub-groups and that include outcome variables that are aligned with patient preferences should improve the clinical applicability of the work. Patient involvement in trial design would assist in improving the relevance of their findings. Integration with the developing HIT architecture should ensure that decision support tools informed by CER and systematic reviews are included in this new technology – moving knowledge to the bedside in order to craft individual treatment plans for individual patients.

Greater roles for patient advocates are essential to advance medicine in collaboration with scientists and physicians to interpret the effectiveness of therapies. Understanding the experiences of patients and the public is important to helping organizations work more effectively to evaluate and improve services. People should have the opportunity to be actively involved in shaping the organization and the delivery of health and social care services for their communities. Changes that are made to the delivery of services should be based on their experiences and views.

The National Working Group on Evidence-Based Health Care appreciates the opportunity to provide the Federal Coordinating Council on Comparative Effectiveness Research with specific comments on its activities as it relates to comparative effectiveness research. The Working Group plans to submit formal comments to the Council with further detail on our recommendations.

Submitted by
Perry D Cohen, Ph.D.
Director, Parkinson Pipeline Project
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My diagnosis with Parkinson's disease 13 years ago directed my life toward developing new roles for patients in medical research and health care. Previously, my doctoral level business school education focused on systems analysis and organizational development and 20 years experience as a management consultant gave me wide exposure to health care and medical research at all levels and with many vantage points. What I did not already know about dynamic systems approaches to management of quality service systems, I learned from active participation in the IOM roundtable on evidenced based medicine, which has detailed the concepts of "learning health systems." These pathways to transform health care provide a context for CER priorities. In earlier priority setting comments I have focused on the role of the patient with serious chronic illness as distinct from the role of consumer with interests in public safety, and suggested that patients empowered by IT via information access and networking with peers are underutilized in our provider oriented delivery system.

My vision for future learning health care systems puts an emphasis on the role of disease oriented sub-specialists as leaders and educators to not only conduct rigorous randomized controlled studies for regulatory approval, but at the same time to provide the 'academic detailing' for community doctors in the context of clinical trials in continuous processes of introducing new therapies (medical innovation). The focal point for from medical research to health care is the community network of providers who treat chronic diseases like PD and provide the bridge to link innovation and quality in knowledge transfer both ways between the sub specialist academic knowledge leaders and the patients

Patients and their doctors will be involved in the system by aggregation and incorporation of electronic medical records (EMR) of physicians and personal heath records (PHR) of their patients into a data base or a distributed network of data elements that will enhance the available information for both doctors and patients through regular updates of data and downloading of research findings as they become available. Issues of privacy and identity will be addressed and ways to enhance communications between doctors and patients will be tested. Data generated will be available for tracking safety over the life cycle of a medical product, quality and utilization management, as well as observational research and other patient oriented activity. Problems of implementation of IT solutions and managing the change process can be addressed through demonstration projects.

In spite of the promised benefits to both cost and quality of medical care, US providers have been slow to adopt these information technologies. The ARRA recognizes the lack of incentives for participation and allocates major funding to buy cooperation from reluctant providers. Care must be taken in implementation of such an approach that we do not merely automate our sub-optimal system. The way to avoid this is to pay for creation of system improvements as well as incentives for providers.

Key among the system improvements, especially given the immediate scale-up of activity

necessary to provide short-term economic stimulus through longer term investments in technology is to enhance the roles of patients and patient advocacy organizations to keep policy decisions focused on patient relevant goals, and enhance the capabilities of these organizations to evaluate and use these technologies. This human resource capacity development will be necessary to gain the trust of patients being asked to share their case history for the common good regarding maintenance of privacy and confidentiality of personal data that can be used against them. Disease specific patient advocacy organizations are among the few kinds of organizations that are trusted by patients to represent our interests faithfully in the execution of privacy and confidentiality standards. The establishment of trust of patient groups should be a major criterion for the designation as a gate keeper to control the collection and aggregation of patient data, whether the patient organization elects to take on this role itself or endorse a third party with greater technical capability. Our experience with implementation of advanced telecommunications networks over the internet indicates that even large non-profit mission-driven advocacy organization generally lack the technical skills and IT capacity to perform the transactions necessary to maintain the data flows required to make optimal use of the data. These facts point to high priority investments in infrastructure development effort up front to enhance the capacity of patient organizations to understand the value and manage the powerful HIT tools expected. Existing coalitions of patient groups, such as the Working Group that have been set up to represent patient interests are ideal for this development activity

Experience from the unintended consequences HIPPA, designed to protect patient privacy, illustrate the difficulty to pre-specify all the special cases where the rules do not apply or have negative effects. Training and development of patient organization boards and executive staff about how to evaluate the HIT implementation plans, including case examples, for their decisions regarding endorsement to their members and their own role in as gatekeeper to represent interests of their constituents

Similar to the key role of patient organizations to make the on-going judgments about standards for data exchange and aggregation, trustworthy input from specialty professional organizations with expertise to define and update knowledge structures, and validate results from aggregate data is necessary to make this process credible and meaningful. Investments in human resources and expertise to manage processes for gaining consensus on technical issues among specialty doctors and patients are necessary to make the systems adaptable over time.

Submitted by
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The American Association of Naturopathic Physicians (AANP) applauds the Obama administration for its commitment to outcomes research and determining best practices. We recommend that this perspective include the evaluation of alternative systems of healthcare. While conventional medicine's focus on treatment of disease has produced many benefits—especially in acute and life-threatening conditions, its effectiveness for the treatment of chronic

disease or the promotion of health is limited. This failure has been a major cause of the out-of-control healthcare costs plaguing the nation.

We propose a project that tests a new model of primary care; one that incorporates the best of what both conventional and complementary and alternative medicine can offer and focuses on prevention, health promotion, and treatment of the whole person. This project involves taking advantage of health information technology and electronic medical records in a practice-based research network (PBRN) containing both conventional and naturopathic medical practice. This PBRN will provide a laboratory to compare "real world" effectiveness and cost-effectiveness, initially in the area of type 2 diabetes.

There is at present a severe shortage of primary care physicians. A number of proposals have been put forth as to how to fill that shortage and many of these are aimed at increasing the number of medical students going in to primary care. However, there are also a number of proposals to utilize the skills of other licensed healthcare practitioners and expand access to patient-centered primary care in both federally funded and private health care offerings—e.g., naturopathic doctors (NDs), nurse practitioners, physician assistants, and health coaches.

Naturopathic medicine is a system and philosophy of medicine that has been in use for over 100 years. The AANP represents licensed naturopathic physicians who are trained at fully accredited four-year residential medical programs. Graduates of our medical schools serve as primary care physicians and have the same training in the basic and clinical sciences as conventional medical doctors. In addition, NDs receive training in a variety of core treatment methods including nutrition, botanical medicine, homeopathy, pharmacology, physical therapy, and minor office surgical procedures. Some licensed naturopathic physicians are also trained in traditional Chinese medicine, acupuncture and Ayurvedic medicine as well as clinical specialties such as natural childbirth. Therefore, naturopathic medicine and NDs provide the ideal laboratory to test a complement to the present primary-care-deficient model of health care.

Researchers at naturopathic academic clinics initiated a practice-based research network (PBRN) and are proposing to expand this network in collaboration with Oregon Clinical and Translational Research Institute (OCTRI) at Oregon Health & Science University (OHSU), Kaiser Permanente, and the University of Washington School of Public Health. The PBRN will also include a number of naturopathic private practices. The data coordinating center will be the newly established center for clinical informatics at the Helfgott Research Institute, National College of Natural Medicine (NCNM). EMR systems compatible with the data in the OCTRI data warehouse will be implemented at participating naturopathic institutions and practices. The EMR system under consideration is EPIC, the same system as used by OHSU, Kaiser and University of Washington clinics. The Helfgott clinical informatics center will coordinate system and coding compatibility among the ND agencies and OCTRI. It will work with OCTRI to securely archive the growing database while making de-identified and customized datasets available to researchers. The proposal calls first for retrospective and pilot prospective studies to test the feasibility of protocols based on an informatics approach to naturopathic medicine outcomes research and to characterize the populations before going on to more definitive study. If there are positive results with comprehensive treatment, the treatment and the outcomes data can be examined for factors associated with improvement.

We propose as our initial disease focus to study type 2 diabetes (T2D). T2D is epidemic and often inadequately controlled (only 37% in adequate glycemic control) by approved treatments. Nearly half (47.9%) of US diabetes patients also use complementary and alternative medicine (Garrow & Egede 2006; Yeh, Eisenberg, Davis & Phillips, 2002). The Diabetes Prevention Program (DPP) conclusively demonstrated that diet and lifestyle changes could prevent diabetes more effectively in a susceptible population than metformin (58% reduction in incidence versus 31%). The DPP lifestyle intervention used lifestyle coaches and a focus on weight loss and exercise, and although it proved less expensive per case of diabetes prevented than pharmacological management alone, it still presents a substantial cost to implement. ND practices will be used to test modifications to this model that include a primary care setting allowing diabetes prevention to be incorporated into patients' overall focus on health. It may be even more effective, e.g., through the inclusion of nutritional supplementation (Bartlett & Eperjesi, 2008; Farvid, Jalali, Siassi & Hosseini, 2005; Bonnefont-Rousselot, 2004)), and potentially, more cost-effective.

Diabetes is paradigmatic of endemic chronic diseases for which NDs have specified well rationalized treatment protocols of which the elements, individually, show promise but which have not been tested in combined practice. A pilot comparative effectiveness study of naturopathic medicine in T2D is currently underway in Seattle.

It is also important to stress that this PBRN can be used to test components of primary care that can also be incorporated into conventional practice. Diabetes is an important chronic condition but is only one of many conditions toward which the informatics-based research infrastructure that we propose can be targeted. Once the system is established for diabetes, it can be extended to any health condition. For prevention studies, long observation periods will be required and the informatics system developed under this proposal will be in place for such studies. The functions of the naturopathic PBRN and its data coordinating center will include recruitment of participating NDs, aiding in the selection and implementation of EMR systems, prioritizing research questions and developing research protocols, facilitating ethical approvals as well as the extraction, compilation, analysis and archiving of naturopathic clinical and economic data parallel to standard conventional medical data.

Summary

We propose the establishment and funding of a PBRN that includes both conventional and naturopathic primary care physicians to act as a laboratory to test components, approaches and models of primary care that can include the best of what conventional and complementary and alternative medicine have to offer. The PBRN will initially address Type 2 diabetes, but can also be used to determine the best approaches to general primary care/family practice, pain, and other chronic disease conditions.

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Submitted by
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Marshfield, Wisconsin

Thank you for inviting me to speak today. My name is Justin Starren. I am a physician, who specializes in a field called biomedical informatics. I make my living, by researching how the availability and presentation of information to patients, physicians, nurses, and other members of the healthcare team can improve healthcare delivery with respect to its patient-centeredness, effectiveness and efficiency, timeliness, safety, and equity.

I work at Marshfield Clinic, the largest private group medical practice in Wisconsin, one of the largest in the United States, with 783 physicians representing more than 80 different medical specialties, 6,490 additional employees, and more than 45 locations spread across over 25,000 square miles. The reason that I left my position at Columbia University in New York, three years ago, is that over the past 40 years, on its own, Marshfield Clinic developed and acquired sophisticated tools, technology, and other resources that complement and support the population health management mission and strategy of the Clinic. These include an electronic medical record, a data warehouse, an immunization registry, a telehealth network, and an epidemiological database that enable research studies of both the effectiveness and cost of healthcare interventions. All of these components together comprise our electronic health record (EHR). Our electronic health records go back to 1960 and contain 9.1 million patient-years of data. In 2007 we closed the last of our paper chart rooms and are now essentially paperless.

The fact that we are essentially paperless was not our goal we have proved beyond a shadow of doubt that an effective electronic health care record partnered with the right clinical care systems allows us to achieve the “holy grail” of comparative effectiveness research: to improve our quality of care while simultaneously reducing costs. And if you have doubts, which you should, simply ask CMS about Marshfield clinic's performance in the Physician Group Practice Demonstration Project.

The reason I am speaking to you today is to strongly encourage you, as you look at comparative effectiveness research, to include the evaluation of the comparative effectiveness of different types of EHR-mediated interventions. I feel compelled to speak about this for two main reasons: (1), my concern about the narrowness of CBO's definition of comparative effectiveness; (2), my concern that people are considering the value of electronic health records without understanding the totality of what an effective system does for the healthcare delivery team, from patient to physician.

The CBO defines comparative effectiveness as "a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients."

Practically, this is interpreted to mean drugs, devices and surgical procedures. I fully support the notion that this research requires rigorous evaluation. But we need to expand this definition to include electronic interventions that are only possible through an EHR.

Most of acute healthcare delivery today occurs at hospitals which serve as a nexus of multitudinous, complex, competing, reactively configured, poorly coordinated processes, directed toward a person, who in this context, represents a complex, physiologically unstable (acutely ill) organism. Furthermore, this care is delivered by a team whose membership not only changes by the hour and whose members are frequently unfamiliar with each other, and consequently who operate under wide ranging assumptions about the status of the patient and their role in at patient's care. In short, poorly informed patients are being treated with poorly informed processes administered by poorly informed nurses directed by poorly informed physicians, supported by poorly informed laboratory, diagnostic, and pharmacy services. Unless we address this context in an energetic, direct, and rigorous manner the benefit of knowing the most effective treatment for a medical condition in a given set of patients will be severely limited.

The context of preventive and subacute healthcare delivery is not any better. This portion of healthcare delivery occurs in an ambulatory care environment that is characterized by competing provider organizations, who incompletely share information about patients. Information that requires expertise in using a highly specialized vocabulary and set of concepts that frequently intimidates patients and their families, without imparting much understanding, let alone the ability to communicate that information to multiple providers.

Healthcare delivery cries out for applied research, comparative effectiveness research that evaluates the impact of different options for providing timely, accurate, understandable and actionable information to all members of the healthcare team, from patient to provider. Hence, my concern that many who are evaluating the value of electronic health records in the context of healthcare delivery reform do not understand the totality of an effective electronic health record.

We are not talking about the importance of an electronic version of the written medical record. We are not talking about a bridge from the billing system to the clinical care system, which is where many of the commercial electronic health records started. We are not talking about an electronic tool that provides non--essential convenience to patients or providers. Quite frankly, the current national discourse on value of EHRs at the level of asking whether “EHRs improve quality and reduce cost.” This is equivalent to asking whether “drugs” treat “disease.” From comparative effectiveness research we know that optimal care is only achieved if we:

- Know what disease we are treating;
- Choose the right drug;
- In the right dose;
- Administered at the right time; and,
- Delivered through the right route.

We also know that if we get any of those factors wrong, we may not only fail to cure the patient, we might make them worse.

The same is true of EHRs. There are many different EHRs on the market. Each one can be configured in many different ways.

At this point, the literature on the impact of EHRs is largely anecdotal. It has been well demonstrated that some electronic interventions at some institutions improve quality. In other setting, seemingly similar interventions show no effect. There have also been widely publicized cases where the quality of care was negatively impacted. Unfortunately, the current state of our knowledge of the interaction between EHRs and quality is analogous to the days when most medical journal articles were single case reports or small series.

By using and integrating EHR tools into rationally designed care and care measurement processes, Marshfield clinic, through it's participation in the CMS PGP Demonstration Project saved CMS over $25 million in the first two years of this demonstration, while meeting or exceeding 27 out of 27 possible quality metrics. We are just scratching the surface of what can be done.

Instead, the national discourse on EHRs and health care quality should be focused on what are the attributes and relative value of electronic systems and tools that effectively and efficiently capture clinical data; provide decision support at the point of care; help convey true understanding and meaning to patients about what they are experiencing; assists patients in making truly informed decisions about their health care; in a manner that engages its patients in a way that motivates advancement of healthy behaviors; and, the nation understanding the performance of our health care system in rightly caring, in an effective, efficient, and compassionate manner for its citizens.

Submitted by
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Dear Sir or Madam:

On behalf of the United States Psychiatric Rehabilitation Association, I would like to submit the following statement for consideration by the Federal Coordinating Council on Comparative Effectiveness Research.

Psychiatric rehabilitation principles and psychosocial supports have been successfully employed for more than 30 years to bring about recovery in individuals with mental illnesses. Psychiatric rehabilitation, including such modalities as supported employment, supported education, and assertive community treatment, have assisted individuals to live a meaningful and satisfying life in the community. Psychiatric rehabilitation promotes recovery, full community integration and improved quality of life for persons who have been diagnosed with any mental health condition that seriously impairs their ability to lead meaningful lives. Psychiatric rehabilitation services are collaborative, person directed and individualized. These services are an essential element of the health care and human services spectrum, and should be evidence-based. They focus on

helping individuals develop skills and access resources needed to increase their capacity to be successful and satisfied in the living, working, learning, and social environments of their choice.

USPRA would like to see a portion of the $400 million in funding for the National Institutes of Mental Health allocated to comparative effectiveness research on crisis residential services as an effective alternative to psychiatric hospitalization. Current studies in this area suggest crisis residential services result in more successful outcomes and a quicker return to the community than traditional psychiatric hospitalization. In addition, USPRA recommends comparative research is developed to examine the degreed to which psychiatric rehabilitation services reduce the use and cost of hospitalization and medication.

Thank you for your consideration.

Submitted by
Alan Mertz
President
American Clinical Laboratory Association
Washington, DC

I am Alan Mertz, President of the American Clinical Laboratory Association. ACLA members develop and perform laboratory testing providing physicians with information that is central to the prevention, diagnosis, treatment, and management of virtually all disease. Laboratory testing provides critical information in 70% of health care decision making, yet spending on laboratory services accounts for less than 3 % of U.S. national health care expenditures.

Laboratory testing is one of the most cost-effective components of our health care delivery system. For example, consider that a *six dollar* glucose test is the primary screening tool for prevention and/or early diagnosis of diabetes – a disease left undetected and untreated that will cost tens of thousands of dollars and untold suffering. While more complex genetic and molecular tests cost more, they often have an immediate substantial benefit for prevention, targeted treatment and management of disease.

There is growing interest in better evidence to support relative benefits and risks of alternative interventions used to treat particular health problems, to drive more efficient use of limited health care resources. Proponents contend that such evidence can contribute to more efficient use of limited health care resources. Although the greater emphasis of CER by far has been on therapeutic modalities, laboratory testing is integral to CER. Laboratory values are essential in CER, including identifying patients to be studied in CER of therapeutic interventions (e.g., to be enrolled in clinical trials or for other prospective or retrospective studies) and for quantifying baseline characteristics, assessing intermediate outcomes, conducting subgroup analyses, and more. You cannot measure the comparative effectiveness of treatments & outcomes for the major chronic disease cost drivers (heart disease, diabetes, cancer, kidney, etc) without utilization of laboratory services. In fact, studies have shown that lab testing is underutilized for these diseases. Other key considerations for CER are the availability and use of laboratory testing data in registries, claims databases, electronic health records, and other sources. There is also increasing interest in CER of diagnostic technologies, with some attention to laboratory

testing. Certainly, some of the new, genomic-based tests could become subject to CER themselves.

An important consideration of CER involving laboratory testing is the relationship between CER, which is typically a population-based form of inquiry, and personalized medicine. Based on growing knowledge about inter-individual genetic variation that influences drug selection, laboratory testing is helping to determine how specific patients are likely to respond to a given drug. It is of significant importance that as CER develops, it will not diminish or counteract progress in personalized medicine, but be able to integrate protocols and study technology that will accelerate this new frontier of medicine.

Another caution -- comparative effectiveness will be a failure if it looks at health care spending in individual silos and uses short time horizons. Comparing different laboratory tests without looking comprehensively at the "costs" (in dollars, health, and quality of life) of failing to prevent or diagnose disease early is bound to be a disaster. If CE looks only at a 5-10 year horizon, the cost of the tests and resulting preventative treatments might look high. Early screening, prevention, early diagnosis, and effective early treatment using the best technology available sometimes costs a more in the shorter (or even medium) term than doing nothing. It will be an error if CE programs stifle these approaches by limiting options and care. Continued innovation requires adequate reimbursement of the current technologies to finance research on the new technologies.

I hope you agree it is important for the clinical laboratory industry to be part of the equation in CER programs. We stand ready to support your efforts in all ways possible.

Submitted by
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Our major recommendation is to move beyond traditional approaches and define comparative effectiveness as the approaches that yield actual improvement of quality on the ground, not simply more standards that take 17 years to implement.

Next Wave is one of 165 Supporting Organizations for **Stand for Quality.** To paraphrase function 6 in its recently released report "Building a Foundation for High Quality, Affordable Health Care: Linking Performance Measurement to Health Reform" - Comparative Effectiveness Measurement should not an end unto itself. An appropriate role for the HSS Office of the Secretary is to support evaluation of how best to get the tools developed by NIH and AHRQ into routine use by practitioners in their daily practices.

Our current health care system does not do this well. Only slightly more than half of existing best practices are actually implemented. Simply creating additional best practices as we have

done traditionally will give us more of the same. What is needed is innovation and increased focus on non-traditional approaches that have been overlooked in bringing standards into use:

- Evaluate better ways of engaging patients in their own chronic care.
- Evaluate which types of community grass roots efforts have achieved rapid and effective implementation for their types of communities (homogenous middle class, diverse immigrant populations, rural, inner city, etc.)
- Patient centered/cross agency (e.g. health, education, environment for children's environmental health issues)

Submitted by
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The American Heart Association (AHA) and its Division – the American Stroke Association – appreciate this opportunity to inform the work of the Council on comparative effectiveness research (CER). We share with other stakeholders – Congress, the Administration, healthcare professionals, policy-makers and, most importantly, patients – the conviction that this is a very important initiative.

The Association feels that our contribution to this key discussion is apropos for three reasons:

- The AHA has 'standing' in this matter, because we represent patients confronting medical problems of massive personal importance and public scope.
    - The Association is the oldest, largest voluntary health organization having as its sole Mission the "build(ing of) healthier lives, free of heart diseases and stroke".
    - Despite considerable progress, cardiovascular diseases and stroke remains the number one and number three killers of residents of the United States. Coronary heart disease alone accounts for one in five deaths. The total direct and indirect costs in 2009 are estimated to be $475.3 B.
- AHA professional volunteers are recognized leaders in research pertinent to this consideration. Last year, the Association invested $160 M, out of $642M in total revenue on research, second only to the Federal government as a funding source for cardiovascular diseases and stroke.
- That AHA is not conflicted. The Association does not accept government funding. Our relationships with industry are limited and fully disclosed on our websites. The primary source of our funding is the individual donations of millions of U.S. residents.

We commend the Administration and the Congress for taking an essential first step in creating an effective CER enterprise.

The Association has developed a document, scheduled for publication next month in *Circulation*, that articulates our principles for CER. They are twelve in number, organized into four broad categories.

1. CER should be conducted and interpreted according to fundamental, established and accepted scientific principles. Analyses need to be disseminated quickly, clearly and effectively to a wide variety of target audiences to have the desired impact. The determination of cost effectiveness requires the same scientific rigor as the determination of clinical effectiveness.

    a. Randomized controlled clinical trials determine efficacy and safety, which forms the basis for CER; several – CASS, TIMI, NASCET, ACAS, WASID, BARI and WARSS – are all landmark clinical effectiveness trials that meet the definition of CER.

    b. Some important questions, however, must be addressed in larger, more diverse patient populations. Different types of evidence are required to do this most effectively, e.g. evidence gathered from registries and other observational studies. This type of evidence may be a better representation of 'real world practice' and is often less expensive and time-consuming to perform, but it is also subject to important methodological concerns:

        - the role of chance;
        - the impact of selection, recall and protopathic bias; and
        - uncertainty re: extent of exposure, including misclassification of data.

    c. It is essential that all stakeholders understand the limits of every type of evidence considered and that that understanding in incorporated into decision-making at every level, from the individual clinical encounter to coverage decisions made by public and private insurers.

2. The goal of CER should be to determine the **value** of an intervention for patients.

    a. Research should define the most important metric: value. Value is the change in key patient-centered variables – clinical outcomes, quality of life and patient satisfaction – per unit investment. Determination of cost-effectiveness alone is insufficient to define value and may lead to unintended consequences: adverse clinical outcomes; and limited innovation. It is necessary to compare both clinical and cost effectiveness to determine comparative value.

b. Both comparative clinical effectiveness and cost-effectiveness need to be considered in the long-term. A focus on short-term gains alone is likely to have a particularly untoward effect on the wellness and prevention agenda.

c. CER, with its focus on the application of drugs and devices already developed and approved, is compatible with innovative healthcare research. All stakeholders should support limiting the use of high-cost products that are shown to be of marginal benefit from the perspective of individual patients.

3. CER should be applied to the care of individual patients, but should not be a substitute for sophisticated clinical judgment. Priority should be given to evaluating interventions that pertain to high-volume, high-cost chronic conditions; reduction of healthcare disparities; and filling gaps in current evidence-based guidelines.

4. The CER process must be transparent, fair and accountable so that findings are credible and actionable.

   a. Structure – governance and funding – are key. It must be inclusive, comprehensive and resistant to inappropriate influence from the public or the private sector. Apparent conflicts of interest raise skepticism and concern about participation by industry, despite its constructive and necessary role in the care process. Experience with the National Center for Health Care Technology, the Office of Technology Assessment and the Agency for Health Care Policy and Research underscore that this is more than a theoretical concern. It must – knowledgably and candidly – advance and balance the concerns of the individual patient and the public's health.

   b. A larger investment in CER will be required to realize its full potential.

   c. CER should not be funded at the expense of traditional forms of research that are supported through the National Institutes of Health, other public agencies and the private sector.

The Association has applied that position statement to the six questions posed by the Council on 4-10-2009 (*Federal Register* 2009;74(68);16398-99).

1. The Council should invest in the development and application of electronic health records, scientifically sound research methodologies, and a research workforce with the necessary size and skill set. This will require consensus on balance between utility and privacy/security. The Council should quickly develop and implement a robust system of evaluation to accurately and completely evaluate the impact of this initial investment and to direct future investment.

2. Short-term gain is more likely to be achieved by focusing on identifying and leveraging existing resources. Long-term gain will result from a careful consideration of priorities, coupled with better ways of estimating savings over time. Investments – both short and long-term – should prioritize the following: high-volume, high-cost chronic conditions; health care disparities; and evidence gaps in current clinical guidelines. These are not mutually exclusive, but need to be balanced, especially in terms of managing stakeholder expectations.

3. The Federal Government has a central role to play in CER for two reasons: it provides many of the services that need to be studied; and it has both a statutory and a moral obligation to advance the public's health. Development of infrastructure and of a process for prioritization are a good place to start.

4. Efforts limited to the public sector may be easier to implement rapidly and have the scope to inform broader efforts if the process is structured as an active learning system.

    a. Particular attention should be paid to the development of unintended adverse consequences, such as increasing health care disparities or limiting innovative research.

    b. Coordination of activity within the Federal Government is crucial for maximum effectiveness, especially over the short-term; the DHHS seems well-suited to performing this coordinating function, especially given the distribution of ARRA '09 funding.

5. A crucial step in advancing the CER enterprise is the establishment of a public-private partnership with transparency in governance and funding and conducted in a manner that is inclusive and fair.

6. Two actions would increase understanding and trust within the broader community: establishment of an utterly transparent process, with realistic opportunity for public response; and a clear statement of whether or not the Federal CER enterprise will inform coverage decisions made by the Government or make those decisions itself .

Thanks you for inviting the Association to participate in today's discussion. We look forward to working closely and constructively with the Council on this important issue. These public meeting represent a good start in making use of the nation's initial, though substantial, investment in CER.

Submitted by
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The purpose of this letter is two-fold. First on behalf of Nutricia North America (Nutricia) a leader in clinical nutrition, I would like to take this opportunity to commend all of you on being selected to serve on the Federal Coordinating Council on Comparative Research. The roles you play are paramount in helping our country's ongoing efforts to improve the quality of care in a cost-effective manner.

A leader in clinical nutrition, Nutricia North America specializes in development and sale of "medical foods" and specialized orphan infant formulas for the nutrition therapy of rare genetic, metabolic, allergic gastrointestinal and neurological disorders. The term "medical food" as defined in section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3)) is "a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation " [1].

"Medical foods are distinguished from the broader category of foods for special dietary use and from foods that make health claims by the requirement that medical foods be intended to meet distinctive nutritional requirements of a disease or condition, used under medical supervision and intended for the specific dietary management of a disease or condition. The term "medical foods" does not pertain to all foods fed to sick patients. Medical foods are foods that are specially formulated and processed (as opposed to a naturally occurring foodstuff used in a natural state) for the patient who is seriously ill or who requires the product as a major treatment modality."

The use of food in the treatment of disease was recognized as early as the 4th century BC and was a key component of the classical Hippocratic Oath. In that version of the oath, physicians were sworn to apply dietetic measures for the benefit of the sick. More recently, the value of medical foods for the treatment of certain conditions like Phenylketonuria (PKU) has become widely accepted as a standard of care.

Over the past 20-30 years, medical foods have played an integral role as primary therapy in many genetic, gastrointestinal and neurologic disorders in infants, children and adults. For example, in individuals with PKU, newborn screening, early diagnosis and use of medical foods early in infancy has prevented permanent retardation and improved quality of life in thousands. With proper use of medical foods, individuals with PKU are now enjoying a normal high quality of living. In patients with seizures unresponsive to drugs, medical foods, as part of a special diet, have been shown to either eliminate seizures completely or significantly reduced the number of seizures per day. Regardless of these outstanding medical benefits, knowledge of the efficacy of medical foods, as a cost-effective treatment of various diseases and conditions remains minimal at best in the U.S.

While no one can deny the impact that pharmaceutical drugs like antibiotics have had on the eradication of many diseases and the overall quality of health in general, clinical nutrition (medical food) as treatment continues to wage an uphill battle against a "Pavlovian" conditioned medical philosophy of a "pill for every disease". Medical foods, as either the primary or partial therapy in managing chronic diseases remain overshadowed by large pharmaceutical companies. In all cases where medical foods are used as therapy the cost is minimal when compared to medications. We agree that there is a important role for pharmaceuticals in treatment to many disorders, but where there is a role for medical foods, they should be considered as part of the therapy.

We strongly believe that there is room for both pharmaceuticals and medical food treatments in specific diseases where medical foods can play an integral role. More importantly, we believe patients should have choices in their treatments, as long as those treatments are found to be comparable.

As such, we welcome comparative effectiveness research studies that include comparisons of medical foods to pharmaceutical drugs especially in the treatment of pediatric epilepsy, pediatric food allergy, genetic metabolic diseases, adult diabetes, and Alzheimer's disease.

Thank you for your time and consideration in this important matter. Please feel free to contact me if you have any questions. We look forward to the progress made by your committee.

A leader in clinical nutrition, Nutricia International specializes in the manufacture, marketing and (mail-order) distribution of medical foods and infant formulas for the dietary management of rare genetic, metabolic, allergic gastrointestinal and neurological disorders.

Submitted by
Jennifer L. Reck, MA,
Policy Director, Prescription Policy Choices
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Dear Council Members,

Comparative effectiveness research will realize its potential to improve the quality of health care in the United States only to the extent that it is effectively disseminated to health care providers. Even the best, most objective data needs to be actively championed in order to make sure it is heard. The relatively limited impact of the federally-funded hypertension trial known as ALLHAT is a sobering reminder of that fact.[i] A large investment in research is ultimately wasted if not matched with a proportionate investment in effectively communicating the results.

Prescription Policy Choices urges the Council to prioritize adequate funding for the dissemination of comparative effectiveness research, including determining the most effective means for educating adult professionals.

Prescription Policy Choices is a national, nonprofit, nonpartisan, educational and public policy organization which provides objective research and expertise on prescription drug policies. We have worked with many states establishing and administering prescriber education programs (also known as academic detailing). Our white paper on this topic is available at: www.policychoices.org.

Submitted by
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Consultant
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Good afternoon, and thank you for the opportunity to speak this afternoon. On behalf of the Friends of SAMHSA, a non-profit organization dedicated to advancing SAMHSA's mission and visibility, I will be addressing the importance of comparative effectiveness research in behavioral health.

We recognize that it is not the charge of this council to recommend clinical guidelines for health care payment, or coverage or treatment. Yet in considering the needs of populations served by federal programs and opportunities, this council will recommend how to build and expand on current investments and priorities.
We appear today to urge the members of this committee not to overlook comparative effectiveness research in the area of behavioral health. Treatments for mental illness and substance abuse disorders must be studied and evaluated. Tens of millions of people require treatment for behavioral health disorders, so therefore much is at stake when determining how best to treat this population.

The Department of Health and Human Services has rightfully received a substantial amount of funds from the 2009 Economic Stimulus legislation recently passed by Congress and signed by the President. We all know that a healthy populace is a productive one, and economic growth cannot be initiated or sustained if the workforce is not well cared for.

Unfortunately, there is much to be disappointed about as well. While many HHS agencies will benefit from the funds allocated by the stimulus bill, there has been a glaring omission. The Substance Abuse and Mental Health Services Administration (SAMHSA) is not slated to receive any funding. As a result of this oversight, it is critical that we address behavioral health when setting priorities for comparative effectiveness research. If our country is to achieve its goal of a healthier and more productive society, behavioral health issues must receive the same attention as other chronic and acute diseases.

Because the issue today is funds from the economic stimulus bill, and the monies to be spent on health in conjunction with economic growth, I wanted to place on the record the economic cost of behavioral health disorders. In lost productivity, in emergency room visits and other health costs, the price tag is hundreds of billions of dollars. Also, consider the critical education that our children are deprived of when behavioral health disorders go undiagnosed and untreated. Untreated children face the high probability of becoming uneducated. Uneducated children leads to a weaker workforce. The link between effective behavioral health treatment and economic prosperity is not, by any stretch, a tenuous connection.

There are countless numbers of health disorders and diseases that require the attention of our government. Each and every one should be addressed using only the most effective treatments and prevention strategies. Yet in setting priorities, difficult choices must be made. It is hard to ignore the sheer numbers of those that require treatment for behavioral health disorders, as it is hard to ignore the money that can be saved with effective prevention and screening strategies.

The landmark mental health and addiction parity legislation that became law late last year has shown that our government is finally willing to take the bold action needed when it comes to behavioral health. Yet more action is required. Please, do what must be done in order to ensure that comparative effectiveness research involves behavioral health. It is a tough decision, one that may be politically or socially unpopular. But the right one.

Submitted by

Charles Homer, MD, CEO
National Initiative for Children's Healthcare Quality
James M. Perrin, MD, Director,
MGH Center for Child and Adolescent Health Policy
Boston, MA
Stephen Berman, MD, Professor of Pediatrics and Chair in General Pediatrics
University of Colorado and Children's Hospital
Debbie Chang, Senior VP and Executive Director
Nemours Health and Prevention Services
Newark, DE
Charles Bruner, PhD, Executive Director
Child and Family Policy Center,
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Christina Bethell, PhD, Executive Director
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Oregon Health Sciences University
Robert Restuccia, Executive Director
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Boston, MA

The National Initiative for Children's Healthcare Quality (NICHQ) is pleased to have the opportunity to provide input to the Federal Coordinating Council on Comparative Effectiveness agenda. NICHQ is an independent, not for profit organization committed to achieving a world in which all children receive the health care they need; our focus is to improve child health by improving the quality of children's heath care, a strategy well aligned with the purpose of comparative effectiveness research. We write today to urge you to include children's health care, broadly conceptualized, in your research agenda.

In order to develop our recommendations, we convened a policy advisory group consisting of both NICHQ senior staff and key external advisors from a broad set of organizations with deep expertise in children's heath care and public policy. The committee quickly came to a number of major recommendations for the Committee's consideration:

1) At least three compelling arguments call for including children's health care as a significant focus of the comparative effectiveness research agenda:
    a. children particularly depend on public sources of funds for their health care (Medicaid, SCHIP),
    b. the consequences of poor health and health behaviors in childhood have long term, costly implications, and
    c. the research base for child health has been historically under-funded.

2) The topics for emphasis for comparative effectiveness research in child health should reflect the unique characteristics of child health and children's health care, specifically, their
    a. Dependency on others, leading to the importance of supporting parental capabilities,
    b. Rapid pace of development, leading to the key role of developmental surveillance and preventive care,
    c. Distinct epidemiology, i.e., the increasing prevalence of a few highly significant chronic conditions such as obesity and asthma and mental health, coupled with the cumulative significance of a large number of relative rare conditions, and
    d. Relative economic disadvantage and cultural diversity
3) Relative economic disadvantage and cultural diversity Considering these characteristics, the scope of comparative effectiveness for children should extend beyond the medical system to include all areas where children live, learn and grow with a focus on assessing how they can best promote and maintain child health.

4) The comparative effectiveness agenda for children should build on the excellent work of previous Institute of Medicine Committees. We recommend the comparative effectiveness research agenda draw from the pediatric priorities articulated by the IOM Committee that established

Priority Areas for National Action. Relevant child health topics on this list included:

- Care coordination (cross-cutting)
- Self-management/health literacy (cross-cutting)
- Medication management—preventing medication errors and overuse of antibiotics
- Children with special health care needs
- Asthma—appropriate treatment for persons with mild/moderate persistent asthma
- Immunization—children and adults
- Major depression—screening and treatment
- Nosocomial infections—prevention and surveillance
- Pregnancy and childbirth—appropriate prenatal and intrapartum care
- Obesity

The key points we would emphasize in considering this list are:

a) A focus on cross-cutting themes (care coordination, family and self management support).
b) Consideration of children with special health care needs as a whole, as many of the issues in management (role of family, organization of services) are cross-cutting, as are many clinical issues (e.g., appropriate use of g-tubes or muscle lengthening procedures).
c) Attention to improving birth outcomes—and, correspondingly, care in pre-conception, prenatal, intrapartum and newborn care—as these are the biggest drivers of infant and maternal mortality and responsible for substantial health care cost as well.
d) Attention to children's mental health, both preventive and therapeutic, especially given the apparent growth in prevalence and long-term implications of mental health conditions.
e) A continued focus on both prevention and treatment of childhood obesity as this is increasingly a major driver (with smoking) of poor health outcomes.
f) A particular focus on addressing conditions relevant to populations experiencing disparate health outcomes (e.g., sickle cell disease, obesity, asthma, mental health).

We also urge a broad conceptualization of comparative effectiveness research. Comparisons of clinical treatments are necessary and valuable; comparisons of models of practice, care financing, information systems, population-based strategies, and practice supports and the like are also crucially important to

arrive at a more effective health care system for children and all Americans. Parents, patients and other consumers should be involved in the formulation, prioritization and communication of this research.

We, the undersigned (CEO of NICHQ and members of Policy Advisory Committee), are extremely supportive of a strong program of comparative effectiveness research and want to make sure that children can also benefit from this new and exciting endeavor. We are available to offer advice in prioritizing areas of children's health comparativeness effectiveness with you.

Submitted by
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I marked my 10th college reunion with a miscarriage. It was my first pregnancy, and although I was well past 30, I was not remotely prepared for the physical loss, let alone the emotional one.

I flew home to the East Coast that night. When I arrived at the registration desk of a highly regarded academic medical center for a D&C the next day, I was handed an "informed consent" document typical of the period. It included provisions to the effect that excess specimens (which otherwise might be discarded) and information about me and the care I received might be used for research and that as a patient at an institution of higher learning, I agreed to this disposition. My profound grief temporarily gave way to outrage, and I marked up the document – an occupational predilection of a young lawyer regardless of distressed mental state – to allow the hospital to bill my insurance company for my treatment but not much more. I handed it back to the receptionist, who curtly informed me that I could sign the form "as is" or be denied treatment. I had no energy to put up a fight and was in too much pain to walk away, so signed a fresh copy with some vague intention to take it up again later.

Many years (and three healthy kids) later, I understand the significance of that simple consent – or really notification – process in enabling the clinical trials, health services research, and related public health activities so critical to learning about disease, developing new prevention strategies and interventions, and improving health outcomes. I appreciate that our expectation of continuous advances in science and medicine requires some measure of sacrifice. And I now recognize that it is imperative to make the best use possible of existing data and human tissue that otherwise would be discarded, especially as health care costs soar, resources disappear, and consumers, payors, and government officials increasingly and loudly demand that health care delivery and payment systems become more efficient and effective. Barak Obama pointed to the importance of health services research during his 2008 presidential campaign in his *Plan for a Healthy America*:

> One of the keys to eliminating waste and missed opportunities is to increase our investment in comparative effectiveness reviews and research. Comparative effectiveness studies provide crucial information about which drugs, devices and procedures are the best diagnostic and treatment options for individual patients. This information is

> developed by reviewing existing literature, analyzing electronic health care data, and conducting simple, real world studies of new technologies.

Congress embraced this approach in the American Recovery and Reinvestment Act ("ARRA"), appropriating $1.1 *billion* to comparative effectiveness research and establishing a Federal Coordinating Council for Comparative Effectiveness Research to "foster optimum coordination" and avoid "duplicative efforts". The legislation requires the Council, whose members were named in mid-March, to submit its first report to Congress on June 30, 2009, and in it to describe current activities and recommendations for use of the appropriated funds.

Yet we are in real danger of squandering the tremendous opportunities those funds provide to advance scientific knowledge and translate that knowledge to medical practice. Why? Because single-issue privacy hawks have succeeded in recent years in focusing public attention and concern on individual privacy rights without regard to the consequences for society. The foundational conceptions of a civil society or the common good seem almost entirely absent – or at best an afterthought – in current academic and political discourse on the "ethics" of research with human biospecimens and personal health data.

For example, the federal HIPAA Privacy Rule, which recently has been integrated in significant part and expanded upon in federal legislation through a section of ARRA titled the Health Information Technology for Economic and Clinical Health ("HITECH") Act, do not recognize research or public health activities as integral to a functioning health care system. The Institute of Medicine is just the latest organization to investigate the challenges this rule imposes to the pursuit of research and public health activities and recommends several policy changes aimed at mitigation. I have opined elsewhere that the challenges are due largely to the failure of the HIPAA Privacy Rule to recognize research as equally integral to the delivery of health care as accreditation, peer review, quality improvement, and other activities without which there is no ultimate accountability in the health care system. This failure, in turn, results in over-valuing "autonomy," a core privacy right that the HIPAA Privacy Rule is designed to protect.

To my knowledge, the cost of this construct to advancing knowledge and discovery and informing public policy has not been systematically weighed against the questionable benefits the HIPAA Privacy Rule and similar efforts bestow on individual rights. Fred Cate's brief but cogent piece delivered to the 2007 Privacy Symposium in Cambridge, *The Autonomy Trap*, explains how our overwhelming concern with individual choice or control, reflected in laws and regulations mandating various procedural protections, does not actually do a very good job serving its intended purpose of protecting substantive privacy rights. He argues forcefully that we should permit the use of medical records for research without individual consent, by recognizing the distinction between "privacy of the body – the right to refuse treatment or to choose among medically appropriate treatments – and privacy *about* the body." Quoting from Helena Gail Rubinstein's article, "If I Am Only for Myself, What Am I? A Communitarian Look at the Privacy Stalemate," 25 *Am. J. Law & Med.* 203 (1999), Cate observes that "relying on consent refuses to recognize 'in exchange for the vast improvements in medical care, a correlative responsibility on the part of the individual, as a potential consumer of health care services, toward the community. As individuals rely on their right to be let alone, they shift the burden for providing the data needed to advance medical and health policy information. Their individualist vision threatens the entire community[.]'"

As the current Administration and responsible agencies work to develop regulations to implement the HITECH Act's (and GINA's) new privacy and security provisions, it will be incumbent on them to consider the impact the regulations will have on comparative effectiveness and other health services research, with the ultimate goal of reasonably balancing individual privacy rights against scientific discovery, biomedical innovation, and quality improvement. The stakes are far too high to continue pursuing the goals of scientific and medical advancement, on one hand, and protection of individual privacy rights, on the other, in separate silos. It is my hope that, pursued together, these initiatives will result in reasonable compromise, for example by enhancing education and transparency about research uses of secondary data (and excess biospecimens) while reducing unnecessary regulatory barriers to *bona fide* research uses of these invaluable resources.

N.B. The views expressed above are my own and are not necessarily those of my current or former employers or clients.

Submitted by
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Thank you for the opportunity to participate in this Listening Session. My name is Patricia Adkins and I am the Chief Operating Officer and the Director of Public Policy for the Home Safety Council, a national nonprofit in Washington, DC.

The mission of the Home Safety Council is to help prevent and reduce the deaths and injuries that happen in and around the home such as falls, poisoning, fires and burns, choking and suffocation, and drowning. The Home Safety Council is one of the leading public health and safety nonprofit organizations working in the injury and violence prevention and response community. Our primary goal is to improve the opportunity for all individuals to lead healthy, active, and fulfilling lives.

I believe it is imperative that the Federal Coordinating Council include public health as a priority, with a specific focus on injury and violence prevention and response, when it makes its recommendations to the President, the Congress and the Federal agencies.

Purpose and Goals of the Federal Coordinating Council

Under the American Recovery and Reinvestment Act of 2009, the purpose of the Council is to assist the Federal departments and agencies in reducing duplication and encouraging coordination of comparative effectiveness and related health services research, as well as advising the President and Congress on its progress through its initial report which is due on June 30, 2009.

By definition, comparative effectiveness research compares treatments and strategies to improve health. And by knowing what works best, clinicians and patients can decide on the best treatment. Ultimately, this research enables our nation to improve the health of communities and the performance of the health system.

The White House Forum on Health Reform

The White House Forum on Health Reform was held on March 5, 2009. The President invited a diverse group of people to participate in this event to begin the process of addressing what he called "one of the greatest threats not just to the well-being of our families and the prosperity of our businesses, but to the very foundation of our economy – and that is the exploding cost of health care in America today."

During Breakout Session One at the Forum, the Executive Summary of the discussions highlighted the following: "Nearly every participant stressed the importance of investing in public health prevention…The group agreed this that would both improve health and reduce costs."

In response to the question, "how can we contain rising health care costs," one Congressional Member observed, "the model of our system is sick care, not health care…" Other comments centered around "wellness programs that focus on managing and preventing illness…" and another "noted the importance of public health and prevention."

Why injury and violence prevention and response are important to the work of the Council?

In 1998, the National Academy of Science stated - "Injury is probably the most under-recognized public health threat facing the nation today."

Each year, injuries resulting from a wide variety of physical and emotional causes – motor vehicle crashes, sports trauma, violence, poisoning, fires and falls – keep millions of children and adults from achieving their goals and making the most of their talents and abilities.

This is some of what we know:

- Nationally and in every state in the United States, injuries are the leading cause of death in the first 44 years of a person's life.
- In a single year, more than 50 million injuries required medical attention, with an estimated total lifetime cost of $406 billion.
- This total lifetime cost includes $80 billion in medical care costs and $326 billion in productivity losses, including lost wages and benefits and the inability to perform normal household functions.

These three statistics clearly show the consequences of injuries and the major burden on the health care system.

Fortunately, because of scientific discoveries and injury research, there are steps that can be taken to stop injuries before they happen and increase the likelihood for full recovery when they do. By incorporating these strategies into the community and everyday activities, we can improve the opportunity for all individuals to lead active, useful, and fulfilling lives.

An Injury Example – Protecting Older Adults

We all want a society where people, including our older citizens, can live to their full potential. And we can help many older adults avoid injuries. There are a variety of actions we can take to prevent injuries and premature death to our parents, grandparents, and friends. Some of the most important include preventing older adults from falling or from being injured in fires or motor vehicle crashes.

Let's focus on one of the injuries that affect the quality of life for older adults – falls. Falls are the leading cause of fatal and nonfatal injuries for those 65 and older. Each year, 1.8 million older adults are treated in emergency departments. Every 35 minutes, an older adult dies from a fall-related injury and every day 5,000 adults 65 and older are hospitalized due to fall-related injuries.

The cost for treatment is enormous - over $19 billion annually; and most of these expenses are paid for by CMS through Medicare.

While falls are a threat to the health and independence of older adults and can significantly limit their ability to remain self-sufficient, the opportunity to reduce falls among older adults has never been better. Today there are proven interventions and strategies that can reduce falls and help older adults live better and longer. They include medication review and adjustment, vision correction, and physical exercise. These evidence-based interventions can help save health care costs and greatly improve the lives of older adults. The costs are small compared to the potential for savings. For every $1 invested in a comprehensive falls prevention program, it returns a $9 benefit to society.

How the Council Can Help

The Council can be a catalyst for changing people's perceptions of the value of preventing and responding to injury and violence by helping to create the social and political will to more fully support this public health issue.

Whether it's the lack of knowledge that solutions exist to reduce the impact of injury and violence; or not understanding that injury and violence are public health issues; or believing that unintentional injury is unpredictable and not preventable, the Council can address these challenges and provide a common foundation for collaboration.

The American Recovery and Reinvestment Act provides for $400 million to be allocated at the discretion of the Secretary of Health and Human Services. Incorporating the public health prevention strategies and interventions into the comparative effectiveness research is critical to developing a comprehensive plan for health reform. I urge the Council to reinforce the adoption of these public health strategies within the injury and violence prevention and response field to ensure that Americans remain healthy and live their lives to the fullest potential.

Submitted by
Stephanie Mensh, Neocure Group
On Behalf of Virtual Radiologic Corporation
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I appreciate the opportunity to provide comments on behalf of Virtual Radiologic Corporation, a provider of teleradiology services, headquartered in Minneapolis. vRad was founded by radiologists and is a leader in teleradiology, with more than 140 contracted U.S.-based radiologists, of which 70% are fellowship trained, serving 1,025 medical facilities nationwide and providing more than 2.2 million interpretations in 2008.

vRad and teleradiology provide a number of well-established healthcare system features including:

- Infrastructure for electronic and digital transmission, reading, interpretation, reporting and record-keeping for diagnostic imaging services
- Efficient and cost-effective 24/7 deployment of highly-skilled diagnostic resources
- Access by rural and small community-based facilities to specialized, subspecialty-trained radiologists
- A resource for supporting clinical registries, clinical data networks, and other forms of electronic health data that could be used to generate or obtain outcomes data.

We recognize the magnitude of the Council's task in recommending priorities for the Comparative Effectiveness funding provided by Congress. You have the opportunity to look broadly across many divergent and competing segments of the U.S. healthcare system and we urge you to select topics that will have broad cross-cutting impacts.

We urge the Council to consider the following issues and priorities for comparative effectiveness research:

- Existing teleradiology and telemedicine resources should be developed and utilized as part of the infrastructure to establish registries, networks and other e-health data to

analyze comparative effectiveness on diagnostic and treatment pathways for targeted health conditions.

- Reimbursement policies and claims processing systems aimed at controlling costs are impeding rather than facilitating the development of teleradiology and other innovative systems that provide cost-effective health IT-based services. Conflicting quality, credentialing and state policies under Medicare and Medicaid also impede access to quality teleradiology. Comparative effectiveness studies involving diagnostic services must be cognizant of the impact of these forces and provide solutions to improve access.
- Coordinating, leveraging, and providing synergies with Recovery Act and ongoing projects in developing and implementing the infrastructure for health IT, digital image transmission, e-health records, telemedicine, and teleradiology.

vRad is extremely well-positioned and very interested in serving as a partner in these important efforts to improve the nation's healthcare system.

Submitted by
Ellen Schwalenstocker,
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National Association of Children's Hospitals and Related Institutions
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The National Association of Children's Hospitals and Related Institutions (NACHRI) is a not-for-profit membership organization of more than 215 children's hospitals. NACHRI promotes the health and well-being of all children and their families through support of children's hospitals and health systems that are committed to excellence in providing health care to children. Children's hospitals work to ensure the health of all children through clinical care, research, training and advocacy.

NACHRI supports increased investment in comparative effectiveness research (CER) to improve the quality of patient care and to support patients, families, physicians and providers in making decisions about the "right care at the right time." We welcome and appreciate the opportunity to provide this statement at the first listening session of the Federal Coordinating Council for Comparative Effectiveness.

Widespread variability in care, such as that demonstrated by the Dartmouth Institute for Health Policy and Clinical Practice in the Medicare population, exists in care for children as well. A chart book on quality of health care for children and adolescents developed for The Commonwealth Fund suggested that "one-quarter to three quarters of children do not receive the health care that is scientifically proven and/or that experts recommend to prevent disease, reduce disease complications, and achieve optimal health and development" and that "up to one in five pediatric patients receives inappropriate care" (Leatherman and McCarthy, 2004).
NACHRI offers the following considerations as the Federal Coordinating Council develops its recommendations.

- Children and adolescents should be specifically included in priorities identified for comparative effectiveness research with careful consideration for separate studies

evaluating only pediatric populations. Because many diseases afflicting adults originate in childhood and, because many of these diseases can be prevented, investing in children's health care effectiveness research is both a moral imperative and cost effective as the "impact of investments in quality will be longer lasting" (AHRQ, accessed April 9, 2009). According to the Agency for Healthcare Research and Quality, improvement in just four quality and patient safety topics could save between 3,700 and 7,400 children's lives in a single year.

- Priorities identified by the National Priorities Partnership (*Aligning our Efforts to Transform America's Healthcare: National Priorities and Goals,* 2008) and the Institute of Medicine (*Priority Areas for National Action: Transforming Health Care Quality,* 2003) should help to guide CER priorities.

- Comparative effectiveness research should address systems issues such as care models that integrate primary and tertiary care, longitudinal management of chronic conditions and transitions from pediatric to adult health care and should not focus only on specific drugs, devices and conditions. The largest immediate impact on quality and cost may not come from trials of specific drugs and devices but from identification of effective models of care delivery and avoidance of medical errors. This is especially true for children. Except for a very few conditions, the numbers of children with a single condition are small, and children and youth with special health care needs (CYSHCN) often have multiple chronic conditions. These children represent approximately 15 percent of children but consume 40 to 60 percent of health care resources devoted to children.

  Similarly, patient safety and prevention of avoidable medical errors, including safety of children's health care, should be an important focus for comparative effectiveness. For example, healthcare-associated infections (HAIs) result in significant disease burden and cost, are of great public interest and are an IOM and National Priorities Partnership priority area. HAIs are among the top 10 causes of death in the U.S. (GAO, 2009). Interventions to prevent HAIs often require adaptation for pediatrics. For example, unlike in adults in whom central line insertion bundles can eliminate Catheter Associated Blood Stream Infections (CABSIs), the insertion bundle alone does not eliminate pediatric CABSIs. A multi-center collaborative on reducing CABSI in Pediatric ICU's found that, in contrast to adult-based efforts, reliable use of nursing-oriented standardized daily line maintenance practices is the most significant factor in reducing CABSI in children. To-date the collaborative effort has reduced CABSI over 50 percent with an estimated 80 lives saved and over $23 million in health care cost savings.
- Sufficient research to establish effective and proven treatments in pediatrics is needed. It cannot be assumed that treatments shown to be effective in adults will translate into effective treatments for children. Accordingly, consideration for how to facilitate inclusion of children in randomized control trials with specific subgroup analyses and other comparative effectiveness research is required.

- Data, as well as demonstration efforts, should not focus only on the Medicare population, but should include Medicaid and CHIP as well. Investment in information to enable identification of variability, similar to that available for the Medicare population, in children's health care is critical.

- Representatives from the pediatric community should be included in any body or committees overseeing and guiding comparative effectiveness research.

Submitted by
Douglas R. Hadley, MD
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Director, Coverage Policy Unit
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Thank you on behalf of CIGNA and the 12 million individuals and the companies that we serve for the opportunity to speak before the Institute of Medicine on establishing national priorities for comparative effectiveness research. It is our belief that in developing national priorities for comparative effectiveness research, there should be three guiding principles.

First, focus research on high impact areas. Research priorities should first focus on high prevalent conditions, which account for significant healthcare spending, and which have competing diagnostic or treatment pathways. Cardiovascular diseases, cancer, obesity, and arthritic conditions all have competing diagnostic and treatment pathways, and they collectively have a major impact on healthcare costs in the US in the adult population.

Second, costs should not be considered as the initial goal of comparative effectiveness research. Costs should only be considered in limited circumstances after the comparative effectiveness research shows that two alternatives are clinically equivalent: For these high impact conditions, there are often competing diagnostic modalities and treatment pathways, the comparative effectiveness has not as yet been established with the available evidence for the typical patient, or selected subpopulations. We should first establish through comparative effectiveness research, if one diagnostic modality or treatment pathway is superior to another, in terms of its safety and clinical effectiveness. This will have the effect of focusing treatment and coverage decisions on the most effective treatments available ***without*** consideration of the cost. However, if comparative effectiveness research shows that the two treatments are essentially equivalent to each other in terms of safety and clinical effectiveness, ***then and only then***, is it appropriate to consider the total medical costs associated for each treatment because patient outcomes would be the same and it would be appropriate to ask which alternative is the better value for our healthcare dollar.

Finally, we should first fund "shovel ready" projects. It is important that national priorities for comparative effectiveness research should focus initially on those studies which can be quickly approved by the NIH, AHRQ or CMS, and which can be up and running in a relatively short period of time.

Thank you for the opportunity to speak before this committee on this important topic.

Submitted by
Tony Curry
Gundersen Lutheran Health System

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To the Federal Coordinating Council for Comparative Effectiveness,

Thank you for your work in guiding the direction of Comparative Effectiveness research as part of the American Recovery and Reinvestment Act. Thank you also for the opportunity to share our comments today.

We believe that comparative effectiveness studies should focus on medical delivery systems and operations, resulting in information that can be leveraged to foster better clinical and cost outcomes.

With a one hundred year history of treating patients, Gundersen Lutheran Health System in La Crosse, Wisconsin is not new to medicine, but studies suggest that if the nation's healthcare organizations and health insurers replicate the way that Gundersen delivers healthcare, we could see a greater than 30% decline in national healthcare costs and improve healthcare value in the United States (2008 Dartmouth Atlas Health Study).

The annual Dartmouth Atlas Study identified La Crosse, Wisconsin as the lowest cost-of-care city in the nation for Medicare patients during the last two years of life. Health care at Gundersen Lutheran in La Crosse costs 72% less than the leading medical center in New York, 71% less than in Florida, 68% less than in California, and even 42% less than the leading medical center in Minnesota.

Why is the cost of care at Gundersen so much lower while the quality of care remains as good or better? Gundersen's cost and quality success is driven by their integrated delivery system. Every Gundersen program, from advance care planning and care coordination to heart care and breast cancer care stems from their integrated delivery approach. And each program that achieves top-in-the-nation status also has a correlating reduction – significant reduction – in healthcare costs.

As one example, Gundersen Lutheran's breast cancer program has become the first and only in the nation to achieve the highest level of distinction from the National Quality Measures for Breast Centers™ (NQMBC) Program. Gundersen Lutheran is also one of only two organizations in the country with every available accreditation for the full scope of breast care, diagnosis and treatment from the American College of Radiology. Gundersen's interdisciplinary model of caring for breast cancer patients has the potential to save an estimated $4.15 billion dollars in healthcare costs if it is implemented on a national scale.

As a lean and medium-sized medical center, however, putting resources into researching their programs' results for wider dissemination or use as a national best practice are resources Gundersen Lutheran just hasn't had in the past. This comparative effectiveness funding represents a mechanism by which organizations like Gundersen Lutheran can share their results and best practices.

So, in addition to investing comparative effectiveness dollars into treatment approaches for specific conditions, comparative effectiveness funds should invest in comparing the most

effective health system delivery approaches in the United States. Only then, can comprehensive, sustainable healthcare delivery and payor system reform take root.

Gundersen Lutheran is a living lab for applied comparative effectiveness research. Understanding how and why Gundersen's approaches work will help lower U.S. healthcare costs, improve quality and create a more sustainable payment system that incentives the outcomes it seeks to achieve. Gundersen Lutheran is willing to share further information and our program data to help move this work forward.

Thank you again for the opportunity to share our recommendations for comparative effectiveness research as part of the American Recovery and Reinvestment Act. We hope for the opportunity to contribute to this movement toward healthcare reform.

Headquartered in La Crosse, Wisconsin, Gundersen Lutheran Health System is a not-for-profit organization that provides quality health services to patients at its hospital and clinics throughout western Wisconsin, southeastern Minnesota and northeastern Iowa. Gundersen Lutheran is comprised of nearly 700 medical, dental and associate staff, and supported by a staff of more than 6,300. Gundersen has been consistently ranked in the top 5% of hospitals in the country.

For additional information, contact Gundersen Lutheran's Joan Curran, Chief of Government Relations and External Affairs, at (608) 775-1400, located at 1900 South Avenue, La Crosse, WI 54601. You may also contact our Washington-based associate Ladd Wiley, Akin Gump, at (202) 887-4083.

Submitted by
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My name is Meridith Mitchell and I am presenting on behalf of M2S, Inc. I thank you for the opportunity to comment today and appreciate the willingness of this committee to seek public input with regard to Comparative Effectiveness Research (CER.) The focus of this statement is on the utilization of a Clinical Technology Organization (CTO) to effectively facilitate CER.

M2S has significant and specific expertise in the development of patient registries, endovascular 3-D modeling, and medical imaging core lab service. M2S is a CTO with experience, flexibility and efficiency, delivering the highest quality benchmarked reporting at the lowest total delivery cost.

As funds are allocated for CER and Health Services Research (HSR) it will be critical to utilize a CTO. A CTO is a clinical research company that focuses on clinical informatics and optimizes

the use of technology to increase efficiency, reduce cost and maintain or enhance clinical quality. Equally important is to rely on a CTO that is knowledgeable in CER initiatives and capable of delivery today. A CTO is powerful when actively used in the clinical pathway as well as for research initiatives such as CER.

M2S recognized the need for CER as it relates to endovascular aneurysm repair beginning in 2001. M2S' Patient Evaluation and Management System (PEMS®) is the largest radiological/clinical footprint in the world relied upon by highly respected physician investigators. It is a database of 150,000 patient entries providing CER of FDA approved Aortic Stent Grafts. The service has been broadly adopted by health care advocates in both practice and industry having recognized the impact that this vast and valuable repository of data has on quality improvement.

In the past Comparative Effectiveness studies have evaluated a particular therapy only against itself. M2S evaluates the full scope of information including the patient health record, radiologic information with patient input. This model is unique, highly effective and patient centric and has direct impact on the quality of patient care.

M2S believes that simplifying the work flow facilitates summarization of comparative data and enhances a clinician's ability to make informed treatment decisions. An effective CTO collects data simultaneously as fields are populated by the institution or Electronic Medical Record (EMR) system. The flow of data provided for comparative analysis is aggregated, risk-adjusted, evaluated and reported on by an independent panel of physician thought leaders.

We all agree that the goal of CER is to enhance patient care; a web-based integrated clinical technology platform is a key component in the success of these projects. Health care organizations are striving to make better use of clinical informatics as an adjunct to Comparative Effectiveness. Involving a CTO early in the planning phase will streamline the process, reduce costs, and facilitate better data aggregation with one accessible portal for all relevant information.

Submitted by
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As an organization focused on information and education for physicians and other healthcare professionals, Medscape, LLC endorses the increased funding for comparative effectiveness research ("CER").

Results of the CER are highly relevant to all stakeholders, consumers, patients, physicians, nurses and healthcare professionals. Furthermore, it is extremely important that the latest results on effectiveness, safety and quality measures be incorporated into clinical practice as rapidly as possible. A multidisciplinary, multi-modal approach is essential to accomplish this result.

Therefore, it is important that communications and education for both healthcare professionals and consumers/patients be incorporated into the overall strategy for CER.

In measuring significance of comparative effectiveness research on clinical practice, one must not overlook the importance of disseminating the results to as wide-spread a professional audience as possible.
It is particularly critical to include educational components and educational outcomes studies in order to determine the effect of this research on changes in clinician behavior and patient health.

An AHRQ Evidence Report on Continuing Medical Education (CME) published in 2007 concluded that, despite limited evidence, CME appears to be effective at the acquisition and retention of knowledge, attitudes, skills, behaviors and clinical outcomes. In addition, the study suggested the following:[1]

- Live media was more effective than print. In fact, print interventions were either not beneficial or very weak in their ability to improve attitudes.
- Multimedia was more effective than single media interventions.
- Multiple exposures were more effective than a single exposure.

Furthermore, evidence is increasingly demonstrating that on-line CME may have particularly large positive effects on outcomes.[2] According to a 2008 meta-analysis published in JAMA, 16 of the 17 studies analyzed revealed improved knowledge, behavioral outcomes, or both for participants using Web-based interventions.[3] In a recent randomized controlled trial of 113 primary care physicians, those who viewed an on-line diabetes seminar were 63% more likely to order an eye exam for diabetes patients than physicians in the control group (27%).[4]

MedscapeCME is currently a major provider of online CME/CE, reaching over 1.5 million physicians and nurses each month. In an analysis of outcomes studies on 97 Medscape on-line CME activities taken by 13,520 physicians, participants were 52% more likely than non-participants to make diagnostic and therapeutic choices based on clinical evidence. [5]

Specifically, over the past two years, Medscape has published CME activities based on several AHRQ Effectiveness Report Executive Summaries:

- Comparative Effectiveness of Treatments To Prevent Fractures in Men and Women With Low Bone Density or Osteoporosis: http://www.medscape.com/viewprogram/17304
- Comparative Effectiveness of Therapies for Clinically Localized Prostate Cancer: http://www.medscape.com/viewprogram/17364
- Comparative Effectiveness of Angiotensin-Converting Enzyme Inhibitors (ACEIs) and Angiotensin II Receptor Antagonists (ARBs) for Treating Essential Hypertension http://www.medscape.com/viewprogram/8669
- Comparative Effectiveness of Off-Label Use of Atypical Antipsychotics http://www.medscape.com/viewprogram/7361
- Comparative Effectiveness of Second-Generation Antidepressants in the Pharmacologic Treatment of Adult Depression: http://www.medscape.com/viewprogram/7793

These activities have been viewed by 5,000 to 18,000 nurses, physicians, and medical students and the CME participation rates for each AHRQ activities have ranged from 3,300 to over

8,500. In 2007, Medscape commissioned an educational outcomes study with a third party research group based on the CME activity for *Comparative Effectiveness of Second-Generation Antidepressants in the Pharmacologic Treatment of Adult Depression.* Over 3,000 physicians participated in this on-line activity and the outcomes study results suggested that these participants are 68% more likely than non-participants to make evidence-based choices in the diagnosis and treatment of adult depression. It should be noted that these 3000 physicians are estimated to see at least 72,000 patients each week who have adult depression, suggesting the significant positive impact of online CME based on these Effectiveness Reports.

In conclusion, funding for a massive educational program is imperative for changing clinician behavior and effecting improved patient health and cost effective care. Both the evidence and Medscape's experience emphasize the benefits of a comprehensive online educational program. It is imperative not to neglect this component of the CER funding available in the Recovery Act. Finally, it should be strongly noted that educating the patient on the results of this research is critical to the effectiveness of these programs in improving health and reducing medical costs.

[1] Marinopoulos SS, Dorman T, Ratanawongsa N, Wilson LM, Ashar BH, Magaziner JL, Miller RG, Thomas PA, Prokopowicz GP, Qayyum R, Bass EB. Effectiveness of Continuing Medical Education. Evidence Report/Technology Assessment No. 149 (Prepared by the Johns Hopkins Evidence-based Practice Center, under Contract No. 290-02-0018.) AHRQ Publication No. 07-E006. Rockville, MD:Agency for Healthcare Research and Quality January 2007.

[2] Casebeer L, Engler S, Bennett N, Irvine M, Sulkes D, DesLauriers M, Zhang S.A controlled trial of the effectiveness of internet continuing medical education. BMC Med. 2008 Dec 4;6:37.

[3] Cook DA, Levinson AJ, Garside S, Dupras DM, Erwin PJ, Montori VM. Internet-based learning in the health professions: a meta-analysis. JAMA. 2008 Sep 10;300(10):1181-96. Links

[4] Weston CM, Sciamanna CN, Nash DB. Evaluating online continuing medical education seminars: evidence for improving clinical practices. Am J Med Qual. 2008 Nov-Dec;23(6):475-83.

[5] Poster Presentation at CME Congress 2008 in Vancouver, BC; C. Grimes, M. Irvine, DJ Sllkes, L Casebeer, M Abdolrasulnia, M DesLauriers, Medscape LLC, New York, NY, USA, Outcomes, Inc. Birmingham, AL, USA

Submitted by
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National Patient Advocate Foundation (NPAF) is a non-profit organization dedicated to improving access to healthcare services through policy reform. Our mission of creating avenues of patient access through improved access to, and reimbursement for,

evolving therapies, therapeutic agents, and devices is influenced by the experience of patients who receive case management services from our companion organization, Patient Advocate Foundation (PAF). Last year, PAF received more than 9.5 million inquiries from patients throughout the United States seeking information and assistance for access to care issues resulting from diagnoses of a chronic, debilitating or life-threatening disease. Of those, 48,369 became full patient cases involving communications made by PAF staff on behalf of a patient in order to reach positive resolution.

The *"American Recovery and Reinvestment Act of 2009"* includes $1.1 billion in new funding for comparative effectiveness research. NPAF supports comparative effectiveness research and believes it should be used as a tool between providers and their patients to determine the best course of action in treatment. However, NPAF is concerned that the Federal Coordinating Council established in the stimulus bill does not include roles for patient representatives.

NPAF strongly believes that the Federal Coordinating Council should consist of all relevant stakeholders, including patient and consumer groups, government, providers, insurers and manufacturers of drugs and medical devices. These stakeholders should be involved in every step of the process, from setting the research agenda, and developing study methodology, to the translation and dissemination of findings.

While we appreciate the invitation to participate in today's public listening session, other health agencies, such as FDA and CMS routinely appoint patient representatives to their scientific research panels, and we encourage the council to seek to correct this situation.

In addition, NPAF is concerned that comparative effectiveness research may ultimately be used to make cost-effectiveness decisions. NPAF would like to emphasize our support for comparative effectiveness research for the purpose of improving the quality, safety and delivery of care; however, NPAF does not support using this research to limit access, deny treatment or reimbursement.

Many European countries have already developed a system for comparative effectiveness research but many of these countries impose cost-effectiveness analysis in ways which ultimately deny patients access to more expensive drugs. In the United Kingdom, the National Institute for Health and Clinical Excellence (NICE) conducts research and develops guidelines for the country's National Health System (NHS). In the last several years, NICE has instituted certain coverage decisions based on cost-effectiveness that severely impact patient access to appropriate care. As recent as January 2009, Ministers in the Welsh Assembly overturned a NICE decision that prohibited kidney cancer patients from accessing drugs such as Sutent, Avastin, Nexavar and Torisel, all of which have been proven to treat kidney cancer effectively. In the United States, it is common practice for these drugs to be prescribed to a kidney cancer patient. Advancing comparative effectiveness research in the U.S. can be a

positive tool for patients and providers, only when it focuses on clinical comparative effectiveness.

NPAF also believes that a national comparative effectiveness program should prioritize the linking of data from federal and private entities to build upon existing data collection efforts and research capabilities. Insufficient funding for any public or private entity responsible for aligning and maintaining a robust data network has resulted in isolated clinical outcomes research efforts. Expansion of CER activities should include public-private coordination of data collection and interoperability of both clinical research networks and healthcare databases in order to increase the available data.

CER should support for the development of "personalized" or stratified medicine by further examining individual factors that contribute to disease susceptibilities and differences in clinical outcomes.

NPAF urges you to consider these issues as the Council makes its recommendations to the Secretary on comparative effectiveness research priorities so that comparative effectiveness research will have beneficial long-term consequences for patient care and access.

Submitted by
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The Society of Thoracic Surgeons (STS) appreciates the efforts of the Federal Coordinating Council for Comparative Effectiveness Research in soliciting input for consideration of priorities for comparative effectiveness research studies as called for in the American Recovery and Reinvestment Act of 2009 (ARRA). We welcome the opportunity to submit the following testimony as you consider recommendations on research priorities.

STS is a not-for-profit organization representing cardiothoracic surgeons, researchers, and allied health professionals worldwide who are dedicated to ensuring the best possible surgical care for patients with diseases of the heart, lung, esophagus, and other organs in the chest. Founded in 1964, the mission of STS is to enhance the ability of cardiothoracic surgeons to provide the highest quality patient care through education, research and advocacy. STS supports data-driven

approaches to quality measurement, quality improvement, and quality reporting because we recognize that the collection of clinical data and the feedback of those data to physicians improve patient outcomes.

The STS National Database is the premier voluntary clinical data registry for cardiothoracic surgery; it includes three component parts: the Adult Cardiac Surgery Database, the General Thoracic Surgery Database, and the Congenital Heart Surgery Database. Surgeons add new patient data to the database semiannually or quarterly each year, thereby providing a highly dynamic, up-to-date picture of cardiothoracic surgical practice.

Our comments focus on the Adult Cardiac Surgery Database that contains detailed clinical information relevant to the processes and outcomes of care in adult cardiothoracic surgical procedures (STS ACD). This information, collected over a 20-year period, has been used to improve the quality of care for patients undergoing cardiothoracic surgical procedures. The STS ACD captures data on all adult patients undergoing cardiac surgical procedures performed by participants throughout the United States and, with more than 960 participants representing more than 2,800 individual surgeons, STS estimates that it captures approximately 90 percent of all adult cardiac surgery performed nationwide. Currently, the STS ACD contains more than 3.6 million surgical records and is the largest clinical cardiac surgery database in the world.

Clinical registries developed by STS and the American College of Cardiology (ACC) have been at the forefront of quality improvement activities in cardiovascular medicine for a number of years. Combined, STS and ACC databases cover virtually the entire spectrum of cardiovascular care. The STS ACD and the ACC's National Cardiovascular Data Registry (ACC NCDR) have provided clinicians with important feedback on their practice patterns and performance, and are invaluable tools for use in real world medical practice and health services research. The ACC NCDR is the ACC's group of clinical databases that measure and quantify outcomes in the delivery of quality cardiovascular patient care. It is designed to improve the quality of cardiovascular patient care and to identify gaps in care by providing information, knowledge and tools, implementing quality initiatives, and supporting research that improves patient care and outcomes.

The information in these registries supports performance assessment, and comparative effectiveness studies, as well as the integration of new treatments into routine clinical practice. These registries contain detailed information, including demographics, cardiovascular history, patient risk factors and co-morbid illnesses present on admission, interventions, care processes, and risk-adjusted outcomes surrounding specific clinical events. The STS ACD and ACC NCDR represent the "gold standard" of clinical databases in the country and can serve as the prototypes for registries across medicine.

Specific provisions in the ARRA call for funding to accelerate the development of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data. STS believes that by linking clinical data from the STS ACD and the ACC NCDR with Centers for Medicare and Medicaid Services (CMS) administrative data, a powerful, longitudinal data set

could be produced. Such a data set would contain process, outcomes, utilization, pharmacy, and cost data spanning at least a three to five year period. This linking project has the potential to cover virtually the entire spectrum of care for cardiovascular disease. Ultimately, the information obtained from this collaborative effort will produce new insight into the comparative effectiveness of coronary revascularization treatment strategies, such as coronary bypass grafting and stenting procedures. Because the STS and ACC databases are mature, there exists the real potential to achieve extraordinarily meaningful results in a timely manner, certainly within the time frame set forth to fund comparative effectiveness research projects under the ARRA.

A similar collaboration between STS and ACC is also under way in congenital heart surgery. ACC is collaborating with STS in the development of its pediatric database, harmonizing data definitions with STS's Congenital Heart Surgery Database. Down the road, this collaboration will facilitate following patients through episodes of care as well as longitudinal follow-up and comparison of outcomes.

The approach used in this project can be adopted by other specialties and their professional societies for comparative effectiveness studies and quality improvement. The techniques of analysis will have broad application to the entire field of medicine.

The results of these comparative effectiveness studies will almost certainly improve the care of cardiovascular patients while shedding new light on ways to provide care more efficiently. The above mentioned linked data set would also be a valuable source of data to examine appropriate use criteria, efficiency of care, cost of care, and value-based health care. Accordingly, we urge the Council to strongly support the development of this collaborative STS-ACC project that would reflect and effectuate the intent of the ARRA to utilize clinical registries in the analysis of comparative effectiveness research studies.

Additionally, we ask the Council to strongly consider the following comparative effectiveness research studies in cardiothoracic surgery:

1. STS-CMS longitudinal follow-up to assess the long-term impact of compliance with NQF-endorsed performance measures;
2. STS-CMS assessment of comparative effectiveness of valve replacement and repair procedures;
3. STS-CMS longitudinal follow-up of patients undergoing surgery for various forms of lung cancer treatment; and
4. Using IT links between the STS National Database, the ACC NCDR, and the CMS MEDPAR dataset, examine the comparative effectiveness of percutaneous *versus* surgical treatment of atrial fibrillation.
5. Assessment of the comparative effectiveness of new technologies and treatments as they are introduced;
6. IT Projects Facilitating Healthcare Data for Research;

We applaud the Council for convening this meeting to obtain input from various stakeholders and inform its development of priorities for comparative effectiveness research. Thank you again for this opportunity to provide testimony.

Submitted by
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Standard Biologics, Inc., is a U.S. Registered and Resident Corporation. Mr. Joseph A Cerceo, is the President and Founder of Standard Biologics, Inc. ('S.B.Inc.').

Mr. Cerceo has many very successful years of experience in the field of **Scientific Applications Research** including work on behalf of the **US Government**.

After studies that involved Chemistry for the Health Sciences; The Vitamins; Advanced Nutrition, Macronutrients and Micronutrients; Nutrition and Exercise Immunology; Nutrition and Gene Expression; Nutrition applied to Sports Medicine and Injury Rehabilitation; Athletic Injuries and Rehabilitation; Cell Biology of Trauma; Endocrine Physiology; Bone, Fracture Repair and Regeneration and the Bioavailability of Nutrients Mr. Cerceo discovered the extensive natural medicinal abilities of a **very high quality Proteolytic enzyme** (i.e. a Protease, termed by S.B.Inc. also as its 'Protocol').

Several years of research were completed culminating in the successful Application of a US Patent that covers inter alia the invention of the techniques of successful assessments for treatment of patients and methods of the Administration of the Protease to mammals (and, by extension, to a broad diversity of other animals) with the initial very successful treatments being of **inflammation** and **trauma** in their *many manifestations*.
The Proteolytic enzyme is attracted to and acts rapidly at sites of inflammation in the host.

So effective are such capabilities of the Protease that lives that could well have been lost due to uncontrollable inflammation occurring post surgery and otherwise, were saved by the administration of the Protease following Mr. Cerceo's invented Administration techniques. S.B.Inc. has documents related to this point in formats applicable to technical explanation as well as several 'Proof of Concept' statements.

Further research and development and a substantial number of 'Proof of Concept' trials firmly established further extremely important capabilities of the Protease that can be described in relation to the dictum, "**Inflammation is everything** !!"

Additional important aptitudes relating to the Protease's beneficial powers are in respect of:

The treatment of **Cancer tumours** including those in inoperable or life-threatening bodily positions thereby helping to reduce dangerous invasive surgery.
The Protease suppresses angiogenesis (the development of the vasculature of the tumours), that 'feeds' the tumour, thereby starving the tumour. A precautionary course of anti-biotics can be applied to treat potential secondary infection from the resulting necrotic tumour tissue.
A consequent further advantage is that there will be a range of cancer cases **where treatment of the patient by radiotherapy and chemotherapy will be unnecessary** with all the benefits to both the patient and to the finite treatment resources available that that advantage will bestow – the avoidance of those treatments will not be the case for all cancer patients.
The neutralization of VEGF, vital to the progress / metastasis of such as bone cancer. **VEGF** is promoted by inflammatory proteins which are hydrolyzed by the Protease.

Cell-signalling, i.e. the utilisation by the Proteolytic enzyme of this phenomenon to enhance its ability to identify and attack non-self proteins - including the hydrolyzation of a **variety of viruses and pathogens** (not just individual viruses or pathogens) such as various strains of Influenza, Common Cold, HCV / HIV (these are currently under long-term trial), and such other serious viruses as Avian Influenza, Ebola hemorrhagic fever, Rift Valley and Yellow Fevers and so on (a capability enabled by a characteristic of the Protease of having a **broad spectrum peptide specificity**).
Other non-self protein-bearing pathogens may be hydrolyzed such as Malarial protozoan parasites and Babesiosis and other zoonotic parasites similarly with non-self proteins (as detailed by Professor Brian Greenwood of the London School of Hygiene and Tropical Medicine).

Pain Medication is brought about in varying meaningful degrees by the beneficial impact of both S.B.Inc. products on the body's pain response mechanisms.

Importantly, this 'Protocol' has the Approval of **EPA** and **FDA** and also it is approved by FDA as a Food Grade Additive (it is considered as safe as any other food ingredient monitored by the US Government).

NB: An important attribute of this Company's products is that when they are administered to a patient in accordance with our straightforward and not unpleasant procedure(s) ***NO adverse side-effects are experienced in virtually ALL patients***.
An American adage with particular relevance to our Adjuvant *Anti-Inflammatory Protocol*, our enzyme, is that it is analogous to 'Chicken Soup', i.e. the enzyme can't hurt; it will only do good. The human body produces, normally, a very small amount of such an enzyme and the corollary is that **the mammalian system actually welcomes the administration of our Protease**.

Product costs: Standard Biologics, Inc. confidently expects that both its medical treatment developments – the Proteolytic enzyme and the complementary Therapeutic Nutritional Supplements will prove to be substantially less costly than pharmaceuticals measured as at both point of use and also when costed taking into consideration that they do not cause adverse side-effects that can be expensive and resource-consuming to treat.

Standard Biologics, Inc., ('S.B.Inc.') has either sent or will send shortly Corporate and Product Information to:

BARDA (Biomedical Advanced Research and Development Authority) @ H&HS;

The DTRA at the DOD;

CDC - The Influenza Division;

CDC - The NCHHSTP Division;

The NIH – the Office of Dr. Anthony Fauci, M.D., Director of NIAID;

U.S. Homeland Security Department;

The Department of State – the Office of the Global Aids Coordinator;

United States Army Institute of Surgical Research, Fort Sam Houston, Texas 78234-6315;

USDA - ADOL in East Lansing, Michigan;

USDA – SEPRL in Athens, Georgia;

And other U.S. Government areas.

Submitted by
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I am Sam Lin, a Family Physician, representing the *American Medical Group Association* based in Alexandria, Virginia. *AMGA* is a professional medical association representing some of this nation's largest, best known, and prestigious integrated health care delivery systems. More than 95,000 physicians practice in *AMGA* member organizations and provide health care services for approximately 95 million patients.

Today, in the context of the Comparative Effectiveness Research to be funded by the Recovery Act, you are hearing comments with a focus on disease conditions and treatments. And while those viewpoints are critical to the discussion, it is imperative that we also examine the context in which healthcare is given. Unless the delivery system is considered, we otherwise would continue to promote fragmented care, leaving our patients to wander on their own. *AMGA* supports the fundamental concepts of comparative effectiveness information use in health care delivery, but we also believe that delivery systems matter. There is an emerging body of evidence that supports this idea, and this premise is also being tested in several CMS demonstration projects. We, therefore, strongly recommend that the Coordinating Council consider comparative effectiveness in its broadest terms and not be limited only to research on treatments and devices alone.

CER should be undertaken for quality, efficiency, effectiveness, and other appropriate dimensions for health care delivery systems along the entire spectrum of systems integration. This spectrum should include integrated delivery systems, multi-specialty group practices, single-specialty groups, "virtual" groups such as IPAs, PHOs, and small medical practices (solo, duo, small groups), and perhaps others.

Use of comparative effectiveness information on delivery systems would facilitate and strengthen provision of patient care, disease states, and related financial determinations. By knowing what works best in treating patients, this nation's healthcare delivery system could make substantial strides toward improving clinical outcomes and closing gaps in geographic and population variations, while reducing health care expenditures. While we favor CER, we temper that view with a caution that such information must be objective, developed by disinterested parties, and should be equally applied.

Submitted by
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University Hospitals (UH) is a world-renowned academic health care facility located in Cleveland, Ohio with research specialties that span 21 academic and clinical departments. UH is also the primary affiliate of Case Western Reserve University School of Medicine (CWRU). Together, UH and the CWRU form the largest biomedical research center in the state of Ohio. In order for UHC to continue to provide effective, quality health care for its patients, the need for comparative effectiveness research (CER) must be met. The mission of UH is To Heal. To Teach To Discover. This drives the ability to responsibly grow research and scientific innovation to improve patient care. In order to achieve the organization's overall mission, the research arm must provide sufficient infrastructure to drive the To Heal part of the mission. CER is a necessary component of that research infrastructure and without out CER the link back to patient care would not be as direct. CER at UH is primarily driven in two ways: 1) interdisciplinary outcome databases to record patient treatment information from admission to discharge are created and maintained throughout the organization; and 2) the large clinical populations in the areas of Surgery, Oncology, Pediatrics, Cardiovascular Medicine and Neurology drive the innate ability to conduct large scale CER.

1) Department of Surgery: Serving as the tertiary referral hub for UH, the Department of Surgery is comprised of the Divisions of General Surgery, Vascular Surgery, Cardiac and Thoracic Surgery, Plastic Surgery and Pediatric Surgery. Surgeons have faculty appointments at Case Western Reserve University School of Medicine and are either Board Certified or Board eligible in their respective surgical disciplines. Across the United States, most hospitals use several databases to record patient treatment information from admission to discharge. To date, such databases are typically unable to communicate with each other, thereby making it time consuming (if not impossible) to efficiently and accurately track patient treatment and outcomes information. Therefore, physicians (and hospitals) are unable to routinely monitor and compare the effectiveness and true costs of different treatments/procedures as well as physician performance. An example of this needed database is currently being developed on a small-scale pilot basis "in-house" by UH physicians in the Department of Surgery to readily view patient outcomes by surgical sub-specialty. This is helping to overcome the difficulties in having so many disparate clinical information systems and makes the data more valuable than before. This program interfaces with each hospital database to extract the information necessary to recreate a patient's hospital stay from admission to discharge, as well as the occurrence of any readmissions and outpatient visits and procedures. It provides physicians and hospitals with treatment and outcome information that was previously unavailable such as: true patient treatment history, including physician's costs and data, combined with hospital costs and data; comparison of recovery time and costs for competitive treatments (e.g., laparoscopic versus open);

physician performance (operating room time, recovery time, length of stay, etc.); readmissions based on type of surgery, physician, etc.; and research or educational purposes (for example, outcomes relating to a particular surgery with patients having particular complications or diagnoses). However, this type of database is needed not just for one clinical and academic department, but for all UH and CWRU departments; and not just for information within UH, but also for information between UH and other institutions. Currently, the cost of creating and sustaining such a system is prohibitive. For the above mentioned "in-house" database for the Department of Surgery is estimated that it will take ten full time employees and $1.1 million to create, run, and sustain this integrated database system. Federal support is necessary if this type of technology is to be fully developed and maintained.

2) Cancer Care: The Ireland Cancer Center at UH is one of only 39 Comprehensive Cancer Centers in the country designated by the National Cancer Institute. (NCI), allowing Ireland to offer cutting-edge treatments earlier than most other cancer centers. Our affiliation with CWRU provides ongoing studies aimed at cancer treatment and prevention. Cancer therapies change on a case by case basis and most institutions are turning to a multidisciplinary approach, employing experts in surgery, medical oncology, radiation therapy, pathology, nursing, social work and psychology working together to create a personalized treatment plan that meets the physical, emotional and spiritual needs of patients. Moreover, new treatments and investigational procedures are tested every day in cancer patients. At UH alone there are more than 300 cancer clinical trials, many of them featuring new drugs developed by our own clinician-scientists. With such a large group of medical care providers and variety of cancer treatments, Federal funding for CER is needed not just to continue expanding available cancer treatments, but also to standardize and prioritize these cancer therapies based on patient outcomes to ensure proper allocation of resources.

3) Children's Health Care: Rainbow Babies and Children's (RBC) Hospital at UH is one of the top Children's Hospitals in the world. RBC is a world leader in the treatment of children with heart disease , cystic fibrosis, sickle cell disease and endocrine and metabolic disorders . As part of the Ireland Cancer Center, RBC's comprehensive pediatric cancer center offers the most promising treatments to children. RBC is ranked #2 in neonatal care/NICU and among the top 10 best hospitals in the country for neurology / neurosurgery and respiratory disorders[7]. Children receiving medical care are at risk because the devices and treatments given to them are often not adapted or tested for pediatric care, but rather for adult care. This results in a focus on pediatric disease treatment instead of pediatric disease prevention even though research is beginning to show that early intervention is not only clinically effective, but also cost effective. While some Federal support has already been given to stimulate pediatric medical device development, there is currently no consensus on the priorities for products needed by pediatric healthcare providers based on verifiable reference data, and there is no roadmap for companies, investors, and grantors seeking to focus on developing the most needed and most feasible products. Federal support is needed to (1) create priorities and focus

[7] US News and World Report 2008

CER in pediatrics, (2) develop treatments and devices specific to children and specific to preventative care, and (3) generate quality system processes to deliver pediatric care.

4) UH has assembled an integrated team of some of the country's foremost experts in cardiology, interventional cardiology, heart failure and transplant, electrophysiology, cardiac and vascular surgery. This highly specialized team of experts forms the foundation of University Hospitals Harrington-McLaughlin Heart & Vascular Institute, a premier center for comprehensive care of patients with diseases affecting the heart and vascular system. Our goal is to create a national center of excellence in cardiovascular research and physician education, as well as patient care. A terrific example of CER under way in the Heart & Vascular Institute has been a large program for calcium screening through computed tomography with the aim of early detection and treatment of cardiovascular disease. In the past year alone over 3,000 people have been screened with this method and today there are significant efforts at putting these data into research databases for CER research. The CER protocols have been developed and approved by the IRB. Federal funding in this area could greatly help expand the significant screening that is already underway and could help to establish a valuable CER database for future development of clinical care screenings.

5) Neurologic Care: The Neurological Institute of UH is the first designated institute in Northeast Ohio dedicated to improving outcomes in patients with diseases affecting the nervous system. The institute includes 14 Centers of Expertise that provide patients access to services at locations across Northeast Ohio. UH offers the latest in innovative technology for the diagnosis and treatment of all neurological conditions and is committed to expanding and integrating translational research into clinical practice. The collaboration with CWRU allows basic science research and clinical trials to be quickly translated to offer patients direct and rapid access to leading-edge treatment alternatives. This is all accomplished through the use of CER methodologies that are in need of additional infrastructure in order to be expanded and maintained on a large scale over time.

UH has been, and needs to continue to be, pre-eminently positioned to design, conduct and report on CER for the benefit of the patients served not only in Northeast Ohio, but regionally, nationally and internationally. The significant resources and efforts described herein provide a snapshot of the already significant programs in this area and outline the basic needs necessary to continue to pursue these lines of scientific and clinical inquiry. Academic Medical Centers like UH and CWRU together have the obligation of providing CER data to physicians and other health care professionals in order to arm the next generation of providers with the information necessary to prevent, treat and cure diseases in the future.

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Good afternoon. My name is Cynthia Reilly and I am the Director of the Practice Development Division at the American Society of Health-System Pharmacists. ASHP represents 35,000 pharmacists, pharmacy students, and pharmacy technicians who practice in hospitals and health systems. Pharmacists' expertise in medication use ensures that drug therapies are used safely, effectively, and in a cost-conscious manner. I appreciate the opportunity to present the Society's perspective on comparative effectiveness research, or CER, to the distinguished members of the Federal Coordinating Council for Comparative Effectiveness.

ASHP is a member of the Alliance for Better Health Care, which advocated for inclusion of comparative effectiveness research funding within the American Recovery and Reinvestment Act of 2009. The Society is also publisher of *AHFS DI*, a comprehensive, independent reference on the clinical use of medications, which is recognized through federal legislation under Medicare Part B, Medicaid, and Medicare Part D as an official compendium. For over 50 years, *AHFS DI* has followed sound and high-quality editorial processes to synthesize complex evidence for dissemination and use by a broad range of stakeholders, including prescribers, pharmacists, individuals who make health-policy and coverage decision, patients, and others. ASHP believes there is significant need to compare the effectiveness and safety of specific drug therapies within pharmacologic classes, drug therapies within different classes, and drug therapies with other treatment modalities. *AHFS DI* can serve as a foundation for medication information to support CER and ASHP looks forward to participating in this research.

Today, ASHP requests that the Council consider three CER recommendations related to health care delivery systems that represent critical information needs to improve patient outcomes:

- Optimal practice models for delivery of patient care,
- Strategies for using IT-enabled decision support for delivery of CER, and
- Best practices for disseminating and implementing CER.

As described by the Dartmouth Atlas, the quality and cost of care is inconsistent across geographic regions, with much of this inconsistency attributed to variation in the care setting and the health care professional that provides the service. Under Medicare Part D, pharmacists provide medication therapy management services that include formulating medication treatment plans; monitoring and evaluating patients' response to therapy; performing medication reviews to identify, resolve, and prevent medication-related problems; and coordinating and integrating MTM services within the broader health care services provided to patients. Pharmacists also participate in chronic disease management and prevention activities under collaborative practice agreements with physicians. MTM programs and published research have demonstrated that

pharmacist management of disease and drug therapy significantly improves patient outcomes, while reducing overall health care costs. However, there is limited research that directly compares this practice model to models in which care is provided by other health care professionals or interdisciplinary teams. ASHP believes such research would demonstrate best practices and strongly recommends models of care as a research priority for CER.

A critical element of CER is ensuring that research findings reach the point of care where clinicians, together with patients, can use the information to make informed treatment decisions. Electronic health records and other technologies are expected to provide point-of-care information; however use of these technologies is currently limited, as described in a recent *New England Journal of Medicine* study that found less than 2% of hospitals have fully implemented an electronic health record with clinical documentation, test and imaging results, CPOE, and decision support. Decision support technology has great potential to deliver CER findings, but strategies for creating and integrating these programs within other technologies, as well as barriers to implementation, are not well-studied. ASHP encourages the Council to support research that compares approaches for using clinical decision support and other technologies in the translation and implementation of CER.

In addition to technology, other effective dissemination and translation techniques are needed to ensure that CER findings are used to make informed decisions that improve patient outcomes. However, there are significant challenges in these activities. It has been estimated that there is a 17-year lag time between evidence generation and its widespread implementation. Many strategies have been used to enhance the rate and extent of adoption of evidence-based best practices, including clinical guidelines, continuing education for health care professionals, patient education tools, and most recently, academic detailing. However, these approaches are not well studied and results are variable. ASHP would encourage the Council to support research that compares the benefits and limitations of each approach in order to determine the strategy, or combination of strategies, that facilitates use of CER by each audience, including clinicians, patients, and payers.

Finally, based on ASHP experience as a drug information publisher, we encourage the Council to consider that, similar to drug information, CER research and dissemination efforts are not single events, but rather an ongoing process that requires sustainable and ongoing effort to ensure the currency and usefulness of the information as evidence evolves.

ASHP appreciates this opportunity to provide recommendations for CER. Along with our members, we look forward to collaborating with the Council and others to ensure that CER is not only useful, but also disseminated to clinicians, payers, and patients and subsequently translated into practice.

Submitted by
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eHealth Initiative

The eHealth Initiative thanks the Federal Coordinating Council for Comparative Effectiveness Research for this opportunity to comment on the use of real world electronic health care information for comparative effectiveness research. The eHealth Initiative (eHI) is an independent, non-profit multi-stakeholder organization whose mission is to improve the quality, safety and efficiency of health care through information and information technology. eHI engages multiple stakeholders across every sector of health care to reach agreement on and drive the adoption of common principles, policies and best practices for mobilizing information electronically to improve health and health care in a way that is responsible, sustainable, responsive to each stakeholder's needs—particularly patients, and which builds and maintains the public's trust. The eHealth Initiative is involved in work related to comparative effectiveness in several ways: through pilot projects, educational efforts, and a multi-stakeholder working group.

Electronic data sources that capture the experience of millions of patients have developed over the past few decades and will expand further. Where such records are searchable, it is possible to efficiently assess such information using statistical queries and methods. Such methods may be able to sort through this large volume of data to provide important information on the relationship of medical interventions, types of patients, and types of medical conditions. Ultimately, this type of assessment may provide more refined information on the effects of medical interventions on different populations and in the context of different mixes of therapy.

The eHealth Initiative Foundation's Connecting for Drug Safety Collaboration

The eHealth Initiative Foundation's Connecting for Drug Safety Collaboration is a public-private sector effort designed to test and evaluate the feasibility and value of using electronic health information to support post-market surveillance and medical product safety. The findings from the Collaboration are intended to help inform the Food and Drug Administration's Sentinel Initiative. This work is strongly related to comparative effectiveness issues, since many of the same issues regarding uses of different data environments and basic scientific approaches would also apply to comparative effectiveness work.

Guided by eHI's multi-stakeholder Leadership Council and the Connecting for Drug Safety Advisory Board, this collaborative effort initially launched with a focus on two community-based healthcare organizations with advanced stage clinical information systems — Partners HealthCare System in Boston, MA and the Regenstrief Institute in Indianapolis, IN. During the course of the Collaboration's work, the Department of Defense was added as a third research community; findings are expected to be available in the near future. The Food and Drug Administration plays a critical advisory role in the Collaboration. The Agency for Healthcare Research and Quality and the Brookings Institution's Engelberg Center also serve in an advisory capacity.

The eHealth Initiative Drug Safety Collaboration sought to answer two basic questions:

1) What value do the different types of health care data sources bring to post-market drug safety questions?
2) What happens when the same drug safety research question is asked of different groups of researchers with expertise in their own data environments?

The eHealth Initiative Working Group on Using Health It for Comparative Effectiveness and Outcomes Research

The eHealth Initiative has also started a Working Group on Using Health IT for Comparative Effectiveness and Outcomes Research. The new working group brings together leaders from more than 25 organizations representing every sector of health care to learn, discuss, and work on this important theme in health care. Building on eHI's extensive efforts working with diverse groups of stakeholders to share best practices, test methods in real-world settings, and find common ground on policies, principles and strategies for improving health and health care through health IT and health information exchange

Objectives for Use of Real World Electronic Health Care Data

There is much that we do not know about the value or approaches of such work. It should not be surprising that trying to understand the basic building blocks of this research is itself a difficult matter. One could start by trying to articulate the goals of such exercises. Below are few objectives that may be connected.

1. Providing the most accurate and scientifically supportable assessment of medical conditions and interventions;
2. Gaining new scientific insights;
3. Assuring transparency, objectivity, and comparability in process and methods; and
4. Providing useful information for patients, providers, policymakers and other key stakeholders.

However, there are many stakeholders interested in this area. It will be important to get a clear understanding among stakeholders regarding such objectives.

Many stakeholders have different understandings of the possibilities of this research, reliability of this work, and definitions related to comparative effectiveness. The medical community, appropriately, does not want to oversimplify the importance and complexity of this process. It is important as we proceed that we develop trust and understanding. This means providing a clear framework for such research and finding a means to evaluate and communicate its reliability to a broad range of stakeholders.

Challenges for the Use of Real World Electronic Health Care Data

We need to acknowledge the many challenges of using real world data for comparative effectiveness research. By using real world data, researchers are outside the controlled setting of a clinical trial, and therefore are studying data on patients who may be taking multiple medications or dealing with multiple medical problems. In actual clinical practice, physicians may choose to prescribe one therapy over another on the basis of severity of disease, patient characteristics, and other factors that may not be apparent in health care data bases.
Unlike clinical trials, studies based on "real world" data are more likely to rely on incomplete information, since the data was collected for clinical care, not specifically for research. First, most patients do not have a comprehensive medical history located in one data source. Second, some patient records are not in electronic form and, thus, not practical for large data base studies. Third, claims data is set out for billing purposes and can be misleading with respect to actual diagnoses. Fourth, medical information terminology can vary from data source to data source. Finally, the type and amount of information available on given patients can vary. In such situations the data may be incomplete or inaccurate. This can also make combining results of analyses from different data sources a challenge.
Studies based on real world data are more susceptible to bias than randomized clinical trials. If one does not design a project to identify and eliminate sources of bias, it will make the conclusions less valid. The process for determining definitions or criteria for "real-world" study populations is important, requires judgment, and is itself a potential source of bias.

Priorities for Research

Among the priorities for research, we want to emphasize the need for research that involves collaborations in different data environments and research that explores the use of different types of electronic health care data. This is important, basic, and practical work that can be very informative in the development of a comparative effectiveness research infrastructure. Using available information, researchers and regulators use scientific procedures and judgment to try to determine the relationship between medical interventions and outcomes among different populations. The use of clinical data, in addition to claims data, as a source of information for adverse event detection is an emerging area. Therefore, researchers are still learning about the application of different methods for using clinical data, and best practices have yet to be defined. Building a base of trust, best practices and appropriate expectations from such comparative

effectiveness research will take time. The eHealth Initiative would like to work with you to help develop such a framework.

Another area that needs attention is research on how health information technology and electronic health information exchange can be used to create more robust data sources and to help evaluate comparative effectiveness issues across a broader range of settings.

Finally, it is important to focus on projects that address how research might facilitate or assist in medical decisions. Knowledge that is generated from this comparative effectiveness research could be extremely valuable to the medical decision-making process.

The eHealth Initiative looks forward to working with the Council as it proceeds to develop its recommendations, and thanks the Council for this opportunity for comment. If you have any questions, please feel free to contact me at Jennifer.Covich@ehealthinitiative.org.

Submitted by
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Thank you for this opportunity to provide comments on behalf of the American Psychological Association (APA) regarding national priorities for comparative effectiveness research. I am Dr. James Bray, APA President and Associate Professor of Family and Community Medicine and Psychiatry at the Baylor College of Medicine.

APA is the largest scientific and professional organization representing psychology with 150,000 members and affiliates. APA is also the largest publisher of behavioral science research, with 52 premier scholarly journals.

Comparative effectiveness research is a critically important tool for advancing an evidence-based approach to health care decision-making. However, the full public health benefits of such research will only be realized if behavioral, psychosocial, and medical interventions for the prevention and treatment of mental and physical health conditions are evaluated individually and in combination. Even when strictly medical treatments are compared, it is important to expand the range of outcome measures to include behavioral and psychological outcomes, such as quality of life and adherence to treatment protocols. It is also essential to evaluate promising new models of care, such as the use of integrated, interdisciplinary behavioral and medical teams in primary care settings. And finally, the effectiveness of health interventions across the lifespan and for different minority and gender groups must be considered.

Therefore, APA is recommending that comparative effectiveness research focus on these five areas:

We encourage research that compares different behavioral and psychosocial interventions for the prevention and treatment of specific health conditions. This research is crucial given that the leading causes of chronic health problems and mortality in the United States—such as heart disease, diabetes, and many forms of cancer—are due to modifiable behavioral factors, such as smoking, improper diet, lack of physical activity, and excessive alcohol consumption, among others. In addition, mental disorders, such as depression, represent a significant disease burden in the U.S. and worldwide. Fortunately, effective behavioral and psychosocial interventions exist to reduce life-threatening behaviors and treat health conditions, such as depression, heart disease, chronic pain, and diabetes. Now is the time to test the comparative effectiveness of these interventions to improve the health of the public.

Next, we strongly encourage research that compares behavioral and psychosocial interventions with medical interventions, and combinations thereof. This type of research allows for an examination of the relative and combined effectiveness of behavioral and medical interventions for specific health conditions.

A classic example of the value of this form of comparative research comes from the randomized controlled trial of the Diabetes Prevention program, which found that intensive lifestyle intervention, as compared to placebo or medication, reduced the incidence of type 2 diabetes to half that of placebo, and was significantly more effective than medication alone. Enhanced outcomes have also been found for combined behavioral and pharmacological interventions for depression and smoking.

Next, we should pursue research that compares integrated systems of care comprised of interdisciplinary teams of medical and behavioral health providers versus routine medical care. There is some indication that co-locating medical and behavioral health providers improves patient access and health outcomes.

For example, the integrated care approach has shown the largest reduction in depression levels and highest patient satisfaction. Interestingly, mortality was reduced on one recent large trial of integrated, primary care-based treatment of depression.

We also believe that all health research studies should include measures of behavioral and psychosocial outcomes, such as life quality, adherence to treatment protocols, behavioral functioning, depression, and anxiety. Such attention to patient-centered care builds upon the IOM's own definition of evidence-based practice.

As new life-saving medical advances are developed, we must strive to maintain patient quality of life. For example, depression and anxiety have been shown to increase in heart disease patients using implantable cardioverter defibrillators. Both the positive and negative outcomes of medical procedures need to be considered and evaluated before they are adopted as standard practice.

And finally, research that examines health intervention outcomes across the lifespan and for different minority and gender groups is needed to understand the effectiveness of

interventions within and between population groups. This type of comparative research is important given the well documented health disparities that exist between different racial/ethnic, age, socioeconomic status, gender, and sexual minority groups, and because it is not clear if specific behavioral and medical interventions are equally effective across groups. This type of comparative research is critical as the U.S. population becomes more diverse.

Thank you for the opportunity to provide this brief statement. The American Psychological Association looks forward to the outcome of your deliberations.

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The ACC strongly supports investment in comparative effectiveness research (CER). Given the high prevalence of heart disease-related illnesses, along with the documented variability in the use of procedures used to treat and/or diagnose it, comparative effectiveness research could yield high returns in terms of improving patient outcomes and reducing costs.

Specifically, comparative effectiveness research is an essential building block to understanding the populations that may benefit from the many treatment options associated with cardiovascular disease, including medical therapy, stents, surgery and defibrillators. Understanding the comparative effectiveness of both diagnostic and treatment strategies in different patient populations, especially the elderly, is very important. Effective detection and risk assessment of coronary artery disease can help physicians determine the most appropriate care for their patients, whether it be more aggressive therapy and procedures or less intensive cardiac follow-up.

In particular, comparative effectiveness research of diagnostic imaging is a high priority for the ACC (see list of top CER priorities below). Understanding the comparative effectiveness of various cardiac imaging and diagnostic tests could help better target the use of these beneficial technologies and help inform policy decisions related to the use of these technologies. Randomized trials and registries will be important to understanding the role of imaging and other diagnostic tests in not only diagnosis but their impact on downstream treatment and outcomes.

Facilitating linkages of clinical and administrative databases is crucial to developing this research agenda (see infrastructure priorities below). Inpatient and outpatient registries could help track key elements of comparative effectiveness, including laboratory results, medication adherence and diagnosis decisions. Translation of the results of comparative effectiveness research will require transparency in terms of how users discuss certainty of the resulting evidence, the trade-off of different types of risks and benefits, and what it means for two or more strategies to be equally effective.

Cost-effectiveness must also be a critical priority for health reform given the reality of finite financial resources and rising costs. However, the College strongly believes comparative effectiveness research must remain strictly focused on comparative clinical science, such that decisions regarding cost effectiveness can be made sequentially later. This segregation of these important processes will be essential to ensuring physician and patient trust in the ethics and integrity of such work.

Comparative effectiveness research has the potential to make it much easier for patients and their doctors to choose the best treatment and avoid unnecessary treatment for not only heart disease, but other diseases a well, thus improving quality and ensuring greater patient value. The College looks forward to working with you on this endeavor.

Top Comparative Effectiveness Research Priorities

1. **Comparison of diagnostic tests for suspected Coronary Artery Disease (CAD) (Randomized Clinical Trial).** Compare the effectiveness of stress electrocardiography, stress echo, coronary computed tomography angiography (CCTA), and single-photon emission computed tomography (SPECT MPI) for the diagnosis and risk assessment of coronary artery disease in adult, intermediate pre-test probability patients with stable chest pain by assessing impact on detection and risk assessment, subsequent treatment (medical and procedures), prior and subsequent invasive and non-invasive cardiac imaging utilization, major adverse cardiac events, and cost. As several proposed trials were submitted to NHBLI in February, this project is "shovel ready."

2. **Comparison of diagnostic tests for Coronary Artery Disease (CAD) (Registry).** Compare the effectiveness of stress electrocardiography, stress echo, coronary computed tomography angiography (CCTA), and single-photon emission computed tomography (SPECT MPI) for diagnosis and risk assessment of coronary artery disease by assessing appropriate use patterns based on published criteria, prior and subsequent invasive and non-invasive cardiac imaging utilization, subsequent procedures, overall radiation dose and other safety issues, major adverse coronary events for normal studies, and testing and downstream costs.

3. **Compare preventive strategies guided by coronary artery calcium scoring versus usual care (RCT).** Compare the effectiveness of using a coronary calcium screening directed prevention strategy versus usual care in asymptomatic individuals who have low to intermediate coronary artery disease risk by assessing major adverse coronary events, medication usage, adoption of secondary prevention therapy, quality of life, and cost

4. **Linking STS, NCDR, and CMS databases.** By linking the clinical data from the Society of Thoracic Surgeons National Database and the ACC National Cardiovascular Data Registry with Centers for Medicare and Medicaid Services (CMS) administrative data, a powerful, longitudinal data set could be produced. Such a data set would contain process, outcomes, utilization, pharmacy, and cost data spanning perhaps a three to five year period. This linking project has the potential to cover the continuum of care for cardiovascular disease. Ultimately, the information obtained from this collaborative effort will aid in the analysis of the comparative effectiveness of coronary revascularization treatment strategies, such as coronary bypass rafting and stenting procedures.

5. **Ablation versus cardioversion.** Compare the effectiveness of ablation therapy for the treatment of atrial fibrillation versus electrical cardioversion by assessing cardiac

function, mortality, major adverse cardiac events, quality of life, and cost in a real world setting by paralleling the current randomized clinical trial.

Top CER Infrastructure Initiatives (What types of investments in infrastructure for comparative effectiveness research should the Coordinating Council consider?)

1. Creation of robust national registries for tracking both short- and long-term performance of therapeutic strategies, drugs, or devices
2. Clinical registry development, application, and networking
3. Clinical data pooling and mining support
4. Capacity to use electronic health records for safety and effectiveness monitoring
5. Clinical trials support (e.g., ad hoc collaborations)

Criteria for Setting Priorities (What criteria should the Coordinating Council consider when evaluating different investment options?)

1. variability in care
2. potential to act on the information once generated
3. disease burden
4. utility of the answer for decision-making
5. cost

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The focus of this testimony is on the contribution comparative effectiveness research can make to the extension of the newly developed consensus standards for treatment of substance use conditions, published by the National Quality Forum (NQF). The Treatment Research Institute and the UCLA Integrated Substance Abuse Program have been asked to provide leadership for the dissemination and adoption of the consensus standards by States financing treatment in the public sector.

Despite the knowledge that addiction to alcohol and drugs is a treatable health condition, the failure to provide care consonant with scientific evidence is manifest in the lack of ongoing care for substance dependence consistent with the condition's chronic nature. Scientific advances have resulted in a spectrum of evidence-based psychosocial and pharmacologic treatments for individuals with unhealthy use or dependence on alcohol and/or drugs----treatments whose results compare well with those obtained with treatments for other chronic illnesses. It is increasingly apparent that patients with more chronic forms of substance use disorders require and do well with appropriately tailored continuing care and clinical support as in other chronic illnesses such as cardiovascular diseases and diabetes.

There are gaps, however, in knowledge of which evidence-based therapies are better than others, how to treat multiple co-morbidities present in so many patients, and how to care for some population subgroups. Despite these gaps, there has been an impressive increase in the number and quality of studies on efficacious therapies for adolescents and adults. Yet, there remain large gaps in the research on the *effectiveness* of these treatments when delivered in usual settings of care, especially on how the costs of treatment are integrated into the research. In particular, there are significant gaps in the efficacy and effectiveness research related to individuals who are neither "unhealthy" but risky users of alcohol and/or drugs nor chronically and severely dependent. For this "middle" group comparative effectiveness research seems particularly critical.

Developing standards of care for addiction treatment in the public and private sectors is a relatively recent activity. Between 2005 and 2007, with funding from the Robert Wood Johnson Foundation and the Federal Center for Substance Abuse Treatment/SAMHSA, the National Quality Forum (NQF) conducted a consensus process to identify evidence-based organizational and clinical practices for treatment of substance use disorders. The basic principles outlined by the NQF and the specific standards and practices that were identified should serve as the basis for moving forward with comparative effectiveness research in addiction treatment. In addition to publishing the standards and practices, the NQF recommended additional research be conducted to improve the identified practices and the development and implementation of performance measures related to each practice standard.

There are differing views about the acceptability of various forms of evidence, what level of evidence is necessary for a practice to be endorsed as "evidence-based," and whether knowledge of evidence-based care for a population can be adapted to meet an individual's unique needs. Although the Agency for Healthcare Research and Quality has (AHRQ) identified sound methods for rating the strength of scientific evidence, its findings have not resolved debates about whether a given intervention is evidence-based, for whom, and under what conditions.

The focus on randomized clinical trials, while the gold standard for generating clinical evidence, cannot be relied upon exclusively to identify evidence-based care due to the sheer numbers of possible psychosocial and pharmacological interventions for substance use disorders. Furthermore, clinical trials do not generally include economic and cost analyses essential for comparative effectiveness research. Costs are borne not only by individuals and families, but by treatment organizations and, in the case of substance use disorders, almost entirely by the public sector. By 2014, the estimates made by a study of national expenditures funded by SAMHSA and published in *Health Affairs* (2008) show that we can expect about 85% of treatment expenditures for substance use disorders to be paid for with public financing—Federal, State, and local dollars.

Of critical importance to advancing the consensus standards identified by the NQF is a focus on the systematic analyses necessary to translate the evidence being generated in scientific research into clinically useful practice guidelines. Many of the numerous professional groups involved in treatment of substance use disorders have conducted their own reviews of the evidence and promulgated their own practice guidelines. The guidelines include little discussion of what might be included in comparative effectiveness research----the characteristics of the individuals

targeted for specific interventions, the structural and financing characteristics of organizations that are necessary for adoption of specific guidelines, the costs of the identified interventions to treatment organizations that are responsible for implementation related to training and staffing requirements, and the like. Numerous studies have identified clinicians' departures from evidence-based practice guidelines for opioid dependence and other substance use disorders.

If we expect improvement in the quality of care for treatment of substance use disorders, the clinical appropriateness of such variations needs become a focus of performance measurement. Performance measurement for addiction treatment at the treatment system level (health plans, States, and Counties) was first advanced by the Washington Circle Group (WCG). The WCG, a group of researchers, policy makers, States, and practitioners, since 1998 has worked to identify the processes of care necessary for addiction treatment, specify and test performance measures, and work with accrediting bodies and purchasers to adopt and utilize the measures. If we want an "uptake" in the use of evidence-based practices, measures are necessary, in addition to comparative effectiveness research, to be able to assure treatment organizations and clinicians that the evidence-based standards and practices they are being asked to adhere to have clear advantages in effectiveness and cost-effectiveness relative to the practices they are employing.

Submitted by
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Members of the Federal Coordinating Council (FCC), I am Andrew Sperling, Director of Legislative Advocacy for the National Alliance on Mental Illness (NAMI). NAMI is the largest national organization representing individuals living with serious mental illness and their families. Through our more than 1,100 affiliates in all 50 states NAMI is engaged in support, education and advocacy around serious mental illness.

NAMI believes strongly in the promise of comparative effectiveness research to improve quality and outcomes in health care. As a member of the Partnership to Improve Patient Care (PIPC), NAMI has endorsed a set of principles that we believe can help ensure that comparative effectiveness meets its full potential and does not become a blunt instrument that limits patient choice and results in cost becoming the dominant factor in guiding treatment decisions. Among these principles for ensuring that comparative effectiveness research is patient-centered:

- Defining CER as a tool to improve patient care;
- Focusing on communicating research results to the public, not making centralized coverage and payment decisions;
- Providing information on clinical value and patient health outcomes, not cost effectiveness assessments;
- Recognizing the diversity, including racial and ethnic diversity, of patient populations and communicating results in ways that reflect the differences in individual patient needs;

- Examining all aspects of health care – including medical interventions, care management, benefit design, and processes of care – that can improve care quality and reduce health care disparities; and
- Requiring open and transparent processes where all stakeholders have equal voice in governance and input into research priorities.

In moving forward to develop recommendations and research priorities for the Secretary, NAMI urges the Coordinating Council to think big and undertake research directed at the most important challenges facing our nation's health care system. The Coordinating Council should resist any focus on short-term clinical trials that simply compare two distinct interventions such as head-to-head comparisons of two medications. Rather, comparative effectiveness research should examine the range of issues that affect the quality of patient care (medical tests and treatments, health care delivery and organization, benefit designs and care management). All of these elements of health care affect patients' quality of care and health outcomes. These elements of care also have a significant impact on health care disparities. Research is needed to identify the best approaches to reducing disparities. In addition, sound comparative effectiveness research should include the different factors important to consumers, including quality of life, independence, productivity and recovery.

CER that starts with cost containment as a central goal will not lead to studies that answer these questions, and will likely result in misapplication of findings in order to achieve cost-cutting objectives. By contrast, CER that begins with the goal of quality improvement can help everyone in health care make better decisions and will ultimately lead to better health care value and greater cost efficiencies. This requires addressing the different factors that can help decide which treatment is optimal for the individual, such as the patient's particular medical condition, past treatment history and genetic variations.

Basing Public Policy Decisions on Comparative Effectiveness Has Limitations

NAMI would like to caution the Coordinating Council with regard to the limitations and difficulties associated with using existing comparative effectiveness studies that are now being used to drive policies related to treatment choice and prescribing decisions. There is no better example of this than the NIMH CATIE trial on antipsychotic medications. The Clinical Antipsychotic Trials of Intervention Effectiveness (CATIE) was the first large scale attempt to compare five different medications used to treat people living with schizophrenia. The study included both newer (atypical) and one older anti-psychotic medication. In general terms, the study found that there was relatively little difference in the effectiveness of any of the medications. Without regard to which medication was randomly assigned, patients discontinued the use of medications at similar rates due to intolerable side effects or failure to adequately control symptoms.

It is important to note that CATIE used discontinuation as a proxy for efficacy. Further, the protocols for the study required that patients be "randomized" to one of the five drugs. In NAMI's view, this randomization does not reflect best clinical practice which calls for a dialog between a medical professional and consumer that helps determine a best-choice medication based upon treatment goals and risk of side-effects. In addition, CATIE was only 18 months in

length, not enough time to accurately measure serious neurological side effects known as extrapyramidal symptoms (EPS) and movement disorders such as Tardive Dyskinesia associated with the older antipsychotic medications.

More importantly, CATIE is NOT the basis for any conclusion that "the older antipsychotic medications are just as good as the newer ones." In fact, 69% of the participants switched to a different medication at least once during the trial and just over half switched twice or more. If anything, CATIE supports a strong conclusion that there is no "one size fits all" in any aspect of schizophrenia treatment. At the systems level, patients and their support system should be skeptical of any attempt to change medications if treatment is demonstrating progress. NAMI believes that states should never engage in the practice of medicine.

Roles and Responsibilities for the Federal Coordinating Council

The announcement for this Listening Session in the Federal Register details 6 specific questions on which the Coordinating Council is seeking input from stakeholders and the general public. NAMI would like to address each of these questions separately

1. What types of investments in infrastructure for comparative effectiveness research should the Coordinating Council consider?

Investments in infrastructure of CER should be carefully considered. The $1.1 billion included in the ARRA is not a permanent authorization and it is uncertain whether Congress will continue to appropriate funding for CER beyond the 24 to 36 month time period authorized in the law. While investment in multi-site clinical trial networks and training for researchers and investigators are critical to furthering research and discovery, they cannot sustain themselves without ongoing funding from Congress over the long-term – far beyond the ARRA timeframe.

NAMI urges that these ongoing costs associated with high quality biomedical research continue to be a part of the NIH where they can be more appropriately maintained and developed over time.

2. What criteria should the Coordinating Council consider when evaluating different investment options?

As noted above, NAMI recommends investment of CER resources into the most difficult challenges facing our health care system, especially in the area of chronic disease management of conditions that represent the greatest public health burden. Investment in short-term head-to-head comparisons of specific interventions may be quicker and easier, but they will not help us move forward in addressing the most costly and difficult challenges such as the growing prevalence of obesity, diabetes heart disease, COPD, etc. The Coordinating Council should set forth criteria that prioritize examination of effective multi-systemic interventions in real world treatment settings among patients with multiple co-morbidities. NAMI would note for the record that adults with serious mental illness are experiencing significantly higher rates of the medical co-morbidities and experience (on average) as much as 25 years of lower life expectancy according to a 2006 study from the National Association of State Mental Health Program Directors (NASMHPD).

http://www.nasmhpd.org/general_files/publications/med_directors_pubs/Mortality%20and%20Morbidity%20Final%20Report%208.18.08.pdf

3. What Federal government activities in the area of comparative effectiveness research should the Coordinating Council focus its attention on?

NAMI urges the Coordinating Council to adhere to the activities set forth by Congress in establishing the Council as part of the ARRA, i.e. fostering coordination and advising the President and Congress. We note that the ARRA specifically bars the Coordinating Council from mandating coverage or reimbursement decisions or policies for both public and private payors. In addition, the ARRA explicitly states that Council recommendations shall not be construed as mandates or clinical guidelines for payment coverage or treatment.

4. How can the Coordinating Council best foster integration of these activities across the programs managed by the Departments of Health and Human Services, Defense, and Veterans Affairs?

NAMI urges regular meetings of the Coordinating Council and an open and transparent process that allows for maximum participation for all stakeholders, including patients. This should include the participation of disease advocacy organizations as the voice of patients, as opposed to the more general views of "consumer organizations." This distinction is critical since individuals living with chronic and life threatening illnesses (including serious mental illnesses) and their families are likely to offer unique perspectives on the importance of maintaining broad access to widest array of therapies and inventions. Inclusion of all stakeholders will also help ensure that unique needs of subpopulations, especially racial and ethnic minorities are integrated into these discussions. It is a perspective distinct from a "consumer" voice for the "average" patient.

5. What steps should the Coordinating Council consider to help ensure that public- and private-sector efforts in the area of comparative effectiveness research are mutually supportive?

Continuing regular public meetings such as this Listening Session will be critical to establishing a mutually supportive environment for public and private CER investments. NAMI also urged that, to the maximum extent possible, all Coordinating Council activities and meetings adhere to the standards in the Federal Advisory Council Act – advance public notice of meetings and meeting agendas, a public record, opportunity for public comment, etc.

6. What information on the Coordinating Council's activities would be most useful?

NAMI would be most interested in viewing information provided to the Coordinating Council by individual federal agencies and officials. It would therefore be helpful if any and all submissions from federal agencies be posted to the Council's website. Likewise NAMI recommends that all of the Committee's deliberations be conducted in public.

Submitted by
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Dear Dr. Conway:

The Suicide Prevention Action Network USA (SPAN USA) welcomes the opportunity to submit comments regarding the Federal Coordinating Council on Comparative Effectiveness Research (FCC-CER). SPAN USA is a 501(c)(3) organization dedicated to preventing suicide through public education and awareness, community action and federal, state and local grassroots advocacy. As you know well, suicide is the 11th leading cause of death in America and the third leading cause of death for younger Americans age 15-24. In addition, it is estimated that there are 800,000 suicide attempts each year. Reducing the number of suicides and suicide attempts among our nation's citizens is a criterion that the Coordinating Council should consider when evaluating different investment options.

As noted in the Federal Register Notice, there are investments in infrastructure for comparative effectiveness research that are needed. Currently, there is woefully inadequate data on suicide and suicide attempts in America. No government agency or private entity can determine exactly how many veterans die by suicide each year across America or how many murder-suicides have occurred. The National Violent Death Reporting System collects data from medical examiners, coroners, police, crime labs, and death certificates to understand the circumstances surrounding violent deaths including suicide. This information is important to develop, inform, and evaluate suicide prevention programs. However, the system does not operate in every state, but the Centers for Disease Control and Prevention (CDC) has the capability to continue expanding the system until all 50 states are covered. The Coordinating Council should consider investment in data systems infrastructure for suicides in order for comparative effectiveness research to be as useful as possible.

SPAN USA recommends focusing attention on government activities involving comparative effectiveness research on best practices for early intervention and prevention of suicide. The federal government has undertaken a number of activities to complete the goals and objectives of the National Strategy for Suicide Prevention. Unfortunately, at this time there are only 16 interventions listed in the Substance Abuse and Mental Health Services Administration's National Registry of Evidence-Based Programs and Practices. Additional research to determine evidence-based programs and practices that reduce suicide and suicide attempts are vital to reducing the public health problem of suicide.

For example, SPAN USA recommends a study to compare the safety and effectiveness of inpatient psychiatric hospitalization vs. the use of alternative service options (e.g., extended observation, partial hospital, and intensive outpatient care) for individuals following a suicide attempt. Following a suicide attempt, it is necessary to evaluate and monitor individuals in safe and risk-appropriate settings. An important research priority is to determine whether psychiatric inpatient hospitalization is the most cost-effective and least restrictive manner of providing care to acutely suicidal individuals, mindful of safety concerns. The use of alternative crisis services — e.g., extended observation beds, intensive outpatient services — may result in fewer repeat inpatient admissions while also allowing patients greater autonomy and increasing their collaboration with community-based treatments. Fear of adverse outcomes has hampered necessary, well-designed, risk-attentive comparative studies.

The Coordinating Council can foster integration of suicide prevention comparative effectiveness research activities across the programs managed by the Departments of Health and Human Services, Defense, and Veterans Affair by working with the Federal Working Group on Suicide Prevention that is co-chaired by staff from SAMHSA and the U.S. Marine Corps. Integration of suicide prevention research is already taking shape in the form of a $50M research study being conducted by the National Institute of Mental Health and the U.S. Department of Army. However, more research specific to comparative effectiveness for treatments, programs and practices to reduce suicide among veterans and military personnel is needed.

Once again, SPAN USA appreciates the opportunity to share our comments with the FCC-CER. Please do not hesitate to contact me at: baltman@spanusa.org or 202-449-3600 with any questions or concerns.

Submitted by
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On behalf of the American Association of Neurological Surgeons (AANS) and the Congress of Neurological Surgeons (CNS), which together represent 4,000 practicing neurosurgeons across the United States, I would like to thank the Federal Coordinating Council for Comparative Effectiveness Research for giving us the opportunity to comment on comparative effective research priorities. My name is Rachel Groman, and I am the Senior Manager for Quality Improvement and Research in the AANS and CNS Washington Office.

Organized neurosurgery supports a ***well-designed*** comparative effectiveness research system that strengthens physician and patient decision-making, improves quality, and supports continued medical progress. Our members are committed to determining what medical treatments work best for their patients and our specialty is taking a variety of steps to ensure that the care neurosurgeons deliver is evidence-based. We have a robust practice guidelines development program and our specialty recently created a new clinical data registry entity called NeuroPoint Alliance. The NeuroPoint Alliance is partnering with Outcome Sciences, Inc. to build a database platform for a specialty-wide patient registry that will serve multiple purposes, including

Maintenance of Certification, clinical research, pay-for-performance and other quality improvement programs.

The AANS and CNS are very enthusiastic about partnering with the federal government, third party payers and others to conduct comparative effectiveness research that is important to neurosurgeons and their patients. To that end, we have identified a research priority that affects millions of American -- the treatment of common spinal disorders.

It is no secret that 75-85% of all Americans will experience back pain at some point in their lifetime and that management of chronic spinal conditions in the U.S. is estimated to cost nearly $85-100 billion each year. Despite advances in the surgical treatment of spinal diseases, which has dramatically expanded the treatment options available to patients, there is a deficiency of high quality clinical research to guide practice. This is largely due to the inherent difficulties of performing randomized controlled trials for surgical procedures and disorders characterized by significant heterogeneity, which often results in low patient compliance with randomized assignment. Consequently, many of the current clinical guidelines lack definitive guidance, resulting in marginal consensus among clinicians on what constitutes best practice and overall clinical uncertainty regarding the treatment of common spinal disorders. This has been reflected in significant regional variations in the treatment of neck and lower back degenerative diseases.

The AANS and CNS recently provided the Institute of Medicine (IOM) with specific clinical research recommendations focusing on two degenerative spinal diseases for which there is little high quality clinical research to guide practice: 1) low back pain without neurological deficit or spinal deformity; and 2) cervical spondylotic myelopathy. Low back pain is the fifth most common reason for seeing a physician in the United States, and cervical spondylotic myelopathy is the most common cause of spinal cord injury in both the United States and the world. Given the limitations of randomized clinical trials, we asked the IOM to consider the value of prospectively obtained data collected through patient registries to help identify specific patient characteristics that would serve as predictors of improved outcomes from different surgical and non-surgical approaches to these two diseases. We strongly encourage the Federal Coordinating Council to similarly consider the value of directing comparative effectiveness research funds to the creation and/or administration of a multi-center, prospective patient registry that could collect comparative data on different treatment approaches for degenerative spinal disorders. The AANS and CNS believe that the NeuroPoint Alliance is poised to serve as this registry, since it will produce meaningful data that will help refine indications, guide clinical decision-making, determine best practices, improve quality, and ultimately lower costs.

Thank you again for the opportunity to provide feedback on national comparative effectiveness research priorities. Organized neurosurgery looks forward to further exploring our registry project with the Federal Coordinating Council and to working with the federal government to collect the data needed to determine which treatments work best for neurosurgical patients.

Submitted by
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Dear Council Members:

The American Osteopathic Association (AOA) appreciates the opportunity to submit comments on comparative effectiveness research to the Federal Coordinating Council on Comparative Effectiveness Research as established by the American Recovery and Reinvestment Act of 2009. The AOA represents 64,000 osteopathic physicians nationwide, promotes public health, encourages scientific research, serves as the primary certifying body for D.O.s, and is the accrediting agency for all osteopathic medical colleges and health care facilities.

The purpose of our comments is to share our views on clinical and cost effectiveness research, inform the Council about AOA's guiding principles on comparative effectiveness research and its priority areas for comparative effectiveness research.

Clinical and Cost Effectiveness of Comparative Effectiveness Research

The AOA understands the purpose of the Federal Coordinating Council is to assist the offices and agencies of the Federal Government, including the Departments of Health and Human Services, Veterans Affairs, and Defense, and other Federal departments or agencies in coordinating the conduct or support of comparative effectiveness and related health services research; advise the President and Congress on strategies with respect to the infrastructure needs of comparative effectiveness research within the Federal Government; and assure optimum coordination of comparative effectiveness and related health services research conducted or supported by relevant Federal departments and agencies, with the goal of reducing duplicative efforts and encouraging coordinated and complementary use of resources.

A March 19, 2009 News Release of the Department of Health and Human Services states that the Council will help coordinate research and guide investments in comparative effectiveness research and will not recommend clinical guidelines for payment, coverage or treatment. Some Federal officials have stated that a portion of Recovery Act funds could be used for comparative effectiveness research that includes comparisons on the cost of treatments, but that the findings could not be used by Medicare as the basis of coverage decisions.

The AOA believes that it is in the best interests of the patient to exclude cost effectiveness of therapeutic or medical interventions from the decision-making process. Comparing the cost of interventions when the activity occurs between a patient and a physician with the final decision resting with the patient is entirely different from the use of cost effectiveness to deny coverage or treatment based on the cost of the intervention.

In our opinion, for comparative effectiveness research to realize its full potential, it must focus primarily on clinical efficacy. If the primary focus is on clinical efficacy, weighted against efficiency, physicians and patients will have relevant clinical information enabling them to make informed decisions on what is the best course of action. If clinical efficacy is removed as the primary focus, patients and physicians potentially may view such information from a negative perspective and the government's investment in comparative effectiveness research would fall short of our joint goals of improving quality, safety, and efficiency.

As stated in the following AOA Principles on Comparative Effectiveness Research, the physician-patient relationship must be protected. This includes the ability of physicians to provide individualized care using comparative effectiveness research as a recommended course of action, not a dictate and not to deny treatment based on cost effectiveness.

AOA Principles Regarding Comparative Effectiveness Research

Physicians and Patients

- Comparative effectiveness research should enhance the ability of osteopathic physicians (D.O.s) to provide the highest quality care to patients utilizing the best proven and widely accepted evidence based medical information at the time of treatment.
- Comparative effectiveness research should not be used to control medical decision-making authority or professional autonomy.
- Comparative effectiveness research should enhance, complement, and promote patient care, not impede it.
- Guidelines developed as a result of comparative effectiveness research studies should be advisory and not mandatory.
- Comparative effectiveness research should be viewed as a positive development for patients and physicians and a useful tool in the physician's armamentarium, working in concert with patients.
- Physicians in practice should be included in any discussions and decisions regarding comparative effectiveness research.
- Comparative effectiveness research should focus on clinical effectiveness, not cost effectiveness, and should not be used to deny coverage or payment.
- The physician/patient relationship must be protected and the needs of the patients should be paramount.

Location of a Comparative Effectiveness Research Institute

- The AOA would prefer that the Agency for Healthcare Research and Quality (AHRQ) be the home for comparative effectiveness research. Section 1013 of the Medicare Modernization Act (MMA) authorizes AHRQ to conduct comparative effectiveness research. AHRQ has been doing so since 2005. Under its Effective Health Care Program, AHRQ published studies on gastroesophageal reflux disease, renal artery stenosis, osteoporosis, osteoarthritis, rheumatoid arthritis, diabetes, depression, psychiatric disorders, hypertension, and prostate cancer.
- The AOA believes that AHRQ could collaborate with the National Institutes of Health (NIH) and other entities in cases where clinical trials or other resources are needed.

Funding

- The AOA believes that Congress should increase the current $50 million authorized in Section 1013 of MMA to a level commensurate with the funds necessary to carry out an expanded role in comparative effectiveness research.
- The AOA believes that there could be room for private funding provided that contributors are not in a position to influence study outcomes.

Governing Board

- The AOA believes that for comparative effectiveness research to be successful, all stakeholders (physicians, patients, researchers, government, and private sector) must be represented in the decision-making process.

AOA Comparative Effectiveness Research Priorities

On March 27, 2009, the AOA was pleased to submit the following research priorities to the Institute of Medicine (IOM) Committee on Comparative Effectiveness Research Priorities pursuant to the IOM's request for comment to its research priorities questionnaire:

- Compare the effectiveness of adding osteopathic manipulative treatment (OMT) to "best care" in low back pain in the primary care setting.

- Compare the effectiveness of coordinated care in the treatment of diabetics in the community versus standard care.

- Compare models of physician led community primary prevention in progression of glucose intolerance to diabetes.

The AOA thanks the Federal Coordinating Council on Comparative Effectiveness for considering our views.

Submitted by
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Comparative effectiveness research is imperative to better understand and improve the provision of care for children with asthma. We recommend prioritizing studies that compare, with respect to both cost savings and clinical outcomes, the effectiveness of an integrated asthma counselor/environmental mitigation chronic disease management model with a non-integrated episodic model for children with asthma. Childhood asthma is a significant chronic disease that has reached epidemic proportions in heavily disadvantaged communities, placing a disproportionate burden on low-income and minority families and communities. Childhood asthma carries high direct and indirect clinical, social, and economic costs - an estimated $20

billion in 2007 alone – in the form of preventable emergency department visits and inpatient hospital admissions, lost productivity, school absenteeism and family stress. Paradoxically, while much is known about effective integrated pediatric asthma management (including USPSTF recommendations for "home-based multi-trigger multi-component environmental interventions" for children with asthma), insurers continue to utilize coverage, cost sharing, and payment design strategies that treat asthma as an isolated episodic illness, and pediatric practice and public health have failed to systematically restructure their approach to emphasize an integrated chronic care/public health intervention.

A proposed model of comparison is an integrated asthma counselor/environmental mitigation chronic disease model with a non-integrated, episodic model of care. This can be accomplished through a range of health services research- including interventions specifically designed to better understand novel treatment pathways and comparisons of the standard of care/ usual care with new models as proposed above. This research should include children of all ages (young children and adolescents) and racial/ethnic minority groups.

The epidemic proportion of asthma among child populations at risk for social risk and heavy disease burden, coupled with the high costs associated with ineffective care, make asthma a prime candidate for comparative effectiveness research in pediatric health. The availability of a growing scientific and public health evidence-base, including appropriate models of integrated chronic disease management, also make asthma a priority where it is possible to improve the quality of life for children and families while reducing health care costs.

Submitted by
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The health professional educational system, particularly the education of physicians developed, over the last 100 years as a series of 'fixes' designed to ameliorate perceived gaps and deficiencies at specific points in time. This evolution took hold most notably with the publication of the AMA Council on Medical Education sponsored report of the Carnegie Foundation in 1910. The Flexnor report detailed the significant heterogeneity and inadequacy of physician training as the legacy of the 19th century. Since Flexnor, medical educators have put into place a cornucopia of solutions intended to assure the public that doctors and their care are of high quality care. These include, but are not limited to, medical school accreditation (LCME), residency program accreditation (ACGME), continuing medical education accreditation (ACCME, AMA PRA), board certification (ABMS), and state licensure (NBME, state licensure boards and FSMB).

Similar and parallel structures have developed in efforts to assure the public of the adequacy of nurses, pharmacists and other health professionals' education and maintenance of skills over a professional life. In addition, a concurrent system of public and private systems to evaluate and

accredit health care delivery systems has evolved.

Despite these efforts designed to assure high professional standards, it appears that the current educational systems for health professionals are inadequate in providing the requisite skills, knowledge and attitudes to assure the public of the highest standards of safe, quality care. This may be due in part to several factors. While not exhaustive, these include: the learning environment within professional schools, post graduate training programs and systems of continuing education, fragmentation of roles and responsibilities within the delivery system, financial incentives/disincentives which reward technical expertise and fail to penalize significantly for failure to measure, account and improve poor performance.

Unlike a heavy and sustained national investment in biomedical research, and more recently in health services research, there have not been significant resources devoted to developing and sustaining an infrastructure upon which to conduct meaningful and longitudinal research regarding the impact of innovation in health professional education. Limited resources have constrained our ability to understand the most effective methods by which to improve our educational systems and drive them toward expected educational outcomes. The limitation includes resources to conduct pilot, demonstration or multi-institutional collaborative projects, and longitudinal cohort studies of the impact of the educational continuum on practice attitudes, values, behaviors and outcomes. Further, despite the wealth of talent that resides within our institutions of higher education, little effort has been expended to capitalize on knowledge and learning from other disciplines which could reasonably be expected to have insight into methods to improve outcomes. Mechanisms designed to facilitate interdisciplinary research should be encouraged.

In sum, comparative effectiveness research funding should be considered broadly by the AHRQ and the oversight group. Comparative effectiveness of methods, mechanisms (i.e., tools) and outcomes from the health professional educational system, at all levels (i.e., pre and post degree) and across disciplines (i.e., medicine, nursing, pharmacy, other) should be considered at this time as part of the total research equation to best understand what works and does not work in the health delivery system.

Jibril M Hirsi
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I am the president of the Somali community in Ohio. I would like to suggest an increase in engagement and outreach services to help the Somali American community in the USA.

The Somali American community faces serious physical and mental health challenges including chronic medical problems, adjustment problems, nutrition issues and other hardships associated with the change in their environment. A basic outreach and community health education initiative can change the lives of many Somali Americans for the better.

Please consider this statement for inclusion in the hearing.

Submitted by
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Dear Committee,

Real world clinical and cost effectiveness of ICDs are hard to extrapolate from clinical trials. Large health care networks are an ideal place to gather this information. For example, HCA owns hospitals that cross different geographies, practice patterns, and their patients are integrated into the community (as opposed to “captive” health systems). By linking their hospitals with a centralized patient tracking and outcome system, one can both measure treatment effectiveness, and test practice enhancements. This could serve as a test tube for what is possible through a fully integrated national electronic health record.

This registry could assess the effectiveness of ICD therapy and patient screening techniques. Microvolt T wave alternans (MTWA) is an inexpensive and non-invasive technique that has significant potential to fill this role, but will require government support to fully explore its potential. Many studies have shown MTWA to predict total and arrhythmic death, and patients most likely to benefit from ICD therapy. Recent “negative” MTWA studies, especially the MASTER Trial, have slowed adoption. However these negative studies have significant limitations that could invalidate the conclusions. Also, since MTWA is dynamic, chronic MTWA measurement (which has not been explored) could have additional benefit. More importantly, MTWA could serve as a means for optimizing medical treatment of CAD, heart failure, and arrhythmia—thereby reducing risk of costly hospitalization.

I propose an integrated data collection system within an organization like HCA to explore the "real world" impact of treatments, tests (including MTWA), and practice enhancements.

Submitted by
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The American Society of Clinical Oncology (ASCO) is the leading specialty society in the United States and throughout the world for physicians who treat patients with cancer and conduct cancer research to improve patient outcomes. ASCO is committed to ensuring that high-quality,

evidence-based practices for the prevention, diagnosis and treatment of cancer are available to all Americans. This statement highlights both the importance of comparative effectiveness research in our community today, and offers suggestions for ways to enhance this science moving forward.

Because cancer is a complex illness that touches many aspects of the health care system, oncology can serve as an important laboratory for comparative effectiveness studies. Comparative clinical research is embedded in the culture of oncology. The extensive cancer clinical trials network across the U.S. has a long track record of pursuing comparative effectiveness research. Examples include:

- National prostate, lung, colorectal, and ovarian cancer screening trials;
- A large portfolio of trials and meta-analyses comparing different regimens used for adjuvant therapy of operable breast, colon, and lung cancer; and
- Correlative studies that compare relative effectiveness of alternative drug regimens or targeted agents in patient subgroups defined by presence or absence of specific biomarkers. An important point for this last example is that, for most of these studies, treatment occurred several years ago, outcomes are already known, and banked tumor tissue permitted subsequent evaluation of predictive biomarkers. This provides a useful model for comparative effectiveness research that can lead to individualized treatment choices.

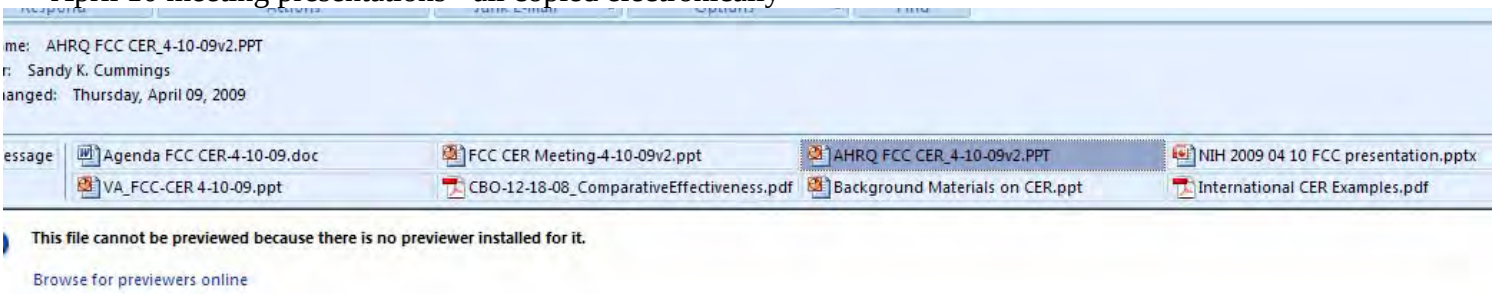
Much of this work has been possible because of our national cooperative group system, including disease site-specific scientific steering committees established by the National Cancer Institute's Coordinating Center for Clinical Trials, broad community involvement through the Community Clinical Oncology Program (CCOPs) and Cancer Trials Support Unit, a national registry program, and the multidisciplinary nature of our specialty. This network is already in place.

As you continue to work on shaping a national program on comparative effectiveness, we strongly recommend:

- Involvement of oncology experts in setting cancer-related priorities,
- Use and strengthening of existing oncology infrastructure to accomplish this work;
- Focus on areas where randomized clinical trials have established baseline data;
- Rigorous, standardized collection and storage of biospecimens in a way that allows broad access;
- Consideration of an oncology-specific evidence-based practice center; and
- Significant investment in expanding, strengthening, and linking national registries to include more robust data on individual patients' baseline characteristics, biomarker assay results, and specific treatment regimens.

Thank you for the opportunity to submit this statement. ASCO looks forward to working with you and others in the medical community as we move forward in this important area.

April 10 meeting presentations -all copied electronically



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Background Materials on CER

Federal Coordinating Council

Contents

- Definitions of CER
- Domestic Public Example - DERP
- Domestic Private example – Blue Cross Blue Shield Technology Evaluation Center

Definitions of CER

- CBO
  - Comparative effectiveness analysis is a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches, such as surgery and drug therapy. The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options. In some cases, a given treatment may prove to be more effective clinically or more cost-effective for a broad range of patients, but frequently a key issue is determining which specific types of patients would benefit most from it.

Definitions

- MedPAC
  - Comparative-effectiveness analysis evaluates the relative effectiveness, safety, and cost of medical services, drugs, devices, therapies, and procedures used to treat the same condition ... Effectiveness implies the “realworld” performance of clinically relevant alternatives provided to patients with diverse clinical characteristics in a wide variety of practice settings. The outcomes that researchers assess in comparative effectiveness studies may include: • clinical outcomes, including traditional clinical endpoints, such as mortality and major morbidity; • functional endpoints, such as quality of life, symptom severity, and patient satisfaction; and • economic outcomes, including the cost of health care services and cost effectiveness. Some comparative studies only contrast the clinical and functional outcomes of alternative treatments while others also compare cost and assess cost effectiveness.

Definitions

- IOM
  - The terms ‘effectiveness’ and ‘clinical effectiveness’ refer to the extent to which a specific intervention, procedure, regimen, or service does what it what it is intended to do when it is used under real world circumstances …Recently, numerous proposals have called for a large expansion in the generation of comparative effectiveness information… These proposals call for systems to compare the impacts of different options for caring for a medical condition (e.g., prostate cancer) for a defined set of patients (e.g., men at high risk of prostate cancer recurrence). The comparison may be between similar treatments, such as competing prescription medications, or for very different treatment approaches, such as surgery or radiation therapy. Or, the comparison may be between using a specific intervention and its nonuse (sometimes called ‘watchful waiting’).

Definitions

- AHRQ
  - A type of health care research that compares the results of one approach for managing a disease to the results of other approaches. Comparative effectiveness usually compares two or more types of treatment, such as different drugs, for the same disease. Comparative effectiveness also can compare types of surgery or other kinds of medical procedures and tests. The results often are summarized in a systematic review. The kinds of results that are studied to compare drugs or procedures include relief of symptoms, length of life, or whether people need to go to the hospital. These results are called outcomes. Many other kinds of outcomes can also be compared.

Contents

- Definitions of CER
- Domestic Public Example - DERP
- Domestic Private example – Blue Cross Blue Shield Technology Evaluation Center

State Experience Using CER: DERP

- Drug Evidence Review Project
  - Initiated by state of Oregon in 2000
  - Collaboration of public entities, the Center for Evidence-based Policy and the Oregon Evidence-based Practice Center, who have joined together to produce systematic, evidence-based reviews of the comparative effectiveness and safety of drugs in many widely used drug classes, and to apply the findings to inform public policy and related activities in local settings
  - The DERP project produces systematic literature reviews of drug classes. Components of these reviews include Key Questions, Reports and Evidence Tables.

Source: Mark Gibson, Deputy Director of Center for Evidence-based Policy and DERP website

DERP Transparency

- Draft Key questions posted and comments incorporated
  - Full disclosure of methods and sources–Which studies included and why
  - Which studies not included and why
  - All industry submissions
- Universal peer review w/ comments public
- Final report in public domain

Source: Mark Gibson, Deputy Director of Center for Evidence-based Policy and DERP website

Reports Completed by DERP

- 2nd Generation Antidepressants
- 2nd Generation Antihistamines
- Alzheimer's Drugs
- Angiotensin Converting Enzyme Inhibitors
- Angiotensin II Receptor Antagonists
- Anti-Epileptic Drugs
- Anti-platelet Drugs
- Controller Drugs for Asthma
- Atypical Antipsychotics
- Quick Relief Medications for Asthma
- Beta Adrenergic Blockers
- Calcium Channel Blockers
- Combination Drugs for Hypertension & Hyperlipidemia
- Constipation Drugs
- Newer Diabetes Drugs
- Renin Angiotensin Aldosterone System Drugs
- Drugs to treat ADHD
- Hepatitis C Drugs
- Hormone Replacement therapy
- Long-acting Opiates
- MS Drugs
- Neuropathic Pain Drugs
- Newer Antiemetics
- Newer Insomnia Drugs
- NSAIDS
- Oral Hypoglycemics
- Proton Pump Inhibitors
- Statins
- 59 more updated reports

Source: Mark Gibson, Deputy Director of Center for Evidence-based Policy and DERP website

DERP Lessons Learned

- Publicly sponsored and governed project can produce highest quality evidence in U.S.
- Research informs not dictates policy
  - Structure of industry interface important–Need evidence not lobbying (independence)
  - Must be formal
  - Must be transparent
- Cost must be considered (policy process)
- Big gaps in evidence need filling

Source: Mark Gibson, Deputy Director of Center for Evidence-based Policy and DERP website

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- Definitions of CER
- Domestic Public Example - DERP
- Domestic Private example – Blue Cross Blue Shield Technology Evaluation Center

BCBS TEC

- Started by BCBS in 1985
- Each TEC Assessment is a comprehensive evaluation of the clinical effectiveness and appropriateness of a given medical procedure, device or drug
- Averaging 20 to 25 assessments a year, TEC provides healthcare decision makers with timely, rigorous and credible information on clinical effectiveness

Source: BCBS

TEC Structure

- Core staff of research scientists
- Multidisciplinary support staff
- Medical Advisory Panel

TEC Criteria to Assess Whether a Technology improves health outcomes

1. The technology must have final approval from the appropriate governmental regulatory bodies.
2. The scientific evidence must permit conclusions concerning the effect of the technology on health outcomes.
3. The technology must improve the net health outcome.
4. The technology must be as beneficial as any established alternatives.
5. The improvement must be attainable outside the investigational settings.

TEC Collaboration and Reports

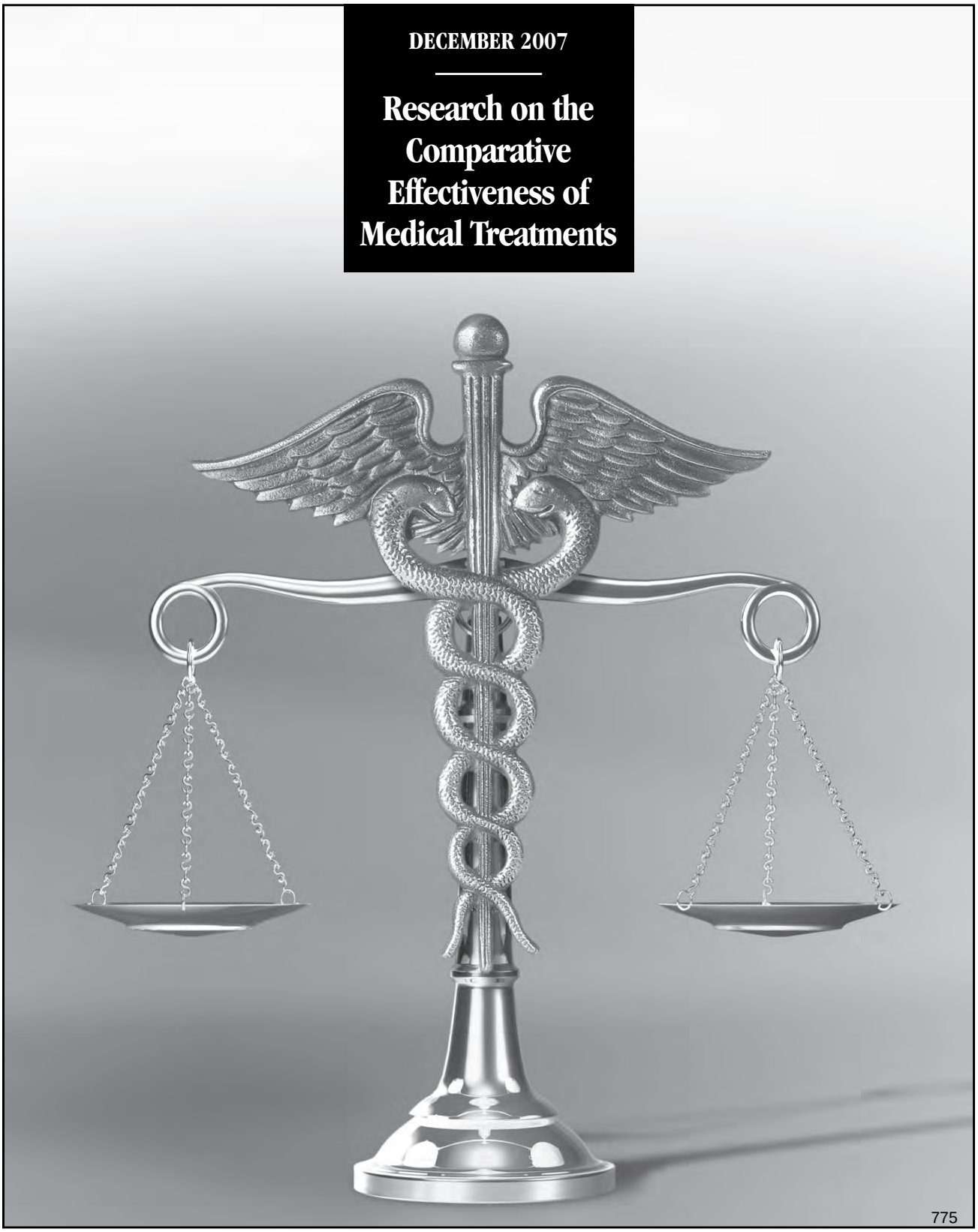
- TEC is an AHRQ Evidence-Based Practice Center
- Collaboration with Kaiser
- TEC assessments at: http://www.bcbs.com/blueresources/tec/tec-assessments.html

CONGRESS OF THE UNITED STATES
CONGRESSIONAL BUDGET OFFICE

A
CBO
PAPER

DECEMBER 2007

Research on the Comparative Effectiveness of Medical Treatments



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CBO

PAPER

Research on the Comparative Effectiveness of Medical Treatments: Issues and Options for an Expanded Federal Role

December 2007

The Congress of the United States ■ Congressional Budget Office

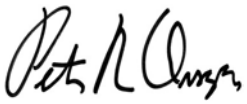
Preface

Rising costs for health care represent a central challenge both for the federal government and the private sector, but opportunities may exist to constrain costs in both sectors without adverse health consequences. Perhaps the most compelling evidence of those opportunities involves the significant geographic differences in spending on health care within the United States, which do not, on average, translate into higher life expectancy or substantial improvements in other health statistics in the higher-spending regions. At the same time, only a limited amount of evidence is available about which treatments work best for which patients and whether the added benefits of more-effective but more-expensive services are sufficient to warrant their added costs. Together, those findings suggest that generating better information about the costs and benefits of different treatment options—through research on the comparative effectiveness of those options—could help reduce health care spending without adversely affecting health overall.

This Congressional Budget Office (CBO) paper—prepared at the request of the Chairmen of the Senate Budget and Finance Committees—examines options for expanding federal support for research on comparative effectiveness. It reviews the current state of such research in both the public and private sectors and discusses several mechanisms for organizing and funding additional research efforts. It also discusses the different types of research that could be pursued and their likely benefits and costs. Finally, it considers the potential effects that such research could have on health care spending and the difficult steps that public and private insurers would probably have to take to achieve substantial savings on the basis of that research—in particular, changing the financial incentives for doctors and patients to reflect that information. In accordance with CBO's mandate to provide objective, impartial analysis, this paper contains no recommendations.

Philip Ellis of CBO's Health and Human Resources Division prepared the paper, with valuable contributions from Colin Baker and Morgan Hanger. The analysis benefited from comments by Dr. Alan Garber, Henry J. Kaiser Professor of Medicine at Stanford University, and Dr. Sean Tunis of the Center for Medical Technology Policy. (The assistance of external reviewers implies no responsibility for the final product, which rests solely with CBO.)

John Skeen edited the paper, and Maureen Costantino prepared it for publication and designed the cover. Lenny Skutnik printed the initial copies, Linda Schimmel handled the print distribution, and Simone Thomas prepared the electronic version for CBO's Web site (www.cbo.gov).



Peter R. Orszag
Director

December 2007

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Research on the Comparative Effectiveness of Medical Treatments

Summary and Introduction

Over the past 30 years, federal spending on Medicare and Medicaid has roughly tripled as a share of gross domestic product (GDP), rising from about 1.3 percent in 1975 to about 4 percent in 2007. According to the Congressional Budget Office's (CBO's) projections, under current policies such spending will reach about 12 percent of GDP by 2050—but substantial uncertainty surrounds that estimate.[1] If costs per enrollee continued growing over the next four decades as quickly as they have grown over the past four—about 2.5 percentage points faster than per capita GDP—then federal spending on those programs would reach about 17 percent of the economy. If, instead, costs per enrollee did not exceed the growth of GDP, those federal costs would reach about 6 percent of GDP in 2050 solely because of demographic changes (see Figure 1). As those figures indicate, the rate at which health care costs grow relative to income is the most important determinant of the country's long-term fiscal balance; it exerts a significantly larger influence on the budget over the long term than other commonly cited factors, such as the aging of the population or the coming retirement of the baby-boom generation.[2]

Rising health care costs represent a challenge not only for the federal government but also for private payers. Indeed, trends in both sectors reflect many of the same underlying forces—including the development and spread of new and more-expensive medical technologies—so controlling those federal costs over the long term will be difficult without addressing the forces that are also causing private costs for health care to rise. Total health care spending, which consumed about 8 percent of the U.S. economy in 1975, currently accounts for about 16 percent of GDP, and that share is projected to reach nearly 20 percent by 2016. About half of overall health spending in the United States is now publicly financed, and half is privately financed.

A variety of evidence suggests that opportunities exist to constrain health care costs both in the public programs and in the rest of the health system without adverse health consequences. Perhaps the most compelling evidence of those opportunities involves the substantial geographic differences in spending on health care—both among countries and within the United States—which do not translate into higher life expectancy or measured improvements in other health statistics in the higher-spending regions. For example, Medicare's costs per beneficiary vary significantly among different regions of the country, but much of the variation cannot be explained by differences in the population, and the higher-spending regions perform no better on available measures of average health outcomes than the lower-spending regions do.

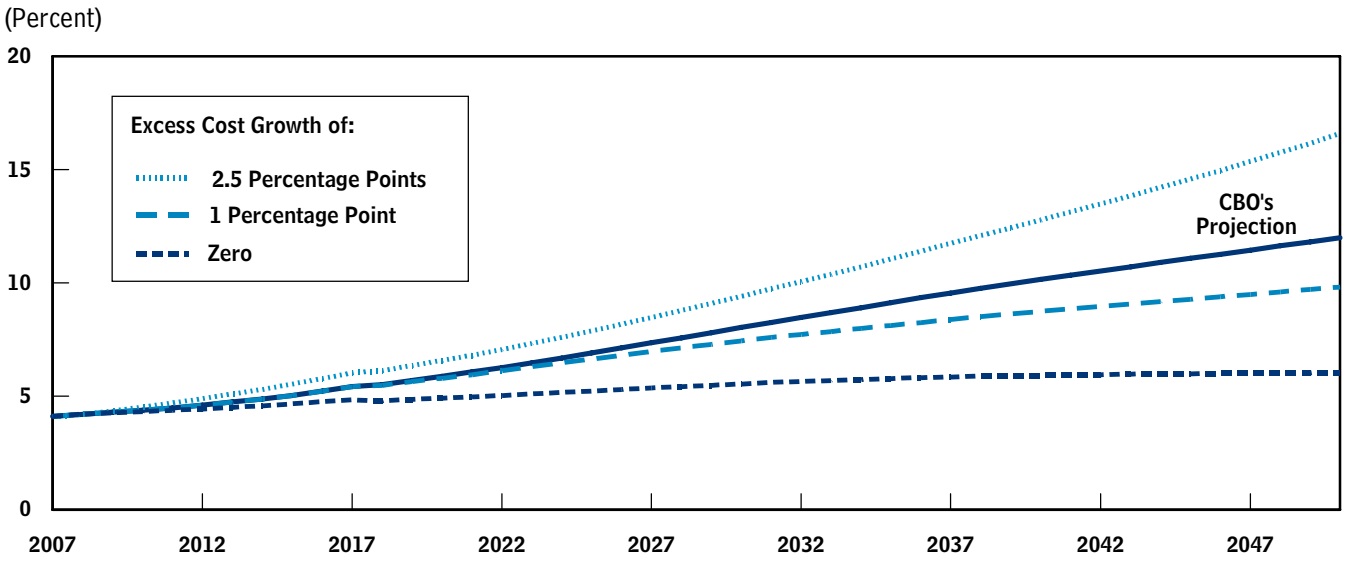
Furthermore, hard evidence is often unavailable about which treatments work best for which patients and whether the added benefits of more-effective but more-expensive services are sufficient to warrant their added costs—yet the current health system tends to adopt more-expensive treatments even in the absence of rigorous assessments of their impact. Indeed, the extent of the variation in treatments may be greatest when evidence about their relative effectiveness is lacking. Together, those findings suggest that better information about the costs, risks, and benefits of different treatment options,

1. Congressional Budget Office, *The Long-Term Outlook for Health Care Spending* (November 2007). The estimates of federal spending reflect Medicare's costs net of the premiums that enrollees pay and other offsetting receipts; the program's gross costs are about 15 percent higher than its net costs.

2. For additional discussion, see Congressional Budget Office, *The Long-Term Budget Outlook* (December 2007).

Figure 1.

Federal Spending for Medicare and Medicaid as a Percentage of Gross Domestic Product Under Different Assumptions About Excess Cost Growth



Source: Congressional Budget Office.

Note: Excess cost growth refers to the number of percentage points by which the growth of annual health care spending per beneficiary is assumed to exceed the growth of nominal gross domestic product per capita.

combined with new incentives reflecting the information, could eventually alter the way in which medicine is practiced and yield lower health care spending without having adverse effects on health. Over the long term, the potential reduction in spending below projected levels could be substantial.

Generating evidence that compares treatments is what research on "comparative effectiveness" does. This Congressional Budget Office (CBO) paper makes the following main points about the options that are available for an expanded federal role in supporting and organizing such research and about the impact that research could have on spending for health care:

- Because any private-sector entity (such as a health plan) has only a limited incentive to produce or pay for information that could benefit many entities—including its competitors—an argument can be made for a larger federal role in coordinating and funding research on comparative effectiveness. In addition, because federal health insurance programs play such a large role in financing medical care and account for such a large share of the budget, the federal government itself has an interest in generating evaluations of the effectiveness of different approaches to health care.

- If policymakers wanted to expand federal efforts to study comparative effectiveness, the endeavor could be organized in different ways—for instance, by augmenting an existing agency, by establishing a new agency, by supporting an existing quasi-governmental organization, or by creating a new public-private partnership. In choosing an organizational arrangement and a mechanism to provide federal funds to it, trade-offs could arise between the entity's independence from political pressure and its accountability to policymakers and other interested parties. Efforts to bolster comparative effectiveness research would be more likely to change medical practice if the organization coordinating the research was respected and trusted by doctors and other professionals in the health sector.

- The level of funding required for a new or augmented entity would depend largely on what its additional activities involved. Synthesizing existing studies or analyzing available data on medical claims would be less expensive than conducting new head-to-head clinical trials to compare treatments but could also yield

less definitive results—and therefore might have a smaller impact on medical practice. Clinical trials could be more persuasive but also more time-consuming, and there is probably a limit to how many comparative trials could be undertaken effectively at any given time. If privacy concerns could be addressed, having more health records available in electronic form would facilitate the use of such data for research.

- Studies might need to compare not only broadly different treatment options—such as surgery versus drug therapy—but also different approaches to the same basic treatment—such as different levels of follow-up care after surgery. Studies that included an analysis of cost-effectiveness would probably have a larger impact than ones that compared only clinical effectiveness, because they would highlight cases where more-expensive treatments or approaches provided added benefits that were modest compared with their added costs (at least for some types of patients).

- To affect medical treatment and reduce health care spending in a meaningful way, the results of comparative effectiveness analyses would not only have to be persuasive but also would have to be used in ways that changed the behavior of doctors, other health professionals, and patients. For example, the higher-value care identified by comparative effectiveness research could be promoted in the health system through financial incentives—the payments doctors receive or the cost sharing that patients face. Making substantial changes in payment policies or coverage rules under the Medicare program to reflect information on comparative effectiveness would almost certainly require legislation.

- Making such substantial changes in the delivery of health care could prove difficult and controversial for a number of reasons. To inform new systems of incentives—designed to discourage the use of more costly treatments that provided little or no added benefits—the results of effectiveness studies would have to be sufficiently robust to minimize the risk of overlooking subgroups of patients who could benefit greatly from a treatment. Even with an expanded evidence base, some patients and providers might object to the use of such incentives, and keeping pace with new treatments and procedures would be an ongoing challenge.

- Generating additional information about comparative effectiveness and making corresponding changes in incentives would seem likely to reduce health care spending over time—potentially to a significant degree. The precise impact, however, depends on several factors and is difficult to predict. Given the time necessary to conduct the research, to alter incentives in a manner reflecting the results, and to affect behavior through those changes, any potential for substantial cost savings from new research would probably take a decade or more to materialize. Even so, generating additional information comparing treatments would tend to reduce federal health spending somewhat in the near term—but that effect may not be large enough to offset the full costs of conducting the research over that same time period.

The Current State of Comparative Effectiveness Research

In weighing options to expand and reorganize research efforts, it is useful to define what comparative effectiveness research means and to consider the arguments for an expanded federal role in conducting such research. Related issues include the reasons why the current stock of research on comparative effectiveness is limited and why treatments and procedures can gain wide use even when evidence about their relative effectiveness is lacking. Reviewing past and current research efforts—by private and public organizations in the United States and by other countries—also sheds light on several issues and challenges likely to arise in any future U.S. efforts. To the extent that past and current efforts are seen as inadequate, careful consideration of those shortcomings would inform the choice of an organizational approach and funding mechanism for new federal activities.

What Is Comparative Effectiveness?

As applied in the health care sector, an analysis of comparative effectiveness is simply a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches, such as surgery and drug therapy. The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options. In some cases, a given treatment may prove to be more effective clinically or more cost-effective for a broad range of patients, but fre-

quently a key issue is determining which specific types of patients would benefit most from it. Related terms include cost–benefit analysis, technology assessment, and evidence-based medicine, although the latter concepts do not ordinarily take costs into account.

While some information about the effectiveness of new drugs, medical devices, and procedures is usually available, rigorous comparisons of different treatment options are less common. Drugs and devices must be certified as safe and effective by the Food and Drug Administration (FDA) before they can be marketed in the United States, but with certain exceptions the regulatory process for approving those products does not evaluate them relative to alternatives.[3] Furthermore, physicians commonly prescribe drugs for "off-label" uses—that is, for treatments that have not been certified by the FDA. For drug manufacturers, the costs of conducting additional trials to demonstrate safety and efficacy for a broader set of patients or conditions may outweigh the benefits from the increased sales that would result; in particular, the potential gains from finding a favorable result for a different population would have to be weighed against the risk that safety and efficacy could not be demonstrated conclusively.

Medical procedures, which account for a much larger share of total spending on health care than drugs and devices do, can achieve widespread use without extensive clinical evaluation. In many cases, it may be reasonable to assume that the benefits of a treatment will be similar for related conditions or a broader group of patients. Without hard evidence, however, decisions about what treatments to recommend often depend on the individual experience and judgment of physicians. Various reasons have been cited to explain why the use of new medical technologies can spread even in the absence of proof about their effectiveness and why health costs tend to increase as a result; those reasons include fee-for-service payment of physicians (common in the private sector and prevalent in Medicare, that payment method typically gives doctors a financial incentive to provide more-expensive care) as well as enthusiasm for the newest technology on the part of both doctors and patients.[4] Furthermore, patients with insurance typically pay only a small share of the costs of their treatments, so their incentives to weigh the costs against the benefits are limited—a trade-off inherent in having insurance protection.

A recent example of a comparative effectiveness study indicates that careful analysis can sometimes disprove widely held assumptions about the relative merits of different treatments. The study, which involved patients who had stable coronary artery disease, compared the effects of two treatments: an angioplasty with a metal stent combined with a drug regimen versus the drug regimen alone.[5] Patients were randomly assigned to receive the two treatments, and although the study found that patients treated with angioplasty and a stent had better blood flow and fewer symptoms of heart problems initially, the differences declined over time.[6] More importantly, it found no differences between the two groups in survival rates or the occurrence of heart attacks over a five-year period.

Other examples of studies comparing the clinical effectiveness of different treatment options illustrate the types of findings that they can generate:

3. Clinical trials of new drugs must compare them to alternative medications only when the manufacturer wants to make a claim of superiority in its FDA-approved marketing materials or when giving trial participants a placebo would be unethical (for example, in the case of a study of AIDS drugs).

4. See Mark R. Chassin, "Is Health Care Ready for Six Sigma Quality?" *The Milbank Quarterly,* vol. 76, no. 4 (November 1998), pp. 565–591.

5. Coronary artery disease, or a buildup of plaque in the heart's arteries, is considered stable if a patient experiences some chest pain (angina) but does not have worsening pain over time and has not had a heart attack. In an angioplasty, a small balloon is surgically inserted into a clogged artery and then inflated to expand the opening; a stent—a small wire mesh tube—is commonly added in an effort to keep the artery open.

6. William E. Boden and others, "Optimal Medical Therapy With or Without PCI for Stable Coronary Disease," *The New England Journal of Medicine,* vol. 356, no. 15 (April 12, 2007), pp.1503–1516. Other studies have found that angioplasty with a stent has clear medical benefits for patients who are undergoing a heart attack, illustrating the point that results for a given treatment may differ significantly among different types of patients.

- One recent trial found that older, relatively inexpensive drugs for treating high blood pressure (known as diuretics) were more effective in preventing cardiovascular disease in patients age 55 or older than commonly used newer drugs known as angiotensin-converting enzyme inhibitors and calcium channel blockers.[7]

- Another trial compared the effects of surgery to reduce lung volume for patients suffering from emphysema—a treatment that had anecdotal support but lacked hard evidence about its effectiveness—with standard medical therapy for that disease. For many patients, lung surgery increased their risk of death slightly and did not improve their functional status, but for patients with certain types of lung problems and a limited capacity for exercise, the surgery yielded small net improvements in their quality of life (though not in their survival rates).[8]

- A trial of two statin drugs, which was sponsored by the maker of one of those drugs, found that its competitor's product was more effective both at lowering cholesterol levels and at reducing the risk of mortality—illustrating the point that comparative trials can be risky for manufacturers to conduct.[9]

- Recent studies have found that magnetic resonance imaging combined with mammography is more effective than mammography alone in detecting breast cancer for women with certain genetic markers that indicate a substantial increased risk of contracting that disease; the impact of that difference on survival rates, however, could not be measured.[10]

The range of findings that those studies yielded highlights several characteristics of research on comparative effectiveness. First, studies can examine not only treatments for health problems but also different procedures to screen for the presence of a disease. Second, the findings may have broad applicability or may pertain only to a very specific subset of patients and may also vary in the outcomes considered—such as effects on mortality or other measures of health gains.

Third, studies are often based on clinical trials, in which eligible patients are randomly assigned to the treatments under review—but there are several other methods available to compare treatments, each with its own strengths and weaknesses. Clinical trials can yield persuasive findings but can also be relatively costly and time-consuming to conduct. In particular, a trial designed to determine whether two treatments differ in their effectiveness may require a large number of enrollees to be followed for an extended period in order to generate results that are statistically significant. Less expensive approaches include systematic reviews of the evidence about treatment options, which are essentially meta-analyses of all available studies, and studies that use medical claims data, which can be used to follow large groups of patients who have already received different treatments. The impact of systematic reviews can be limited, however, by the fact that they simply reflect existing evidence, and studies using claims data can be subject to bias because the treatments are not randomly assigned to comparable patients.

The studies cited above focus on relative clinical effects, and not cost-effectiveness. For reasons discussed below, gauging cost-effectiveness as well as clinical effectiveness is sometimes controversial, and some observers believe that the two considerations are in separate fields. But cost-effectiveness analysis appears to be well within the scope of research on comparative effectiveness—and

7. Officers and Coordinators for the ALLHAT (Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack Trial) Collaborative Research Group, "Major Outcomes in High-Risk Hypertensive Patients Randomized to Angiotensin-Converting Enzyme Inhibitor or Calcium Channel Blocker vs. Diuretic," *Journal of the American Medical Association*, vol. 288, no. 23 (December 18, 2002), pp. 2981–2997.

8. National Emphysema Treatment Trial Research Group, "A Randomized Trial Comparing Lung-Volume-Reduction Surgery with Medical Therapy for Severe Emphysema," *The New England Journal of Medicine*, vol. 348, no. 21 (May 22, 2003), pp. 2059–2073.

9. Christopher P. Cannon and others, "Intensive Versus Moderate Lipid Lowering with Statins After Acute Coronary Syndromes," *The New England Journal of Medicine*. vol. 350, no. 15 (April 8, 2004), pp. 1495–1504. Note that this study was undertaken in response to a similar one financed by the manufacturer of the other drug, which also showed that drug to be superior at lowering cholesterol levels but did not address mortality risks.

10. Ellen Warner and others, "Surveillance of BRCA1 and BRCA2 Mutation Carriers with Magnetic Resonance Imaging, Ultrasound, Mammography, and Clinical Breast Examination." *Journal of the American Medical Association,* vol. 292, no. 11 (September 15, 2004), pp. 1317–1325; and Mieke Kriege and others, "Efficacy of MRI and Mammography for Breast-Cancer Screening in Women with a Familial History or Genetic Predisposition," *The New England Journal of Medicine,* vol. 351, no. 5 (July 29, 2004), pp. 427–437.

Box 1.

Research on Comparative Effectiveness in Other Countries

Other developed countries also face challenges financing health care costs and have taken various steps to assess the comparative effectiveness of treatments. Unlike the United States, many of those countries establish overall budgets for their national health systems and regularly use the data on comparative effectiveness that are available to help determine the treatments and procedures to be covered and, in some cases, the payment rates. Despite differences in other countries' health insurance systems, the approaches that they have taken to organizing and funding those research and review activities could have lessons for any increased U.S. efforts.

Perhaps the best known example of an agency that assesses comparative effectiveness is the National Institute for Health and Clinical Excellence (NICE), which was established in 1999 as part of the United Kingdom's National Health Service (NHS). It analyzes both the clinical effectiveness and cost-effectiveness of new and existing medicines, procedures, and other technologies and provides guidance on appropriate treatments for specific diseases or types of patients. To date, NICE has published appraisals of over 100 specific technologies, guidance on the use of about 250 medical procedures, and about 60 sets of treatment guidelines—a substantial but not exhaustive list. If NICE approves a drug, device, or procedure, it must be covered by the NHS, but local health authorities make coverage decisions about treatments that NICE has not yet evaluated. With a staff of about 200 and an annual budget of about 30 million pounds (roughly $60 million), NICE does not fund new clinical trials or other forms of primary data collection. Instead, it commissions systematic reviews of existing research on clinical effectiveness and combines those findings with models of cost-effectiveness. Clinical trials are funded by the British Ministry of Health but (as in this country) data on total spending in the United Kingdom for research on comparative effectiveness are hard to come by.

Other countries such as Australia, Canada, France, and Germany have similar review processes, though the organizational and financing arrangements vary—and in several cases, the structures have recently been changed.[1] For example, France established a new agency in 2004 to bring together a number of related activities, including the evaluation of drugs, devices, and procedures, publication of clinical guidelines, accreditation of providers, and dissemination of medical information. Germany established a new agency in 2000 that conducts technology assessments and a new Institute for Quality and Efficiency

1. For additional information, see Institute of Medicine, *Learning What Works Best: The Nation's Need for Evidence on Comparative Effectiveness in Health Care* (September 2007), Appendix 2, available at www.iom.edu/ebm-effectiveness.

has been applied to many of to the treatments discussed above. For example, an additional analysis of lung-volume-reduction surgery, which focused on the patients likely to benefit from the surgery, found that it would be cost-effective if its benefits persisted for 10 years but might not be so if those benefits dissipated after three years.[11] (That study did not follow patients for a decade and therefore had to estimate the future benefits.) Similarly, another study examined the cost-effectiveness of more-expensive screening mechanisms for breast cancer and found that it varied substantially with the age of the patient.[12]

11. National Emphysema Treatment Trial Research Group, "Cost Effectiveness of Lung-Volume–Reduction Surgery for Patients with Severe Emphysema," *The New England Journal of Medicine*, vol. 348, no. 21 (May 22, 2003), pp. 2092–2102.

12. Sylvia K. Plevritis and others, "Cost-Effectiveness of Screening BRCA1/2 Mutation Carriers with Breast Magnetic Resonance Imaging," *Journal of the American Medical Association,* vol. 295, no. 20 (May 24/31, 2006), pp. 2374–2384.

Box 1.

Continued

in 2004 that evaluates health care services. Discussions about the use of comparative effectiveness in those countries sometimes focuses on their review processes for prescription drugs, but their efforts generally encompass all forms of acute medical care. (For all the attention they receive, drug costs represent less than 15 percent of health care spending in the United States—so research that focused only on medications would miss the vast majority of services and would not be able to compare drug therapy with surgical procedures or other interventions.)

Although those countries all have government-run health care systems, they have taken different approaches regarding the placement of and funding for their assessment bodies. In the United Kingdom and Australia, the agencies are part of the government's health departments; France and Canada have established independent not-for-profit organizations; and Germany has taken a mixed approach (the Institute for Quality and Efficiency is independent, but the technology assessment agency is an arm of the health ministry). Financing arrangements vary correspondingly: Funding in the United Kingdom and Australia comes from their health departments, whereas Germany's independent institute is funded by a levy on inpatient and outpatient health care services (which are mainly reimbursed by the country's regional health insurance funds), and the French agency gets its funding from a combination of taxes on promotional spending by drug companies, government subsidies, and accreditation fees. Health ministries in Australia, Canada, France, and Germany also help fund clinical trials and other forms of primary research, but total spending related to comparative effectiveness in those countries is also difficult to estimate.

Given the interest that has developed in many countries, it is not surprising that several international organizations have become involved in comparative effectiveness research. The best known may be the Cochrane Collaboration—a nonprofit organization that has a network of volunteers who conduct systematic reviews of treatments. Many of its activities are organized through centers located around the world, including one in the United States. Founded in 1993, the Cochrane Collaboration maintains an accessible database that now contains more than 4,500 reviews; its limited funding comes primarily from subscription fees for its quarterly journal. Any new or expanded U.S. entity that would organize and fund research on comparative effectiveness would probably draw upon Cochrane's findings and the results of research conducted in other countries (to the extent such research was applicable to U.S. patients).

More generally, the relative cost-effectiveness of treatment options is clear when a less expensive treatment yields comparable or superior health gains. In other cases, however, determining whether the additional medical benefits of a more expensive treatment warrant their added costs is complex. Typically, the benefits of different treatments are summarized as an increase in life expectancy or, more commonly, as an increase in quality-adjusted life years (QALYs) to account for effects on morbidity as well as mortality. That calculation reflects estimates of how much people value improving their health or avoiding various side effects, which are combined to create a single metric. By convention, cost-effectiveness analyses report results as the cost per QALY gained, so a lower dollar amount indicates a more cost-effective service. If that metric is used to determine whether specific health procedures are covered by an insurance program, choosing a cost-effectiveness threshold can be a controversial endeavor—but that need not be the manner in which such research is applied.

Research in the Private Sector

In the United States, most of the formal research that is done to examine the effects of drugs or medical devices is

conducted by the manufacturers of those products in the course of their development; as noted, however, it is the exception rather than the rule that those studies directly compare treatments or products.[13] Nevertheless, various other private organizations have also produced assessments and comparisons of some treatments. (Analyses conducted in other countries represent another source of information about treatments; see Box 1 on page 6.)

Several private-sector organizations exist primarily or exclusively to assess medical treatments and technologies. One prominent example is the Technology Evaluation Center that is part of the Blue Cross Blue Shield Association. Its analyses are based on systematic reviews of the available literature and therefore rely on clinical trials or other studies that have already been conducted. (In such reviews, more weight is given to studies that are judged to be of higher methodological quality.) The center produces about 20 to 25 new assessments of drugs, devices, and other technologies each year; the analyses consider clinical effectiveness but generally do not assess cost-effectiveness.

For-profit private-sector firms that specialize in technology assessments represent another source of analysis. Hayes, Inc., is one of the larger firms in the field. Such firms also conduct systematic reviews and evaluate medical and surgical procedures, drugs, and devices in return for a fee or on a subscription basis. Organizations that are similar but operate as nonprofit entities—sometimes affiliated with academic or medical centers—include the ECRI Institute and the Tufts-New England Medical Center's Cost-Effectiveness Analysis Registry (which provides an extensive list of the cost-effectiveness ratios that are available from published studies).

In addition, private health plans—most commonly, larger or more integrated ones—conduct their own reviews of evidence and sometimes undertake new analyses of comparative effectiveness using claims data for their enrollees.[14] Health plans may choose to publicize the results, or they may decide to keep their findings confidential and use them to shape their policies regarding coverage of and payment for the treatments in question. For example, health plans usually have an entity known as a pharmacy and therapeutic committee that considers the evidence regarding the relative effectiveness of different prescription drugs and makes recommendations about which ones should be covered (that is, included on formularies) or given preferred status. An example of a more public and collaborative effort is the HMO Research Network, a consortium of more than a dozen health maintenance organizations from different parts of the country; started in the mid-1990s, it brings together researchers to share findings and, in some cases, uses data from several plans as the basis for analysis.[15]

Notwithstanding those current efforts, the private sector generally will not produce as much research on comparative effectiveness as society would value. The knowledge created by such studies is costly to produce—but once it is produced, it can be disseminated at essentially no additional cost, and charging all users for access to that information is not always feasible. As a result, private insurers and other entities conducting research on comparative effectiveness often stand to capture only a portion of the resulting benefits and therefore do not invest as much in such research as they would if they took into account the benefits to all parties. In health plans that do not have exclusive provider networks, some of the benefits probably "spill over" to other health plans using the same doctors, because physicians tend to use a similar approach to care for all of their patients. Even if organizations could keep their findings confidential, so that they captured all of the benefits, some duplication of effort would probably occur. In such a situation, research constitutes a "public good," and economists have long recognized a role for government to increase the supply of such research toward the socially optimal level.

Another reason for the limited availability of information on comparative effectiveness is that public-sector health

13. In the limited number of instances in which manufacturers sponsor head-to-head trials, the comparisons tend to focus on the relative merits of products used to provide the same basic treatment. For example, a number of industry-sponsored trials have been conducted comparing different brands of coronary stents that are used during an angioplasty.

14. Although the Technology Evaluation Center discussed above is affiliated with the Blue Cross Blue Shield Association (which is an umbrella group that represents the Blue Cross and Blue Shield insurers from each state), that center does not work directly or exclusively for those insurers.

15. Medical specialty societies, such as the American Heart Association, represent another source of analysis of different treatment options—which typically take the form of treatment guidelines for various types of conditions and patients.

insurance programs—which collectively account for about 40 percent of all health care spending—have not sought to make extensive use of it. In particular, the Medicare program has made only limited use of comparative effectiveness data in making decisions about which treatments to cover and how much to pay for them. It stands to reason that the limited demand for such research from such a prominent payer has constrained the supply correspondingly. Conversely, increasing the amount of credible and objective research that was available could facilitate moving Medicare toward what former program administrator Mark McClellan has called a "fee-for-value" system rather than a fee-for-service one. (Options to incorporate research findings into Medicare's coverage and payment policies, along with the issues they raise, are discussed in the final section.)

Past and Current Federal Efforts

In the United States, the federal government has a rather long but somewhat checkered history of involvement in comparative effectiveness research and related efforts. Federal efforts date at least to the late 1970s and the short-lived National Center for Health Care Technology. Established in 1978 as part of the Department of Health, Education, and Welfare, it was given a broad mandate to conduct and promote research on health care technology, and it included an advisory board appointed by the Secretary to assist in setting research priorities. The center sponsored or cosponsored major evaluations of coronary artery bypass graft surgery, dental radiology, and cesarean delivery and made about 75 recommendations to the Medicare program about coverage. The center ceased operations at the end of 1981, however, reflecting changes in priorities for the new Administration and the Congress as well as opposition from some provider and industry groups.[16]

In that same period, the Office of Technology Assessment (OTA) was created as an advisory agency to the Congress, covering a broad set of issues, including health care. Given the agency's focus on evaluating technologies, much of its work would now be called research on comparative effectiveness; over the years, it studied a variety of health care topics, including the costs and benefits of screening tests for several diseases. OTA also produced an extensive review and analysis of the issues involved in and options for improving evidence about the clinical effectiveness and cost-effectiveness of medical treatments.[17] For a variety of reasons, however—having little to do with its health care studies specifically but instead reflecting broader questions about the agency's role—OTA was eliminated in 1995.

More recently, the Agency for Health Care Research and Quality (AHRQ) has been the most prominent federal agency supporting various types of research on the comparative effectiveness of medical treatments. Established in 1989 as the Agency for Health Care Policy and Research, AHRQ is an arm of the Department of Health and Human Services (HHS).[18] It currently has a staff of about 300 and an annual budget of over $300 million, which primarily funds research grants to and contracts with universities and other research organizations covering a wide range of topics in health services.

AHRQ has undertaken a number of initiatives related to comparative effectiveness. One such step—initially taken in collaboration with the American Medical Association and America's Health Insurance Plans, a coalition of insurance companies—has been the creation of a national clearinghouse for treatment guidelines, which are designed to summarize the available medical evidence on the appropriate treatments for various conditions. AHRQ has also endorsed about a dozen evidence-based practice centers around the country. Generally affiliated with a university, those centers analyze and synthesize existing evidence about treatments and technologies. Although many studies sponsored by AHRQ have examined only the relative clinical benefits of different treatments, some have also analyzed their cost-effectiveness. Research on comparative effectiveness has accounted for only a modest portion of AHRQ's budget, though.

As with other agencies examining the effectiveness of medical treatments or evaluating medical technologies, support for AHRQ has varied over time. In the mid-1990s, controversies arose after an agency-sponsored research team concluded that there was insufficient

16. See Seymour Perry, "The Brief Life of the National Center for Health Care Technology," *The New England Journal of Medicine*, vol. 307, no. 17 (October 21, 1982), pp. 1095–1100.

17. See Office of Technology Assessment, *Identifying Health Technologies that Work: Searching for Evidence,* OTA-H-608 (September 1994).

18. Prior to AHRQ's establishment as a separate agency, some of its functions were carried out by the National Center for Health Services Research within HHS.

Table 1.

Requested, Proposed, and Actual Funding for the Agency for Health Care Research and Quality

(Millions of dollars)

	Agency's Request	House Proposal	Senate Proposal	Appropriation
1991	109	88	138	115
1992	122	115	127	120
1993	125	118	130	128
1994	158	148	158	154
1995	171	154	166	162
1996	194	66	127	125
1997	144	125	144	143
1998	149	149	143	147
1999	171	171	171	171
2000	206	175	211	204
2001	250	224	270	270
2002	306	306	291	299
2003	250	0	314	304
2004	279	304	304	304
2005	304	304	319	319
2006	319	319	324	319

Source: Congressional Budget Office based on data from the Department of Health and Human Services, Agency for Health Care Research and Quality.

evidence to support certain spinal surgeries and, on the basis of that work, the agency issued practice guidelines for the treatment of back pain.[19] Strong opposition from back surgeons, along with broader questions about the value of the research that the agency had funded and other factors, led to proposals to eliminate the agency. Ultimately, the agency was retained, but its funding for fiscal year 1996 was reduced from prior levels (see Table 1). Since then, its overall budget has generally been maintained, at least in nominal terms, or increased. Again in 2002, however, the House of Representatives voted to cut off all funding for AHRQ, though in the end the agency received a small increase in its fiscal year 2003 appropriation.

Most recently, section 1013 of the Medicare Modernization Act of 2003 authorized AHRQ to spend up to $50 million in 2004 and additional amounts in future years to conduct and support research with a focus on "outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs)" for Medicare and Medicaid enrollees. The actual funding appropriated for that initiative has been $15 million per year. Using that funding, AHRQ has established an "Effective Health Care" program consisting of three main functions: reviewing and synthesizing existing evidence (using its evidence-based practice centers); generating new information using a set of approved research centers (such as the HMO Research Network) that have access to data from medical claims and electronic medical records; and publishing findings in formats that are geared to the differing needs of clinicians, patients, and policymakers.

Other federal agencies also engage in various activities related to comparative effectiveness research—efforts that receive less attention than AHRQ's activities but that are probably larger in dollar terms. The Department of Veterans Affairs (VA) has a very substantial research program that reviews evidence from the medical records of its patients, focusing particularly on the clinical effectiveness of treatments. The department also sponsors evidence reviews through a technology assessment program and helps fund clinical trials—including the study comparing

19. For a discussion, see Bradford H. Gray, Michael K. Gusmano, and Sara R. Collins, "AHCPR and the Changing Politics of Health Services Research," *Health Affairs,* Web Exclusive (June 25, 2003), pp. W3-283–W3-307.

stents to drug therapy mentioned above. Indeed, over the past 30 years, some of the most influential clinical trials have been supported by and conducted in the VA health system, including the first major trials that demonstrated the value of bypass surgery over medical therapy for some forms of coronary artery disease as well as head-to-head studies of drugs that treat prostate enlargement. Another source is the National Institutes of Health (NIH), part of HHS, which is the leading federal sponsor of medical research—primarily in the form of clinical trials. Although comparative effectiveness is not a focus of that research, over the years NIH has sponsored a number of trials that compare treatments directly.

The Centers for Medicare and Medicaid Services (CMS) has helped to sponsor a limited amount of research on comparative effectiveness (for example, it covered the medical costs of the study of lung-volume-reduction surgery). When making decisions about what services are covered, however, CMS generally considers only whether devices and procedures are clinically effective. It has sponsored some studies comparing the effectiveness of different treatments but has done so largely to determine whether to establish separate payment rates for similar treatments. For example, CMS is currently cosponsoring a trial with NIH that may eventually compare the effects of daily dialysis for kidney patients with the conventional treatment of dialysis three times per week.[20] If daily dialysis proves more effective for certain patients, CMS could modify its payment policy to cover the additional costs of more frequent treatment for those patients.

Estimating the total amount that is spent in the United States each year on research that compares the effectiveness of medical treatments is difficult. According to one recent analysis, the federal government spent about $1.5 billion in 2005 on all health services research, a broader category that includes some of the work on comparative effectiveness but also encompasses many other types of studies.[21] For example, that total included AHRQ's entire budget of roughly $300 million, whereas the funding devoted to the agency's effective health care program has been $15 million per year. At the same time, that aggregate figure may not include all federal funding for comparative trials or other efforts that are outside the traditional scope of health services research.

Estimating private expenditures is even more challenging. Although drug and device manufacturers spend billions of dollars each year on clinical trials aimed at demonstrating the safety and efficacy of new products, the vast majority of those efforts contribute to comparisons of treatments only indirectly. Data are simply not available on how much is spent by private organizations such a health plans, medical specialty societies, and technology assessment centers to compare medical treatments and procedures. Nevertheless, one recent study estimated that less that $2 billion is spent annually on comparative effectiveness research in this country—and even that rough estimate is subject to uncertainty.[22]

The Consequences of Limited Information

Whether the cause is limited supply or limited demand, the relative scarcity of rigorous data about comparative effectiveness has several effects. First and foremost, it means that decisions about what treatments to use often depend on anecdotal evidence, conjecture, and the experience and judgment of the individual physicians involved. In many cases, that basis may be sufficient; as some observers have noted, it is not necessary to conduct a randomized trial to determine whether to use a parachute when jumping out of an airplane. But if the benefits of a treatment—or risks of not providing it—are less obvious, the lack of hard data makes determining the appropriate choice of treatment difficult. Although estimates vary, some experts believe that less than half of all medical care is based on or supported by adequate evidence about its effectiveness.[23]

Evidence about treatments' effectiveness remains limited even though the number of rigorous studies has grown substantially in recent decades. To illustrate that point, one study simply examined the number of articles that were published each year in peer-reviewed medical journals that reported results from randomized trials.[24]

20. Initially, the study sought to test the feasibility of randomly assigning conventional or daily dialysis to a representative sample of patients.

21. AcademyHealth, *Placement, Coordination, and Funding of Health Services Research within the Federal Government* (September 2005), available at www.academyhealth.org/publications/placementreport.pdf.

22. See Institute of Medicine, *Learning What Works Best: The Nation's Need for Evidence on Comparative Effectiveness in Health Care* (September 2007), p. 8, available at www.iom.edu/ebm-effectiveness.

23. Institute of Medicine, *Learning What Works Best*, p. 2.

24. Mark R. Chassin, "Is Health Care Ready for Six Sigma Quality?"

Between 1966 and 1995, that number increased dramatically, from about 100 to nearly 10,000—with about half of the cumulative total over that period having been produced between 1990 and 1995. But even if the proportion of treatments based on hard evidence has increased as a result, the share remains relatively low. Furthermore, having the evidence base keep pace with the rapid development of new medical treatments and technologies will remain an ongoing challenge.

Another important effect of limited evidence—indeed, an indicator of that scarcity—is that the use of certain treatments and the types of care provided vary widely from one area of the country to another. For example, even after adjusting for differences in the age, sex, and race of Medicare enrollees, researchers at Dartmouth found about a fourfold variation in the share receiving a coronary artery bypass graft; and those differences were not correlated with rates of heart attacks in each region.[25] At the same time, those researchers found that overall surgery rates did not vary systematically; areas with above-average rates for certain procedures had below-average rates for others. Those differences in the use of treatments reflect at least in part the local practice norms that have arisen in each area, and the apparent variation in those norms indicates that there is not sufficient evidence to determine which approach is most appropriate.

Geographic differences in the types of care provided can remain substantial even among patients who turn out to be in their last six months of life. (Examining that period is an analytic approach that can be used in an effort to control for differences in the prevalence and severity of diseases patients have, on the grounds that large groups of patients who are nearing death are likely to have comparable health problems regardless of where they live.) For example, such patients spend nearly 20 days in the hospital over those last six months, on average, in the highest-use areas, compared with an average of about six hospital days in the lowest-use areas. Similarly, the average number of visits to physicians in that period is as high as 50 in some of the highest-use regions and as low as 16 in some of the lowest-use regions.[26]

The observed variations in the use of services correspond to substantial differences in Medicare spending per enrollee in different parts of the country (see Figure 2). In 2003, average costs ranged from about $4,500 in the areas with the lowest spending to nearly $12,000 in the areas with the highest spending (those averages were adjusted to account for differences in the age, sex, and race of Medicare beneficiaries in the various areas). Some of those differences in spending reflect varying rates of illness as well as differences in the prices that Medicare pays for the same service, which are adjusted on the basis of local costs for labor and equipment in the health sector. But according to the Dartmouth researchers, differences in illness rates account for less than 30 percent of the variation in spending among areas, and differences in prices can explain another 10 percent—indicating that more than 60 percent of the variation is due to other factors.[27] Other studies have found that a larger share of the variation in spending can be accounted for by differences in health status and demographic factors, but even so, the remaining differences are substantial in dollar terms.[28]

Of particular relevance to the issue of comparative effectiveness, there is some evidence that the degree of geographic variation in treatment patterns is greater when less consensus exists within the medical community about the best treatment to use. For example, patients who have fractured their hip need to be hospitalized, and there is relatively little variation in admission rates for Medicare beneficiaries with that diagnosis—but for hip replacements and for knee replacements, more discretion is involved and the surgery rates vary more widely

25. See John E. Wennberg, Elliott S. Fisher, and Jonathan S. Skinner, "Geography and the Debate Over Medicare Reform," *Health Affairs,* Web Exclusive (February 13, 2002), pp. w96–w97. The analysis divided the country into about 300 "hospital referral regions," which reflect where Medicare beneficiaries typically receive hospital care. In 2003, bypass surgery rates ranged from about 2 to 3 per 1,000 Medicare beneficiaries in the lowest-use regions to about 9 to 10 per 1,000 in the highest-use regions. Although higher rates of bypass surgery could reflect higher rates of heart attacks, higher surgery rates could also prevent some heart attacks—a factor that could help explain the lack of correlation between those two measures.

26. Based on data from 2000 to 2003, available from www.dartmouthatlas.org.

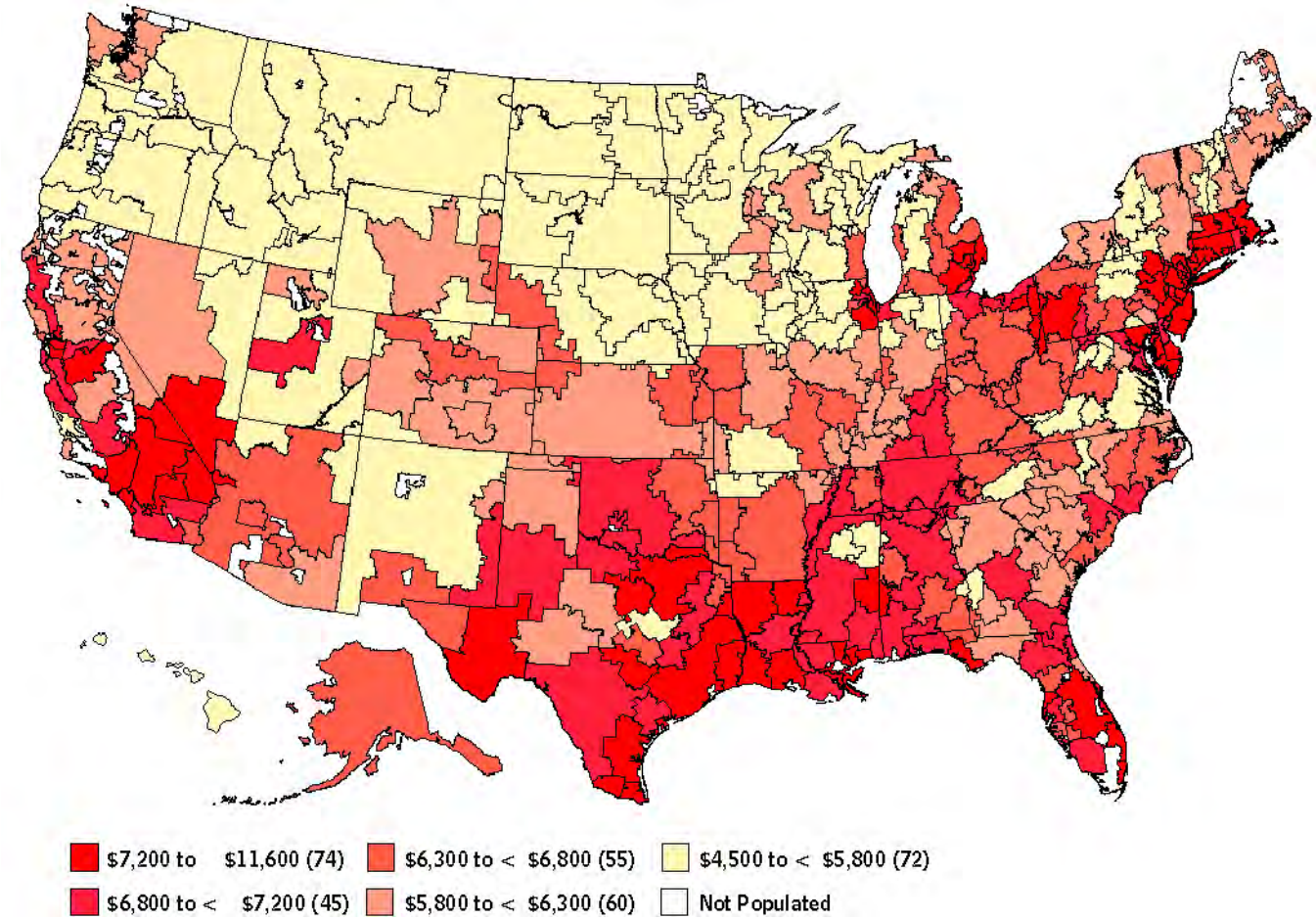
27. John E. Wennberg, Elliot S. Fisher, and Jonathan S. Skinner, "Geography and the Debate Over Medicare Reform"; and The Center for the Evaluative Clinical Sciences, Dartmouth Medical School, *The Dartmouth Atlas of Health Care 1999* (Dartmouth, N.H.: Health Forum, Inc., 1999), pp. 22–23.

28. David Cutler and Louise Sheiner, "The Geography of Medicare," *American Economic Review,* vol. 89, no. 2 (May 1999), pp. 228–233.

Figure 2.

Medicare Spending per Capita in the United States, by Hospital Referral Region, 2003

(Percent)



Source: *The Dartmouth Atlas of Health Care.*

Note: Numbers in parentheses refer to the number of hospital referral regions with per capita spending in each interval.

(see Figure 3). And there appears to be even more variation in the rates of back surgery—a treatment whose benefits have been the subject of substantial questions. Determining what share of any geographic variation in the use of procedures is due to differences in the treatments that doctors recommend and what share is due to differences in underlying illness rates is challenging, however, so the comparison of procedures may be sensitive to the manner in which the differences in illness rates are estimated.[29]

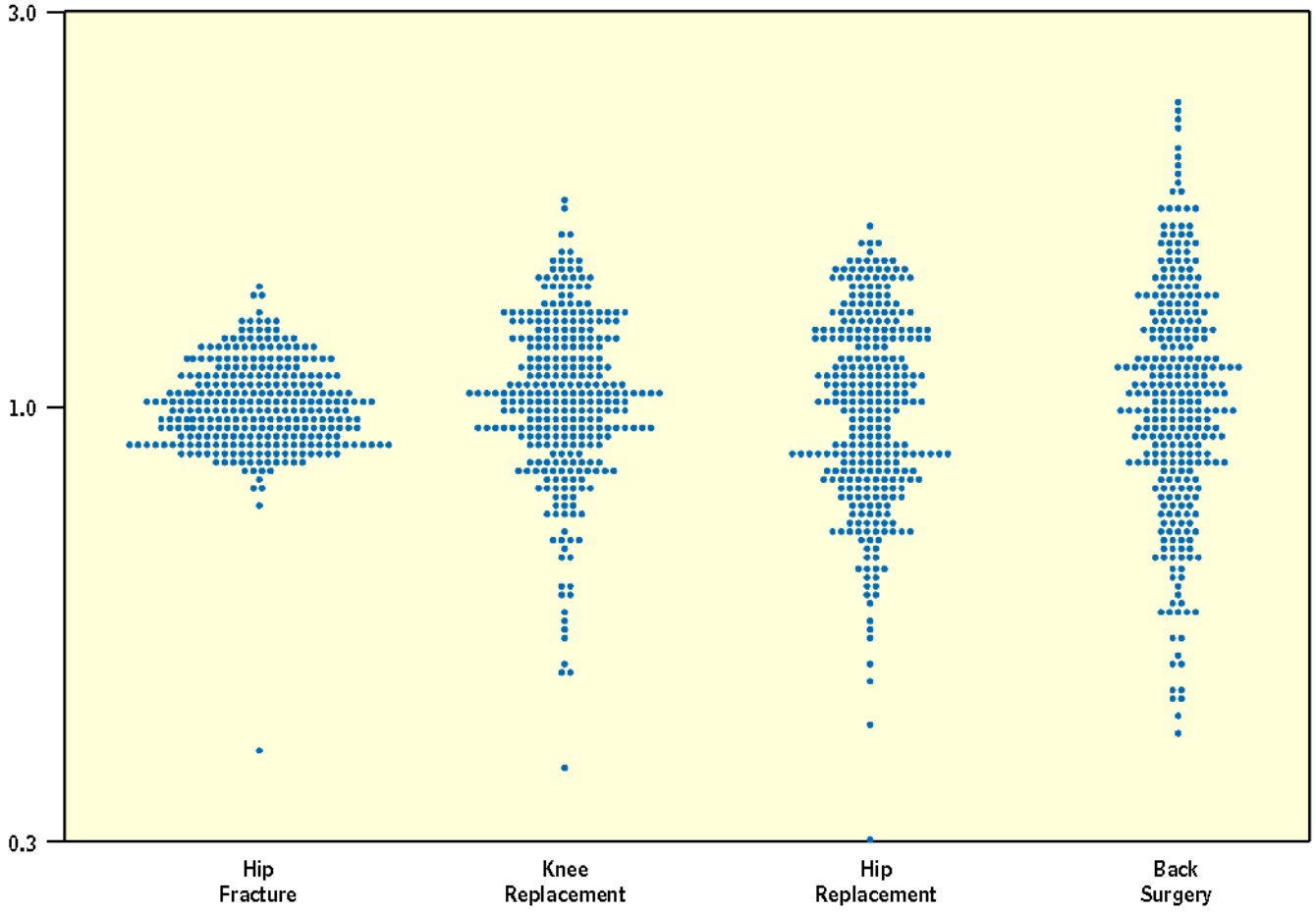
The implications of the observed variations in treatments and spending depend importantly on their relationship to health outcomes. If life expectancy and other measures were better in the areas with higher spending, that result would imply that increased spending in the low-cost areas would yield health benefits. One recent and well-

29. The data used in Figure 3 were adjusted to account for differences in illness rates among areas using data on five conditions, one of which was hip fracture. In the unadjusted data, the variation in knee and hip replacements is somewhat larger than the variation in hip fracture surgery—and variation in back surgery rates is larger still—but the differences are not as substantial. Whether the adjusted results were affected by including hip fracture rates both as an adjustment factor and in the comparison of procedures is not clear. Whether the prevalence of other diseases is correlated with the prevalence of those five conditions is also uncertain.

Figure 3.

Rates of Four Orthopedic Procedures Among Medicare Enrollees, 2002 and 2003

(Standardized discharge ratio, log scale)



Source: Dartmouth Atlas Project, *The Dartmouth Atlas of Health Care*.

Notes: In the figure, each point represents a hospital referral region; the country was divided into about 300 such regions on the basis of where Medicare enrollees typically receive their hospital care.

The points indicate how the rate at which the procedure is performed (per 1,000 Medicare enrollees) in each referral region compares with the national average rate (which has been normalized to 1.0). Differences in procedure rates were adjusted to account for differences among regions in the age, sex, and race of enrollees and for measures of illness rates.

designed study examined differences in hospital spending in Florida and found that areas with higher spending had lower mortality rates among Medicare patients who were treated in the emergency room for a heart attack.[30] Using data on Medicare enrollees nationwide, however, another study found that higher-spending regions did not, on average, have lower mortality rates than the lower-spending regions, even after adjustments to control for differing illness rates among patients and regions.[31] That study also found that higher spending did not slow the rate at which the elderly developed functional limitations (reflecting their ability to take care of themselves). Although more research is needed about the impact that differences in spending have on patients' morbidity and quality of life, perhaps using more-extensive measures of health outcomes, those findings suggest that spending in the high-cost areas could be reduced without adverse effects on the overall health of residents in those areas.

How much could spending be reduced? Some estimates of the potential savings from reducing the variations in treatments are quite large, although questions remain about what mechanisms could achieve those savings and what the effects on health would be. The Dartmouth researchers have suggested that Medicare spending—and perhaps all health spending in the country—could be cut by about 30 percent if the more conservative practice styles used in the lowest-spending one-fifth of the country could be adopted nationwide.[32] While they note the need for more research about the specific steps needed to reduce spending levels without harming health, their analysis indicates that the added spending is not contributing to better health outcomes. Other studies suggest that overall health might not suffer in the process of changing practice patterns but that patients who would benefit most from more-expensive treatments might be made worse off as a result, while patients who would do better with treatments that were less expensive would gain.[33]

Other studies of geographic variation indicate that there may be room to reduce spending without harming health in both high-use and low-use areas of the country. One older study, for example, had independent panels of doctors conduct after-the-fact reviews of the medical charts of Medicare enrollees who had had certain surgeries.[34] In areas with high use of the procedures, the study found that the share of surgeries that was clinically appropriate ranged from about 35 percent to about 70 percent; the remainder were either clinically inappropriate or of equivocal value. In low-use areas, the share considered appropriate ranged from about 40 percent to about 80 percent. In other words, the share of procedures deemed appropriate was slightly higher in the low-use areas, but that share was well below 100 percent in both high-use and low-use areas.

Options for Organizing and Funding New Federal Research Efforts

The approach that is taken for organizing and funding any increased federal efforts to support research on comparative effectiveness could play an important role in determining their impact. Some approaches would seek to insulate those efforts from political pressure by setting up an organization at "arm's length" from the government and by providing a dedicated source of financing. Many of the options that have been proposed seek to coordinate and centralize existing activities through one entity—which would tend to give any conclusions it reached more weight—but developing several competing sources of information about comparative effectiveness could also have value.

30. Joseph J. Doyle, Jr., "Returns to Local-Area Health Care Spending: Using Health Shocks to Patients Far From Home," NBER Working Paper 13301 (National Bureau of Economic Research, August 2007).

31. Elliott S. Fisher and others, "The Implications of Regional Variations in Medicare Spending, Part 2: Health Outcomes and Satisfaction with Care," *Annals of Internal Medicine*, vol. 138, no. 4 (February 18, 2003), pp. 288–298.

32. Elliott Fisher, "More Care is Not Better Care," *Expert Voices*, Issue 7 (National Institute for Health Care Management, January 2005), available at www.nihcm.org/publications/expert_voices.

33. Amitabh Chandra and Douglas O. Staiger, "Productivity Spillovers in Health Care: Evidence from the Treatment of Heart Attacks," *Journal of Political Economy,* vol. 115, no. 1 (February 2007), pp. 103–140.

34. Mark R. Chassin and others, "Does Inappropriate Use Explain Geographic Variations in the Use of Health Care Services? A Study of Three Procedures," *Journal of the American Medical Association,* vol. 258, no. 18 (November 13, 1987), pp. 2533–2537. The procedures studied were coronary angiography (which generally involves inserting a tube and special dyes into the heart to see how well blood flows through it), carotid endarterectomy (in which plaque is removed from the main artery that goes to the brain), and gastrointestinal endoscopy (in which a flexible tube with a small camera mounted on it is inserted into the intestines).

Specific options that have been put forward for organizing federal research on comparative effectiveness include the following (each of which could have many variants):[35]

- Expanding the role of an existing agency that already conducts or oversees research on health services generally—and comparative effectiveness specifically—such as AHRQ or NIH.

- Creating or "spinning off" a new agency, either within the Department of Health and Human Services or as an independent body that is part of either the executive or the legislative branch. The Federal Trade Commission and the Medicare Payment Advisory Commission (MedPAC) are potential models for such an option.

- Augmenting an existing quasi-governmental organization, such as the Institute of Medicine or the National Research Council. Such entities are often Congressionally chartered, but they are not subject to regular governmental oversight.[36] Even so, the Institute of Medicine receives most of its funding from government agencies, which is provided to finance specific studies that have been requested.

- Establishing a new public–private partnership to oversee and direct research. That option could be structured in various ways, but one such approach would be to set up a federally funded research and development center (FFRDC). FFRDCs are not-for-profit organizations that can accept some private payments but that get most of their funding from a federal agency that provides oversight and monitoring.

Regardless of the type of organization, several potential mechanisms (either individually or in combination) could be used to fund research on comparative effectiveness. Federal spending could be authorized and appropriated annually, as with other discretionary programs. Alternatively, funding could be drawn from Medicare's Hospital Insurance trust fund (which is financed primarily by payroll taxes) or specified as a percentage of mandatory federal outlays on health insurance programs.[37] Instead of or in addition to using existing sources of revenues, another option would be to require direct contributions from the health sector. For example, a new tax on health insurance premiums or other payments within the health sector could be established, with the resulting revenues dedicated to research on comparative effectiveness.

Trade-offs might arise between an entity's independence, credibility with the medical profession, and ability to reach controversial conclusions, on the one hand, and its accountability and responsiveness to policymakers and to other interested parties, on the other. For example, funding through appropriations would allow lawmakers to assess the new entity's contributions and accomplishments and to balance spending on those efforts against other federal priorities on an annual basis. But some observers have raised concerns that relying on annual appropriations would leave a new entity vulnerable to outside pressure and thus reluctant to undertake controversial studies or to reach conclusions that might generate opposition from affected groups. Indeed, the elimination of agencies engaged in such research that were funded by annual appropriations—or in the case of AHRQ, the occasional threat of elimination or substantial cuts in funding—may suggest the need for a different arrangement.

Alternatively, housing the new activities in an organization that was separate from the federal government and establishing automatic or dedicated funding mechanisms would give a new entity greater autonomy and potentially more influence on doctors and other health professionals. To be sure, lawmakers could change any funding formula that had been established—as is done frequently in Medicare—mitigating the degree to which the entity would lack oversight. Even with automatic funding, policymakers would want to periodically review the activities they

35. For a discussion of this issue, see Gail R. Wilnesky, "Developing a Center for Comparative Effectiveness Information," *Health Affairs,* Web Exclusive (November 7, 2006), pp. w572–w585.

36. Congressional Research Service, *The Quasi Government: Hybrid Organizations with Both Government and Private Sector Legal Characteristics,* RL30533 (updated February 13, 2007).

37. Current funding for AHRQ resembles a dedicated financing source in that it comes entirely from funds that are designated under the Public Health Service Act as available for evaluation activities. The total amount of funds available for such activities had been limited to 1 percent of certain expenditures (primarily those for research by NIH), but in recent years, that limit has been set at about 2 percent. As a practical matter, however, the agency's funding is like other discretionary appropriations. In previous years, some funding for AHRQ (and its predecessor agencies) came from regular appropriations, and a few million dollars was transferred from Medicare's trust funds.

were funding either to consider changes in the levels of spending or to adjust any funding formula to keep dedicated resources in line with spending trends—which could also provide a vehicle for pressure from interest groups. Nevertheless, automatic or dedicated funding mechanisms would tend to limit the influence of political pressure to some extent. But such mechanisms also would raise questions about how the entity set its priorities and allocated resources—and how it would be held accountable for those decisions. A nongovernmental organization might be able to act more quickly than a federal agency, but that speed could come at the expense of transparency.

Under any option, an advisory board (or governing council) could be established to serve several functions: providing guidance to the entity and establishing priorities for its research projects; creating an independent process for reviewing and possibly approving the findings that resulted from that research; and serving as a channel for interested parties to participate. For example, the board could include representatives of major federal health programs, private insurers, health care providers, advocacy groups for patients, and drug and device makers—as well as members of the general public and disinterested policy experts. Alternatively or in addition to including various stakeholders, a regular process could be established for getting input from interested parties. An example of that type of structure is the U.S. Preventive Services Task Force (see Box 2).

In designing such an oversight group, a number of issues would arise. The types of participants on any board and the manner in which members were chosen and replaced would have to be determined carefully to avoid giving one perspective undue influence. Similarly, conflict-of-interest rules governing the entity's staff would probably be needed. Trade-offs could exist between the extent to which many views and interests were represented and the ability of the council or board to make timely decisions or to reach consensus on contentious issues. Whether any oversight group was involved in reviewing or approving the results of research projects or focused instead on which projects to initiate and what those reviews entailed would also affect the entity's staffing requirements and the types of expertise that board members needed.

Another organizational issue is whether to establish a single or highly centralized entity or, instead, to design a more loosely coordinated system encompassing several distinct centers to produce independent analyses. Many of the options that have been proposed seek to centralize research activities through one entity—partly to address concerns about the lack of coordination among current U.S. efforts. An advantage of that centralized approach is that it would tend to give more weight to any conclusions reached. At the same time, that potential for having a greater impact could also lead the organization to adopt findings that were watered down to reach consensus; even if the entity did not have a formal approval process and instead simply released any results of approved projects, a single agency might be more reluctant to pursue research into more contentious questions. A decentralized approach could give individual research centers more latitude and encourage more competing perspectives to emerge. However, a more pluralistic approach could also involve some redundant efforts and, if it yielded any conflicting findings, would leave users with the task of reconciling the results.

An additional consideration—particularly if a new entity was created—would involve start-up costs and other implementation challenges. If funds were directed through an existing federal agency, some ongoing costs for additional staffing would be incurred, but the basic support infrastructure would largely exist already. By contrast, establishing a new agency or public–private partnership could require a greater effort before research could begin. At the same time, a quasi-governmental organization or public–private partnership could have more flexibility to develop and maintain its staff than a new or existing federal agency would have. Creating a new source of revenues (such as a tax on health insurance premiums) to help fund research on comparative effectiveness would also involve time and administrative costs.

Among existing organizations, their relative strengths and weaknesses could affect which one was best suited for new research efforts. NIH has extensive experience overseeing clinical trials but may not see research on comparative effectiveness as central to its mission of expanding the frontiers of biological and medical knowledge. AHRQ has substantial expertise in many areas of comparative effectiveness but has limited experience managing trials, and some observers have raised concerns about the impact that significantly expanded research about comparative effectiveness might have on that agency's other research endeavors. For its part, the Institute of Medicine is widely respected but does not have an extensive organizational capacity to conduct or oversee primary research,

Box 2.

The U.S. Preventive Services Task Force

The U.S. Preventive Services Task Force was established in 1984 by the Department of Health and Human Services to produce recommendations about which preventive health care services should be routinely provided to individuals who do not have any symptoms of a given disease. Such services include immunizations, tests to screen for the presence of diseases, and behavioral counseling (such as programs that encourage smokers to quit).[1]

The size and composition of the task force has varied over time, ranging from 10 to 20 members; the members are not federal employees but have generally been practicing clinicians. The task force's work is currently supported by the Agency for Health Care Research and Quality (AHRQ), with an annual budget of about $3 million. As a rule, the task force does not fund studies that evaluate preventive services but instead relies on existing evidence. Two research centers that AHRQ has designated generate summaries of that evidence—which are similar to but perhaps not as rigorous as systematic evidence reviews. Given the available time and resources, the task force has not sought to review all preventive services but instead has assigned priority to services that address significant health problems, that are likely to have new evidence available, or that have generated controversy about their use.

In developing its recommendations, the task force considers both the strength of the evidence and the magnitude of the expected benefits and risks. Risks can include adverse reactions to vaccines, false-positive test results that lead to unnecessary or even harmful follow-up care, and complications from invasive test procedures—which can have substantial aggregate effects even if their probabilities are low, because preventive services may be provided to very large numbers of people. The task force's recommendations cover which types of asymptomatic individuals should receive the services, taking into account how the risk of contracting a condition or disease varies by age, sex, and other factors.

1. For a general discussion, see Eileen Salinsky, *Clinical Preventive Services: When Is the Juice Worth the Squeeze?* Issue Brief No. 806 (Washington, D.C.: National Health Policy Forum, August 24, 2005).

and some observers believe its consensus-building process could make timely action difficult.

Among the options for a new entity, establishing an FFRDC has generated some interest, partly on the grounds that it would be somewhat insulated from political pressure. But most of an FFRDC's funding would have to come from a federal agency, so it is not clear why its activities (most of which, presumably, would also be contracted out to private researchers) would be subject to less pressure than the activities of an agency receiving direct funding. The argument is sometimes made that private contributions would make private payers more likely to accept and use the results of the research. If such contributions were voluntary, however, the incentives to make them would be modest because the benefits of the research would accrue to many parties. If such contributions were instead required, then the arrangement would be essentially equivalent to having the government collect the money and appropriate the funds via a federal agency.

More generally, competing perspectives exist about how the relative roles of public and private payers in funding research on comparative effectiveness would affect perceptions about the results of that research. In some quarters, the findings of research funded by the government are seen as reflecting political pressure, perhaps to accommodate the views of interest groups or to support budgetary objectives. Those concerns could be attenuated to some degree if the agency conducting the research was not also a payer for health care, such as CMS. At the same time, other observers have raised concerns about privately sponsored research, which is also seen as advancing cost-cutting objectives (if sponsored by insurers) or as promot-

Box 2.

Continued

The task force has presented its recommendations in a periodic series of reports, the most recent of which covers about 60 specific services. Those services are now given a letter grade, as follows:

- A, for services that are strongly recommended on the basis of solid evidence that the benefits of improved outcomes outweigh the risks of harm;
- B, for services that are recommended on the basis of reasonable evidence of net benefits;
- C, for services with no recommendation because the balance of benefits and risks is too close;
- D, for services that should not be routinely provided because the evidence indicates the services are ineffective or that the risks outweigh the benefits; and
- I, for services that do not have sufficient evidence on which to base a recommendation.

Initially, when formulating recommendations, the task force did not take into account the costs of providing preventive services or their cost-effectiveness.[2] According to one recent summary, however, the task force now "considers the total economic costs that result from providing a preventive service, both to individuals and to society, in making recommendations, but costs are not the first priority."[3] Although some immunizations against a disease have been shown to reduce total spending on health care, many other preventive services appear to increase spending on net—either because of the costs of providing those services to large segments of the population (only some of whom will be found to have the disease) or because the overall effects on treatment costs are modest. Analyses of cost-effectiveness would shed light on how the health benefits of preventive services compared with those increases in spending.

2. See Somnath Saha and others, "The Art and Science of Incorporating Cost-Effectiveness in Evidence-Based Recommendations for Clinical Preventive Services," *American Journal of Preventive Medicine*, vol. 20, no. 3 (April 2001), pp. 36–43.
3. Russell P. Harris and others, "Current Methods of the U.S. Preventive Services Task Force," *American Journal of Preventive Medicine*, vol. 20, no. 3 (April 2001), pp. 21–35.

ing the interests of drug and device manufacturers and of providers of health services.

Options for Comparing the Effectiveness of Treatments

The appropriate organizational form for any new or expanded federal entity, along with the mechanism and level of funding, may depend in large part on what activities it would carry out. For example, analyzing existing data would require a different set of skills, and would cost less, than overseeing new clinical trials that compared different treatments. In addition to setting priorities among the various methods of research, a new or expanded entity would have to define the scope of its analyses—both the types of comparisons it would commission and the questions that analyses would address. In particular, would the organization focus only on trying to determine which treatments conferred the greatest medical benefits, or would it also assess which treatments were most cost-effective? Whatever approach was taken, the manner in which the results were communicated to doctors, patients, and health insurers could play an important role in determining the impact on medical practice.

Methods of Research

Federal efforts to assess different treatment options could be pursued in a variety of ways. Options range from synthesizing existing research—a process known as a systematic review—to conducting new studies using data that are already available to funding new head-to-head clinical trials. Although those options are not mutually exclu-

sive—indeed, they could all be pursued at the same time—each one presents certain challenges, with potential trade-offs arising between the costs of the activities and the value of the information they provide.

Systematic Reviews of Existing Research. The approach that would probably be easiest to implement would be to review and summarize the results of existing studies in a systematic and rigorous way. For example, even though existing studies may only compare a single treatment to a placebo, the results of several studies of individual therapies could in some cases be combined to measure those treatments against one another. That effort could also critically assess the strengths and weaknesses of the available evidence and seek to reconcile conflicting findings or determine what the preponderance of the evidence indicated. Such reviews would be comparable to some of the work that AHRQ is already undertaking and to some current efforts based at universities or other public and private research centers such as ECRI and Hayes, Inc. One advantage of this approach is its relatively low expense; a single systematic review might cost a few hundred thousand dollars.

Because the evidence base for comparing treatment regimens is itself limited, however, how much additional insight can be gleaned from systematic reviews of existing research is not clear. Data from clinical trials that had already been conducted would naturally be the focus of any systematic review, because trials can provide the clearest evidence about a treatment's effects, but such studies also have limitations. Some analyses have indicated that clinical trials sponsored by interested parties—which is often the only source of such data—are more likely than independent studies to find favorable results.[38]

Another potential limitation is that existing information may not be sufficient to reach definitive conclusions. Studies may be difficult to compare or reconcile, either because they use different methodologies or analyze different populations of patients, or simply because they yield conflicting findings. For example, a number of independent studies have examined different screening techniques for colorectal cancer, each of which provides an estimate of the cost per enrollee for each increase in QALYs. But according to a recent review of those studies, the results varied to such an extent that reaching a definitive conclusion about which technique was most effective or most cost-effective was difficult (see Table 2).[39]

Available studies of treatments may have even more limitations than studies of screening tests, because trials of treatments for particular diseases frequently exclude patients with other health problems, elderly enrollees, or other populations that may be of considerable interest in gauging comparative effectiveness; as a result, determining how broadly the results apply or whether they will hold for other groups of patients is hard to do. The fundamental issue is that, no matter how rigorously a systematic review is conducted, its contribution is by definition constrained by the extent and quality of the underlying evidence.

A recent systematic review of drug treatments for one form of diabetes that was sponsored by AHRQ illustrates both the strengths and weaknesses of such research.[40] The review covered a large body of literature, consisting of over 200 reports, and it was able to reach a relatively clear conclusion: Older drugs were found to be at least as effective as newer drugs in controlling patients' blood sugar and cholesterol levels. Most of the studies that were reviewed had relatively short durations, however—two years or less—so they were not able to address the impact on mortality or other effects of diabetes on morbidity (which can take a long time to materialize). The studies also tended to focus on nonelderly white patients, so they could not address the effectiveness for other populations; indeed, the review recommended that several clinical trials be conducted to fill in those gaps. Moreover, study subjects typically had no other significant health problems, whereas most patients with diabetes also have other

38. See Justin E. Bekelman, Yan Li, and Cary P. Gross, "Scope and Impact of Financial Conflicts of Interest in Biomedical Research: A Systematic Review," *Journal of the American Medical Association,* vol. 289, no. 4 (January 22/29, 2003), pp. 454–465; Stephan Heres and others, "Why Olanzapine Beats Risperidone, Risperidone Beats Quetiapine, and Quetiapine Beats Olanzapine: An Exploratory Analysis of Head-to-Head Comparison Studies of Second-Generation Antipsychotics," *American Journal of Psychiatry,* vol. 163, no. 2 (February 2006) pp. 185–194; and Jeffrey Peppercorn and others, "Association Between Pharmaceutical Involvement and Outcomes in Breast Cancer Clinical Trials," *Cancer,* vol. 109, no. 7 (April 2007), pp. 1239–1246.

39. Medicare Payment Advisory Commission, *Report to the Congress: Increasing the Value of Medicare* (June 2006), pp. 232–233.

40. See Shari Bolen and others, "Systematic Review: Comparative-Effectiveness and Safety of Oral Medications for Type 2 Diabetes Mellitus," *Annals of Internal Medicine*, vol. 146, no. 6. (September 18, 2007), pp. 386–399.

Table 2.

Cost-Effectiveness of Different Screening Methods for Colorectal Cancer

(Dollars)

Screening Method	Lowest	Highest
Colonoscopy		
Every 5 Years	17,316	36,612
Every 10 Years	10,633	26,693
Fecal Occult Blood Testing		
Annually	4,643	25,860
Every 3 Years	2,942	10,861
Sigmoidoscopy		
Annually	1,391 [a]	1,391 [a]
Every 3 Years	16,318	20,727
Every 5 Years	14,384 [b]	42,310

Source: Congressional Budget Office based on Medicare Payment Advisory Commission, *Review and Analysis of Cost-Effectiveness Analyses for Two Medicare-Covered Services* (prepared by the Institute for Clinical Research and Health Policy Studies, New England Medical Center, June 2006), available at www.medpac.org.

Note: The cost-effectiveness ratio is the estimated cost per one-year increase in quality-adjusted years of life expectancy, in comparison with the result of no screening.

a. Only one study was available for analysis.

b. One study found that screening every five years yielded lower costs and better health outcomes than no screening.

diseases, limiting the potential usefulness of the findings. In addition, the implication of the review—that older drugs for diabetes should be tried first—was already the protocol recommended by the American Diabetes Association. Thus, although the review was relatively inexpensive to conduct and may well have been worth its costs, its contribution was also limited.

In some cases, the existing evidence may permit more clear-cut determinations, but many systematic reviews are inconclusive—so views differ about their overall contribution. Britain's National Institute for Clinical Excellence (NICE) relies solely on systematic reviews of available studies. It has nonetheless been able to analyze many different treatments on the basis of their cost-effectiveness and to develop an extensive set of clinical guidelines and recommendations about using medical technologies.[41] Whether that record indicates the greater strength of the evidence on the reviewed treatments or a greater willingness on NICE's part to draw conclusions from that evidence is not clear. Typically, though, systematic reviews find that the available evidence is not adequate to address many important questions, so the primary value of such reviews may lie in clearly identifying the gaps in knowledge that should be the subject of future research.

Analyses of Claims Records. A somewhat more challenging approach than reviewing existing studies would be to fund new analyses comparing medical treatments using existing sources of data, such as health insurance claims records. An advantage of that approach is that it could provide new information to help resolve uncertainties about treatments at relatively low cost—using data on patients that had already been treated.

A central difficulty in such studies, however, is accounting for the differences in patients' health status that play a role in determining which treatment they get—which can make simple comparisons misleading. Insurance claims typically do not include any information about health status. Yet patients with more severe heart disease, for example, are more likely to receive invasive and expensive surgical procedures such as an angioplasty or a bypass operation. The greater severity of their condition may also make them more likely to have a subsequent heart attack and more likely to die. As a result, a comparison with patients receiving less aggressive treatments—who are probably not as sick, on average, to begin with—could understate the benefits of more aggressive treatments. In other settings, patients receiving more aggressive treatments may be healthier, so even well-designed observational studies can generate misleading findings regarding the benefits of those treatments. Studies of

41. To estimate cost-effectiveness, NICE generally combines the results of such reviews with its own models of the impact of different treatment options on the use of health services and health care spending.

cancer care, for instance, may be particularly susceptible to such confounding effects.[42]

To address such problems, researchers might be able to analyze geographic differences in treatment patterns to compare the impact of different treatments on comparable types of patients. For example, one study using claims data for Medicare enrollees sought to exploit the fact that patients living farther away from hospitals that treat a high volume of heart attacks were less likely to receive an intensive treatment for that condition (such as an angioplasty or a bypass operation).[43] At the same time, patients living farther from such hospitals appeared to be about as healthy as patients living nearby—so grouping patients by distance could address the analytic problem noted above whereby sicker patients get more intensive treatments. The study found that patients living closer to high-volume cardiac hospitals had slightly lower mortality rates, but the difference arose on the first day of admission and thus did not seem related to which procedure (if any) they ultimately received.

That example illustrates some of the challenges involved in using observational studies that are based on claims data. A key one is finding a factor that can be used in the analysis that is correlated with the treatment that patients receive but is not correlated with their underlying health status. (Economists refer to such factors as "instrumental" variables.) Even if such factors are available, proving that other confounding effects did not influence the results can be difficult. Whether that study's findings were persuasive enough to reduce the use of intensive medical treatments for heart attack patients, for example, is not clear. And the case of heart attacks—where urgent hospital care can mean the difference between life and death but where a range of potentially effective treatments are available—may be easier to analyze than other conditions.

Other issues surround the claims data themselves. First, maintaining the privacy of the patients whose records were being examined would be an important matter but could also present a barrier to conducting such studies. For statistical reasons, extracting meaningful results could require a large volume of claims data (as was the case in the study of heart attack treatments). Second, the quality of the study that could be conducted would depend on the level of detail that the data provided. Comparisons of the effects of treatments on mortality rates would be easier to generate because that information is relatively easy to obtain. Effects on morbidity or on the extent to which symptoms are relieved, however, might be more difficult to ascertain—depending on whether the relevant data were readily available. In addition, private health plans might have difficulty in conducting longer-term comparative effectiveness studies using claims data on their enrollees given the turnover in insurance coverage; if patients who changed plans were different from those who remained, statistical obstacles might undermine the comparison.

The expanded use of electronic health records could facilitate more-sophisticated analyses, if the issues of access and privacy could be addressed. In particular, those records could provide more comprehensive information both about the health histories of different patients and about their health outcomes. That additional information would make controlling for differences among patients receiving different treatments easier and would allow studies to address a broader set of outcomes than mortality. Some work of that nature is currently being conducted through the HMO Research Network and through a broader network of centers that have access to electronic databases that AHRQ established in 2005.[44] One challenge, however, is that the electronic records of different health plans are not always compatible, making aggregating data difficult.

42. See Alan M. Garber, "Cost-Effectiveness and Evidence Evaluation as Criteria for Coverage Policy," *Health Affairs,* Web Exclusive (May 19, 2004), pp. W4-284–W4-296. Some analyses have found similar results for observational studies and randomized controlled trials of the same treatment, but others have found important differences in the magnitude of the treatments' effects, particularly when the nonrandomized studies were done retrospectively. See Kjell Benson and Arthur J. Hartz, "A Comparison of Observational Studies and Randomized, Controlled Trials," *The New England Journal of Medicine,* vol. 342, no. 25 (June 22, 2000), pp. 1878–1886; and John P. A. Ioannidis and others, "Comparison of Evidence of Treatment Effects in Randomized and Nonrandomized Studies," *Journal of the American Medical Association,* vol. 286, no. 7 (August 15, 2001), pp. 821–830.

43. Mark McClellan, Barbara J. McNeil, and Joseph P. Newhouse, "Does More Intensive Treatment of Acute Myocardial Infarction in the Elderly Reduce Mortality? Analysis Using Instrumental Variables," *Journal of the American Medical Association*, vol. 272, no. 11 (September 21, 1994), pp. 859–866.

44. For a discussion of those efforts, see Lynn M. Etheredge, "A Rapid-Learning Health System," *Health Affairs,* Web Exclusive (January 26, 2007), pp. w107–w118; and related articles contained in that supplemental issue.

Medical Registries. Another option that could supplement or help improve analyses of claims data would be to establish medical registries, which generally track patients who have a particular disease or who have received a specific treatment. Registries collect additional information that is typically not contained in claims records, such as measures of health status or test results. In the United States, a number of registries—established or managed by various entities, including medical specialty societies and product manufacturers—have been used to help determine the clinical effectiveness or cost-effectiveness of various products and services.[45] Some health plans establish registries of their enrollees, although a centrally managed registry would have the advantage of being able to track patients if they moved or changed health plans.

Data from medical registries could help improve claims-based analyses both by allowing a broader set of outcomes to be measured and by providing information to control for differences among patients getting different treatments, including the severity of their illness. But a number of challenges and trade-offs would exist. One issue would be how to recruit patients and their providers to participate in and provide information to the registries and to retain them over time. Voluntary participation might be easy to implement but could introduce bias into analyses if patients choosing to participate differed in important ways from patients who had opted out. Some form of mandatory participation could avoid that problem but might raise objections from participants. Registries focused on specific treatments could also be subject to bias if those patients differed systematically from patients who did not receive those treatments—a problem that could be addressed by including a comparison group in the registries. Another trade-off concerns the data elements to collect; a more extensive list would permit richer analyses but would raise the burden of participation. More-extensive registries and registries involving more patients would also be more expensive to operate, although the annual costs of maintaining a typical registry are probably on the order of several million dollars.

The establishment of registries could affect medical practice in various ways. For example, CMS recently instituted a policy of "coverage with evidence development" for Medicare, to address treatments with potentially promising but uncertain medical benefits. Under that policy, Medicare now covers the costs of implantable cardioverter-defibrillators for a broader set of heart conditions than had previously been eligible—but only if those new patients are included in a registry that is supposed to track their progress.[46] If CMS would otherwise have decided not to cover that treatment for those patients, then the new policy means an increase in spending in the near term, but it also allows broader access to that technology in order to help generate the kind of evidence needed to reach a conclusion about its value. The registry may also help ensure, through its documentation requirements, that all patients meet the medical criteria required for Medicare coverage. Another example comes from Sweden, where a registry of patients undergoing hip replacement surgery has been used to provide periodic feedback to doctors about their surgical techniques and to track which specific models of artificial hip have the lowest rates of complications. That effort is credited with reducing health costs by avoiding repeat operations to fix faulty or poorly installed hips.[47]

Randomized Controlled Trials. The method of research that would probably yield the most-definitive results involves randomized controlled trials to compare treatments head to head, but that approach would also be the most expensive and would take the longest to conduct. The main advantage of random assignment is that it usually ensures that any differences in outcomes reflect true differences among treatments and not confounding differences among patients (such as their health status or other factors that are more difficult to observe). But detecting differences that are statistically significant—that is, unlikely to have arisen simply by chance—can require a substantial number of patients to participate, and in some cases, they must be followed for several years. Total costs for conducting an extensive trial can exceed

45. For more information, see Richard E. Gliklich and Nancy A. Dreyer, eds., *Registries for Evaluating Patient Outcomes: A User's Guide*, AHRQ Publication No. 07-EHC001-1 (Rockville, Md.: Agency for Healthcare Research and Quality, April 2007).

46. See Sean R. Tunis and Steven D. Pearson, "Coverage for Promising Technologies: Medicare's 'Coverage with Evidence Development,'" *Health Affairs,* vol. 25, no. 5 (September/October 2006), pp. 1218–1230. An implantable cardioverter-defibrillator (ICD) is a device designed to quickly detect a life-threatening rapid heartbeat and to deliver an electric shock that converts the rhythm back to normal. Apparently, CMS has not yet implemented the longitudinal registry for ICD patients.

47. See Henrik Malchau and others, "The Swedish Total Hip Replacement Register," *The Journal of Bone and Joint Surgery,* vol. 84, no. 11 (November 2002), pp. S2–S20.

$100 million over the course of the study, although many trials are less expensive, and some may cost only a few million dollars. (One factor affecting the costs of funding a trial is whether the health care services that the participants receive will be paid for by a third party, such as Medicare.)

Although the number of studies reporting results from randomized controlled trials has increased sharply, a number of questions have also been raised about the findings that can be derived from the existing stock of trial results. Many trials are aimed at demonstrating efficacy rather than effectiveness—the distinction being that efficacy reflects optimal conditions, whereas effectiveness requires a demonstration in real-world medical settings.[48] Partly as a result, patients with other health problems or groups such as the elderly are often excluded from trials. Further, many trials focus on demonstrating efficacy for a narrowly defined set of patients, so the results may not be generalizable; and combining studies in order to compare multiple treatment options may offer its own difficulties because of the differences among the patients studied. Finally, questions about the objectivity of industry-sponsored trials have also been raised.

To address those problems, some observers have recommended a greater emphasis on "practical" clinical trials.[49] The two key features of such trials are that they compare treatment choices that clinicians face and include a wide variety of study participants drawn from a range of practice settings. Traditionally structured trials, such as those typically sponsored by NIH, can involve a relatively large number of participants and relatively long periods of follow-up observation and analysis. As a result, they may be relatively costly to implement. Trials that are simpler and less expensive and that take less time to carry out could provide a greater "bang for the buck," but at some risk of reduced accuracy.[50]

Because their results can be persuasive, well-structured trials can have a noticeable effect on the use of treatments. For example, according to one report, the findings of the trial (discussed above) comparing the use of angioplasty and a metal stent with nonsurgical management of patients with stable coronary artery disease—which found minimal advantages of stenting—may have reduced the use of that procedure.[51] Determining the precise effect of the trial is difficult, however, in part because the downward trend in stenting procedures began about eight months before the trial's results were publicized. Another example comes from the trial that CMS sponsored assessing lung-volume-reduction surgery. Although that study identified some types of patients who would benefit from the procedure, and Medicare decided to cover it nationwide in those cases, the number of Medicare enrollees undergoing that surgery actually declined after the study was published (apparently reflecting the risks of undergoing the procedure that were discovered).[52] Such effects on medical practice may not be typical, however, and in any event, it took seven or eight years to complete those trials and release the results.

In addition to trials' relatively high costs and long durations, other constraints limit the number of trials that can

48. In other words, a finding of efficacy shows that a treatment can work for some patients in some circumstances, whereas a test of effectiveness determines whether the treatment usually works for a broader set of patients.

49. See Sean R. Tunis, Daniel B. Stryer, and Carolyn M. Clancy, "Practical Clinical Trials: Increasing the Value of Clinical Research for Decision Making in Clinical and Health Policy," *Journal of the American Medical Association,* vol. 290, no. 12 (September 24, 2003), pp. 1624–1632.

50. A recent example may illustrate the risks of drawing conclusions from trial results too quickly. In 2002, a trial of hormones used to treat menopause was halted abruptly when the initial findings indicated widespread increases in the risk of heart attack for participants. Subsequent analysis, however, found that the effects varied substantially depending on the ages of the patients and that some groups would benefit from hormone replacement therapy. See Tara Parker-Pope, "How NIH Misread Hormone Study in 2002," *The Wall Street Journal,* July 9, 2007. Even so, the trial's results indicated that observational comparisons had generally overstated the benefits of hormone replacement therapy because they did not adequately account for differences between the patients who received that treatment and the ones who did not.

51. See Keith J. Winstein, "Stent Implants Declined in April; Doctors Attribute Drop to Study Showing Drugs May Have Similar Benefits," *Wall Street Journal,* May 17, 2007. According to that report, total spending in the United States on angioplasties with stents was about $14 billion in 2006, but the number of stenting procedures began to decline in mid-2006.

52. Prior to the initiation of the trial, Medicare did not have a national policy regarding coverage of lung-volume-reduction surgery, but many of the local organizations that process Medicare claims had been approving it and paying for it under existing billing codes. See Tunis, "Coverage Options for Promising Technologies"; and Gina Kolata, "Medicare Says It Will Pay, but Patients Say 'No Thanks,'" *New York Times,* March 3, 2006.

feasibly be conducted at any given time. One is getting a sufficient number of patients to participate to allow valid statistical comparisons of treatment outcomes. For medical conditions that are common, that may not be a substantial challenge, but the difficulty increases the more narrowly the target population is defined—just because fewer patients meet the criteria for participation in the trial. Ethical issues can also arise if one set of participants is assigned a treatment that is generally considered less effective, although such concerns may be less likely to arise when significant uncertainty exists in the medical community about the relative benefits of different treatments. In light of those constraints, significantly expanding comparative effectiveness research is likely to require a combination of randomized trials and other research methods.

Modeling. Another approach that has been suggested—as an alternative or supplement to clinical trials—is the use of computer models to simulate the effects of treatments on different populations of patients. While many well-designed models exist, perhaps the most prominent one is known as Archimedes; its development has been led by Dr. David Eddy with the support of the Kaiser Permanente health plan.[53] One benefit of that approach is that, once such a model is developed, it can be used to answer questions about effectiveness at relatively low cost. Indeed, that approach can even have advantages over analyses of claims data, electronic health records, or medical registries: If the model can accurately predict the effects of a new treatment, waiting for those treatments to be used and then tracking their effects on actual patients over time can be avoided in some cases.

Achieving that objective may be quite difficult, however, and a particular obstacle is that models rich enough to simulate real-world medical care may not be transparent enough to generate confidence in or acceptance of their results. Archimedes, for example, is a highly complex model that seeks to capture not only the behavior of doctors and patients but also many of the biological processes of the human body. Tests of the model have shown that under certain conditions, it is able to predict the results of trials with high accuracy. In those tests, a set of trials is examined—and usually, about half of them are used to calibrate the model, while the rest are used to test its predictions. It is not clear, however, how well the model would do when starting with a less extensive evidence base, so its primary contribution might be to fill in some gaps between existing trial results and to permit modest extensions of completed trials at relatively low cost. For more ambitious efforts, it would not be possible to tell whether the model's predictions proved correct or incorrect until after the treatment in question had been used and analyzed via the other methods described above.

The Scope and Focus of Analyses and the Dissemination of Results

In addition to determining what types of research to conduct, any organization sponsoring research on comparative effectiveness would have to make a number of decisions about the scope and focus of that research—or policymakers might decide to set parameters for those decisions. One important question is whether federally sponsored research would seek to assess both the relative clinical benefits and the cost-effectiveness of treatments. A second is what balance to strike between evaluating treatments already being used widely and examining new treatments that seemed likely to become common—and more generally, how to keep up with the rapid pace of technological development in health care. Another issue is whether and to what extent the research would compare the performance of different providers or types of providers (such as high-volume and low-volume hospitals). Last but not least is the issue of how to communicate results to doctors, patients, and other interested parties.

Clinical Effectiveness or Cost-Effectiveness? There are arguments both for and against having federally sponsored research on comparative effectiveness consider cost-effectiveness as well as clinical effectiveness. Those arguments involve the practical steps needed to do the analysis and the ultimate effects of the research.

One practical reason a federal entity might not seek to assess which treatment was most cost-effective for a given type of patient is that the answer to that question might vary by health plan. Health insurance plans have different cost structures and may pay different prices for the same services, so there is an argument for giving insurers (and other interested parties) more information about the relative benefits of different treatments and letting those parties calculate which one was most cost-effective. Indeed, the prices of the inputs involved are often subject to

53. See David M. Eddy, "Linking Electronic Medical Records to Large-Scale Simulation Models: Can We Put Rapid Learning on Turbo?" *Health Affairs,* Web Exclusive (January 26, 2007), pp. w125–w136.

negotiation. But those negotiations could be influenced by objective comparisons of medical benefits. Australia's health agency, for example, calculates a price at which a new drug is cost-effective, given its clinical benefits relative to existing therapies. (That agency conducts the reviews of clinical effectiveness as well—but because it also administers that national health insurance program, the example may not shed light on this country's debate about whether and where to conduct cost-effectiveness analyses.)

More fundamentally, objections to considering cost-effectiveness reflect concerns that such efforts would at least taint the analysis of clinical effectiveness—and might ultimately be used to restrict access to valuable treatments. To the extent that federally funded analyses of clinical effectiveness facilitated calculations of cost-effectiveness by other parties, however, the same concerns about their ultimate impact would seem to arise. Moreover, well-designed studies would primarily affect treatments whose added benefits did not appear to justify their added costs, and access to treatments would depend largely on how the results were applied by public and private insurers. Thus, a more substantial concern about the research itself is that having the same organization fund analyses of both clinical effectiveness and cost-effectiveness could reduce the impact of any findings about the former—because those findings might be perceived as reflecting cost-control objectives.

An alternative view, however, holds that federal sponsorship of research addressing cost-effectiveness would give that research more credibility. Such sponsorship could help address concerns about the consistency of the methodologies used to calculate cost-effectiveness and about the transparency of the process by which those calculations were made.[54] In addition, some observers believe that federally sponsored analyses would be viewed with less suspicion than are studies conducted by private insurers. As a practical matter, having the federal entity develop or support an initial cost-effectiveness analysis, along with a template that insurers or others could use to modify the calculation using different prices, could also avoid some duplication of effort.

A more basic argument in favor of including cost-effectiveness is that achieving the greatest possible gains in the efficiency of the health sector ultimately would require assessing both the benefits and costs of different treatment options to see whether the added benefits of more-expensive options were worth their added costs. On balance, research that included an analysis of cost-effectiveness would probably have a larger effect on medical practice than research that analyzed only the comparative clinical effectiveness of different treatments—primarily because the results would sometimes highlight that benefits were small relative to the incremental costs.

Even so, extending the scope of research to include cost-effectiveness would raise a number of additional challenges. For example, the methods of calculating quality-adjusted life years could be a source of controversy. Although there may be substantial agreement within the scientific community about the relative benefits of avoiding different adverse outcomes—such as degrees of disability and risks and side-effects of surgery—converting those differences into the common metric of QALYs might nevertheless raise concerns among patients and other interested parties. Similarly, deciding how broadly or narrowly any findings applied would be a very important consideration, because some treatments might be more effective for certain subgroups of patients than for an average patient. That consideration would affect the design of studies and the comparisons that would be undertaken; that is, the studies would need to be sufficiently robust to examine the potential variation in benefits among subgroups of patients—in order to limit the risk of overlooking patients who could benefit greatly from a treatment.

Finally, the very practice of placing a dollar value (or range of values) on an additional year of life has generated controversy; many people find the notion uncomfortable if not objectionable, and the sentiment that no expense should be spared to extend a patient's life is often expressed. Nevertheless, researchers have developed estimates of that value reflecting choices that individuals are observed to make in other settings (for example, when they purchase life insurance or accept the risks of driving). Estimates of about $100,000 per year are commonly cited, though higher and lower figures are often used. An agency charged with analyzing cost-effectiveness would not, however, have to determine what the appropriate threshold or range was—that decision could be left to purchasers and other decisionmakers. Instead, the agency

54. Standards for conducting analyses of cost-effectiveness have already been developed; for a discussion, see M. C. Weinstein and others, "Recommendations of the Panel on Cost-Effectiveness in Health and Medicine," *Journal of the American Medical Association,* vol. 276, no. 15 (October 16, 1996), pp. 1253–1258.

could estimate cost-effectiveness ratios and rank treatment options on that basis.

Other Questions of Scope and Focus. In addition to choosing which methods of research to pursue and whether to consider cost-effectiveness, a new or expanded agency would need to consider several other questions of scope and focus as well. Would it make recommendations about coverage of treatments as well? On which treatments would it focus attention, and how would it set those priorities? Would it compare different ways of providing a given treatment or concentrate on assessing broadly different options? Would it also try to assess doctors and other providers in terms of their effectiveness? And should it take explicit steps to expand the capacity for comparative research or anticipate that supply would grow to meet demand?

The question is whether the new or expanded federal entity would make recommendations about which treatments should be covered by insurance—either generally or for public programs—is related to but separate from the issue of whether to assess cost-effectiveness. Some observers have suggested that a U.S. entity focusing on comparative effectiveness should steer clear of making such recommendations because they would be controversial in themselves and because they might be seen as tainting findings about relative medical benefits. As a practical matter, furthermore, the entity would not have to make formal recommendations in order for its research to affect the use of medical care, as long as its findings on clinical effectiveness or cost-effectiveness were considered credible by doctors and other health professionals and could be easily used by insurers and other parties.

A more pressing issue is how a new or expanded entity would choose the specific treatments on which to focus its attention. Selecting broad areas of treatment (such as cardiovascular disease) might be relatively easy, but tradeoffs could arise between focusing on specific treatments that were widespread, expensive, and had uncertain benefits or, instead, on emerging treatments and technologies that promised to be expensive and might be adopted widely but had not yet become common practice. In the former case, data might be more readily available, but changing ingrained practice patterns might be difficult (short of producing evidence of actual harm). In the latter case, analyses might be more difficult to conduct given the limited claims data that would be available, while generating new data via clinical trials would take several years and thus might not be timely. A related question is how frequently to reassess treatments or variations on them; according to one study, systematic reviews typically require revision after about five years.[55]

An additional issue is whether to expand the scope and structure of comparisons so that they analyzed degrees of service use within a given treatment approach, not just broadly different approaches. As noted above, the literature on geographic variations in health care indicates that overall surgery rates do not vary systematically or in a manner that is strongly correlated with the variation in total Medicare spending. Rather, spending differences reflect more intensive use of hospital and physician services (as well as more use of ancillary services like tests). Therefore, future studies might need to examine different approaches to providing the same basic treatment, such as the extent of follow-up care provided or the frequency of using tests and imaging services—in addition to the "either/or" question of whether a given type of imaging or test was informative. Such analysis could also be applied to structured programs of care coordination or disease management, in order to assess their impact on health and their cost-effectiveness.

Another question is whether assessments would be limited to procedures and treatments or would also seek to evaluate the performance of individual doctors. In particular, the data from medical records that were used to compare the effectiveness of different treatments for a given type of patient could also be used to analyze the quality with which doctors provided each treatment. The potential gains from such analysis would include identifying doctors who delivered high-quality care and encouraging doctors who were not performing as well to improve—and doing both on the basis of objective evidence. At the same time, concerns could arise that evaluating doctors would detract from the focus on identifying effective procedures. Further, controlling for differences among patients that could affect the ratings of numerous individual doctors could be even more challenging than controlling for differences in patients when comparing a small set of treatments. Although such an approach could

55. See Karen J. Shojania and others, "How Quickly Do Systematic Reviews Go Out of Date? A Survival Analysis," *Annals of Internal Medicine*, vol. 147, no. 4 (August 21, 2007), pp. 224–233.

have a larger impact on health care than examining treatments alone, it could also be highly controversial.[56]

A final question regarding the scope of the activities funded revolves around the capacity to conduct research on comparative effectiveness. Several observers have indicated that the capacity is currently rather limited, which could serve as an important constraint on any expansion of federally funded efforts. In particular, some recent discussions have suggested that federal spending on that research should increase by billions of dollars per year, but it is not at all clear that such sums could be spent in an effective way in the near term.[57] At a minimum, additional research efforts would probably reach a point of diminishing returns. The entity that oversaw those efforts might therefore want to consider the option of using some funds to expand the pool of skilled researchers and to encourage steps that would make it easier to incorporate comparisons of effectiveness into the routine practice of medical care.[58]

The Dissemination of Results. Whatever types of results were produced, any new or augmented entity focused on comparative effectiveness would want to consider carefully how those results were communicated to doctors, patients, and other interested parties. (Such communication efforts represent an important element of AHRQ's program on effective health care.) Providing information to both technical and general audiences that was both useful and accurate would be challenging, though; a particular difficulty might be conveying the degree of uncertainty surrounding conclusions. A useful first step might be to conduct a critical assessment of past dissemination efforts in order to identify their strengths and weaknesses.

The mechanisms by which the results of comparative studies were disseminated could have important implications for their impact on medical practice. In particular, one such pathway could be the incorporation of any findings into computerized decision-support tools that some physicians and health plans now employ. Rather than having to recall any relevant evidence from memory, physicians could call up the results of comparative effectiveness research for a given patient's symptoms—or be presented with those findings (or their practical implications) automatically. The limited infrastructure for information technology that currently characterizes the health system, however, presents an obstacle to capturing the full potential of this approach. Alternatively, a few studies have found that presenting patients with comparative information about the benefits and risks of treatment alternatives—particularly in cases when elective surgery is one of the options being considered and when patients may vary in their valuation of the benefits and risks—leads them to choose less intensive treatments for certain conditions.[59] What process is most effective for presenting such information to patients, however, and how broadly those findings apply are less clear.

Implications for Health Care Spending

To affect medical treatment and reduce health care spending, the results of comparative effectiveness analyses would ultimately have to change the behavior of doctors and patients. For any large-scale changes to occur, the new or expanded entity would have to generate new findings for a substantial number of medical conditions—which would take many years. To have the maximum effect on behavior, those findings would then have to be incorporated into the incentives for providers and patients, a process of adjustment that might also take time. Although some patients and providers might object to such changes, over the long term the combination of

56. CMS has taken some initial steps toward assessing the quality of care that individual doctors provide. The Tax Relief and Health Care Act of 2006 provided for modest bonus payments under Medicare to doctors who elect to report information on certain measures of the care they provide in 2007. Although CMS will be able to provide feedback to doctors on how their performance compares to their peers', the payments do not depend on that performance. Furthermore, the measures that have been chosen cover areas of substantial consensus in the medical community about appropriate treatment protocols (for example, prescribing drugs known as beta blockers to patients who have had a heart attack). For a broader discussion, see Government Accountability Office, *Medicare: Focus on Physician Practice Patterns Can Lead to Greater Program Efficiency,* GAO-07-307 (April 2007).

57. The Health Industry Forum, *Comparative Effectiveness Forum: Executive Summary* (summary of a conference, Washington, D.C., November 30, 2006), available at http://healthforum.brandeis.edu/meetings/materials/2006-30-Nov./ExecBrief.pdf.

58. For a discussion, see Sean A. Tunis, "A Clinical Research Strategy to Support Shared Decision Making," *Health Affairs*, vol. 24, no. 1 (January/February 2005), pp. 180–184.

59. See Annette M. O'Connor, Hilary A. Llewellyn-Thomas, and Ann Barry Flood, "Modifying Unwarranted Variations in Health Care: Shared Decision Making Using Patient Decision Aids," *Health Affairs,* Web Exclusive (October 7, 2004), pp. VAR-63–VAR72.

additional information and revised incentives would tend to reduce spending for health care below currently projected levels, potentially to a substantial degree.

Currently, Medicare is effectively precluded from taking costs into account when making decisions about coverage and would probably need new legal authority to adjust payments to providers or cost-sharing requirements for enrollees to encourage the use of more cost-effective care. For their part, private insurers might not face legal barriers to limiting coverage of or altering payments for treatments that were shown to be less effective but still might be reluctant to do so if Medicare did not alter its own policies regarding coverage and payment. Thus, beyond conducting the analyses themselves, many difficult steps would probably need to be taken before spending on comparative effectiveness research translated into substantial savings for federal programs and the health care system. Even so, additional information comparing treatments would tend to reduce federal health spending in the near term—but probably not by enough to offset the full costs of conducting that research over the same period.

The Potential for Savings on Health Care

Predicting the impact that research on comparative effectiveness could have on health care spending is difficult because it is hard to know what that research will show. In some cases, the research could provide clearer evidence than exists today that the benefits of an expensive treatment outweighed the costs—in which case spending on such treatments could increase. Some observers have therefore suggested that comparative effectiveness research could also cause spending to increase on treatments already considered effective but not used as extensively as recommended protocols indicate.[60] By itself, however, new research on comparative effectiveness seems unlikely to increase the use of services that are already deemed effective, for two reasons. First, that research is unlikely to focus on such cases—instead, it would presumably target treatments of uncertain value. Second, even if it did address those types of care, an additional finding of effectiveness would be unlikely to have much impact on the use of already-recommended services without corresponding changes in the incentives to use them.

Although spending increases in some areas would be possible, current incentives already favor the adoption and spread of more-expensive treatments, so new research that found those treatments to be more effective or more cost-effective would probably increase their use only modestly. As a general rule, the fee-for-service reimbursement system by which health care is primarily financed in the United States—especially but not exclusively in Medicare—typically provides financial incentives for doctors and hospitals to adopt new treatments and procedures broadly even if hard evidence about their effectiveness is not available. For their part, insured individuals generally face only a portion of the costs of their care and, consequently, have only limited financial incentives to seek a lower-cost treatment. Although private health insurers have incentives to limit the use of ineffective care, they are currently constrained both by a lack of information and by public concerns about overly aggressive management (as was evident in a recent "backlash" against managed care plans).

Conversely, credible and well-designed studies that found that more-expensive treatments and approaches to care yielded little or no additional health benefits would have a greater potential to affect health care spending. Moreover, the evidence that additional spending and use of services in some parts of the country is not producing better health suggests that additional comparative research would be more likely to question than to support the value of more-expensive services. Research that affected the demand for treatments would also affect their supply; in particular, if the developers of new medical products and procedures had to demonstrate their value more clearly, those parties would not only have incentives to produce more evidence but also would be encouraged to focus their developmental efforts on approaches that were more clinically effective or more cost-effective. Over the long term, therefore, generating additional objective information about the relative costs and benefits of treatments seems much more likely to reduce total health care spending than to raise it—particularly if public and private insurers incorporated the findings into their coverage and payment policies.

Getting to the point at which additional research on comparative effectiveness could have a noticeable impact on health care spending would take several years. In addi-

60. One recent study found that patients typically received about half of recommended services, whether for preventive care, treatment of acute conditions, or treatment of chronic conditions. See Elizabeth A. McGlynn and others, "The Quality of Health Care Delivered to Adults in the United States," *The New England Journal of Medicine*, vol. 348, no. 26 (June 26, 2003), pp. 2635–2645.

tion to the time required to get the new activities under way, a lag would exist before results were generated, particularly if they depended upon the completion of new clinical trials. Initially, the available results would probably address a relatively small number of medical treatments and procedures; additional time would elapse before a substantial body of results was amassed. And in areas of medicine with significant levels of spending, many studies could be needed before a consensus emerged about the appropriate conclusions to be drawn—even if those studies did not generate conflicting results. For all of those reasons, it would probably be a decade or more before new research on comparative effectiveness had the potential to reduce health care spending in a substantial way.

The magnitude of that impact in the long term would depend primarily on how private and public insurers used that information and whether and how the results were incorporated into the incentives facing providers and patients. But additional information could have a modest effect on health care spending in the near term even if those incentive systems remained largely unchanged. The information would primarily affect spending in the private sector, where the scope for using comparative information is currently greater, but some "spillover" effects for enrollees in public programs would also be likely because doctors are inclined to provide similar care to all of their patients.

Possible Responses by Private and Public Insurance Plans

To affect medical treatment and reduce health care spending, the results of comparative effectiveness analyses would ultimately have to change the behavior of doctors and patients—that is, to get them to use fewer services or less intensive and less expensive services than are currently projected. Bringing about those changes would probably require action by public and private insurers to incorporate comparative effectiveness information into some combination of their coverage and payment policies. Those steps, however, could be difficult and controversial.

Private Insurers. One option for private insurers would be to not cover drugs, devices, or procedures that were found to be less effective or less cost-effective. That approach might prove to be particularly controversial, however, and the insurers would have a number of additional options as well. They could simply provide more information to providers and patients, which could improve compliance with treatment guidelines. For example, the use of medicines known as beta blockers, which is recommended following a heart attack to prevent a recurrence, has grown substantially in recent years—apparently as a result of reporting on the share of patients who receive prescriptions for them.[61] The availability of that information may have encouraged individuals to seek health plans whose doctors were more likely to prescribe beta blockers and may have encouraged doctors to prescribe them.

Alternatively, insurers could require enrollees to pay some or all of the additional costs of more-expensive treatments that were shown to be less effective or less cost-effective (in which case enrollees would have to decide whether the added benefits were worth the added costs); that approach is sometimes called value-based insurance design.[62] Or insurers could adjust payments to doctors and hospitals to encourage the use of more-effective care. According to one recent study, private insurers will currently cover a more effective treatment in nearly all cases—even if it is more costly—but it is also common for them to require that more costly treatments receive prior authorization before they are used or that patients try a less costly intervention first.[63] Research on comparative effectiveness could be used to determine when to apply those requirements.

Making substantial changes to insurance design and reimbursement would not be easy. Some patients, providers, and other interested parties would probably object to such arrangements or to the manner in which insurers established them. A particular concern would be that the average effects found by studies might not apply

61. Since 1996, the National Committee for Quality Assurance (NCQA), a not-for-profit organization that provides information about health care quality, has required private health care plans to report that information in order to receive accreditation. The average share increased from 63 percent in 1996 to 95 percent in 2005, and as a result, NCQA has now adopted a more stringent measure (which tracks actual use of those drugs). See Thomas H. Lee, "Eulogy for a Quality Measure," *The New England Journal of Medicine,* vol. 357, no. 12 (September 20, 2007), pp. 1175–1177.

62. See Michael E. Chernew, Allison B. Rosen, and A. Mark Fendrick, "Value-Based Insurance Design," *Health Affairs*, Web Exclusive (January 30, 2007), pp. w195–w203.

63. Garber, "Cost-Effectiveness and Evidence Evaluation as Criteria for Coverage Policy."

to all types of patients that were considered—so that subgroups of patients who could benefit greatly from a treatment might be overlooked. And as discussed above, having research studies keep pace with the development of new technologies would be an ongoing challenge. Consequently, any new incentive systems would probably be applied only in areas of care where the evidence was convincing.

Making such changes would also generate some new costs for insurers. Some administrative costs would be incurred to monitor whether patients met the medical criteria for which a given treatment had been proved effective or cost-effective. An exception or appeals process might also be needed to permit case-by-case reviews, and negotiating more complex reimbursement arrangements with providers would entail some costs. Those costs would probably be small in comparison to the change in health spending, given that insurers already monitor the use of treatments to ensure that they are medically necessary and generally have appeals processes in place. In addition, providing stronger incentives for patients and providers to use effective care would probably increase the use of services that are already deemed effective. The types of effective care that studies find are underprovided, however, tend to be relatively inexpensive screening and monitoring services for chronic health problems.

The steps that private insurers took could both affect public spending and be affected by public programs' responses to additional information about comparative effectiveness. To the extent that changes instituted by private insurers affected doctors' methods, there would probably be some "spillover" benefits for public programs. However, private insurers might be more reluctant to pursue such approaches aggressively, at least in the short term, if public insurance programs were not adopting similar methods.

Medicare. To reduce spending substantially under Medicare on the basis of comparative effectiveness research would probably require additional legislative authority to allow the program to consider relative benefits and costs in a more extensive way and to modify the financial incentives facing doctors and enrollees accordingly. Under current law, Medicare does not have clear authority to take costs into account when making decisions about what treatments are covered and has made only limited use of information about relative clinical effectiveness. Federal law does not explicitly prohibit Medicare from considering costs, but the Medicare statute provides that the program will pay for items or services if they are deemed "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member."[64] A regulation was proposed in 1989 that would have included cost-effectiveness as a factor in determining whether a treatment was reasonable and necessary, but that proposed regulation generated considerable opposition and was eventually withdrawn.[65]

Most recently, Medicare officials sought to clarify the definition of "reasonable and necessary" for the purpose of determining whether a new treatment or procedure would be covered. In 2000, they issued a "notice of intent" to publish a proposed rule on that topic.[66] Under the concept outlined in that notice, Medicare would generally require new treatments to provide "added value," which was defined in the following way:

- A "breakthrough" technology (one conferring substantially more benefits than existing treatments) would be covered without regard to its cost.
- A new item or service that had some medical benefits would be covered regardless of its cost if no other medically beneficial alternative was available or if the alternative treatment used a different "clinical modality." (That term was not defined precisely, but drug therapy and surgery would clearly be treated as different modalities.)
- An item or service equivalent in its benefits to a similar currently covered service (using the same modality) would be covered only if its costs were comparable to or lower than the cost of the currently covered service.

64. See section 1862(a)(1)(A) of the Social Security Act.

65. See Peter J. Neumann and others, "Medicare and Cost-Effectiveness Analysis," *The New England Journal of Medicine*, vol. 353, no. 14 (October 6, 2005), pp. 1516–1522.

66. Health Care Financing Administration, "Criteria for Making Coverage Decisions," *Federal Register,* vol. 65, no. 95 (May 16, 2000), pp. 31124–31129.

That nascent proposal also generated opposition, however, and to date, CMS has not taken the next step of issuing a proposed rule.[67]

As a practical matter, therefore, under current policy and law, Medicare generally covers any treatment or procedure that has net medical benefits—that is, benefits that outweigh the risks of the procedure—regardless of its cost or its effectiveness relative to alternative therapies. As noted earlier, Medicare officials recently developed an initiative that provides provisional coverage for new treatments that have uncertain medical benefits—but also requires the resulting evidence about their effects to be analyzed so that a more informed final decision on coverage can be made using those data. That initiative, however, may not involve comparing different treatments to see which is more effective and does not appear to take the costs of treatments into account. At the same time, CMS officials have given some indications that they will consider whether a new treatment is as good or better (on purely medical grounds) than currently covered alternatives when making coverage decisions, and a recent decision not to cover artificial spinal discs took into account a comparison of that option with other spinal surgeries that are covered.

Medicare currently has somewhat more flexibility regarding the payments it makes for covered services, which can take comparative medical benefits—and, in some cases, costs—into account on a limited basis. For example, in order for a hospital to receive an additional payment for using a new device during a covered procedure (known as a "pass-through" payment), the device must be shown to provide a substantial clinical improvement for Medicare beneficiaries compared with the current technology. Over time, however, Medicare's payments to hospitals are adjusted to account for the costs of new technologies (on an aggregate basis) without requiring an explicit analysis of their effectiveness. Similarly, CMS requires evidence that a new procedure or device offers improved medical benefits compared with similar items or services in order to qualify for a new procedure code (which is then assigned a payment rate). As noted above, CMS has been supporting research to determine whether more frequent dialysis for certain kidney patients has clinical advantages; if so, the agency could establish a new procedure code and payment amount for that service.

Although Medicare has not generally used information about effectiveness to set payment levels, a recent exception is its policy that bases payment rates on the "least costly alternative" for certain types of items. Under that policy, Medicare will not cover the additional cost of a more expensive product if a clinically comparable one is available that costs less; in other words, the program's payment rate for both products is set at the level of the least expensive one. That policy has been applied to payments for durable medical equipment and to certain comparable drugs, but wider application to products that are not very close substitutes would probably require additional statutory authority.[68]

Even those limited steps toward using information about comparative effectiveness have proven controversial, however. Medicare's decision to apply the least costly alternative policy to set the payment rate for certain drugs that treat prostate cancer, for example, has raised concerns about whether the policy has been administered consistently and questions about whether that approach has been superseded by a new system that sets the reimbursement rate for each drug as a function of its market price.[69] Similarly, CMS set the payment rate for a new antianemia drug equal to the rate for two existing drugs on the grounds that the products were "functionally equivalent"—but then the Medicare Modernization Act of 2003 prohibited CMS from applying a standard of functional equivalence in any future case involving Medicare's payments to hospital outpatient departments. (CMS's decision regarding antianemia drugs was not overturned.) Similarly, the provisions of that act governing AHRQ's research on comparative clinical effectiveness also specified that the CMS administrator could not use the results to withhold coverage of a prescription drug—although the private drug plans administering that benefit could presumably use relevant findings when designing their formularies.

67. For a further discussion, see Susan Bartlett Foote, "Why Medicare Cannot Promulgate a National Coverage Rule: A Case of *Regula Mortis*," *Journal of Health Politics, Policy and Law*, vol. 27, no. 5 (October 2002), pp. 707–730.

68. For further discussion about Medicare's current use of information on comparative effectiveness, see Medicare Payment Advisory Commission, *Report to the Congress: Issues in a Modernized Medicare Program* (June 2005), pp. 180–182.

69. For a discussion of the least costly alternative policy, see Medicare Payment Advisory Commission, *Report to the Congress: Impact of Changes in Medicare Payments for Part B Drugs* (January 2007), pp. 10–11.

If changes in law were made, Medicare could use information about comparative effectiveness to promote the use of more-effective care. It could, for example, choose not to cover treatments that were less effective or less cost-effective or it could exclude extremely inefficient providers from participating in the program—just as private insurers may do today. Alternatively, Medicare could tie its payments to providers to the cost of the most effective or most efficient treatment. If that payment was less than the cost of providing a more expensive service, then doctors and hospitals would probably elect not to provide it—so the change in Medicare's payment policy could have the same practical effect as a coverage decision. Even so, patients and providers might object more strongly to a decision not to cover a treatment than they would to a change in Medicare's payment for it. Alternatively, enrollees could be required to pay for the additional costs of less effective procedures (although the impact on patients' incentives and their use of care would depend on whether and to what extent they had supplemental insurance coverage that paid some or all of Medicare's cost-sharing requirements).

More modest steps that Medicare could take would include smaller-scale financial inducements to doctors and patients to encourage the use of cost-effective care. Doctors and hospitals could receive bonuses for practicing effective care or reductions in their payments for using less effective treatments (although the evidence to date about the effect of such pay-for-performance initiatives on health care spending is somewhat mixed).[70] Likewise, enrollees could be asked to pay only a portion of the additional costs of less efficient procedures. Or Medicare could simply provide information to doctors and their patients about their patterns of practice, which would create some pressure for doctors to use more-efficient approaches and could encourage patients to select more-efficient doctors. Adopting more modest measures to incorporate the findings of comparative effectiveness research, however, would probably yield smaller savings for the program.

Medicaid. As for Medicaid, state officials generally determine what specific services are covered—subject to broad federal requirements—and are reimbursed by the federal government for a portion of the reported costs using formulas specified in law. Because enrollees have low income, options for adjusting cost-sharing requirements to encourage the use of cost-effective care may be limited. Furthermore, a substantial portion of Medicaid spending pays for long-term care services such as nursing home care for elderly and disabled enrollees, which may be less amenable to comparative effectiveness research. At the same time, most of the poor mothers and children enrolled in the program receive their care through a private health insurance plan under contract to Medicaid, so spending for them would be directly affected by any changes that private insurers made. Another portion of Medicaid spending goes to cover cost-sharing requirements and payments of premiums for enrollees who are also on Medicare, so the impact on that spending would depend largely on what the Medicare program did.

An additional issue in applying the results of comparative effectiveness studies in Medicaid relates to the sharing of program costs between the federal and state governments. Federal matching rates under Medicaid currently range across states from 50 percent up to about 75 percent, and, by CBO's estimates, the federal government now covers 57 percent of the reported costs of health services provided by that program across the nation as a whole. At least in principle, those financing arrangements reduce the incentives for state Medicaid officials to limit coverage of less effective services—because, on net, states would face only a portion of those costs currently and would see only a portion of the savings that resulted from a programmatic change. Some coordination between state and federal officials might therefore be required to incorporate the results of comparative effectiveness research.

At the same time, many states recognize the growing fiscal burden posed by Medicaid costs, and several of them have already expressed interest in comparative effectiveness research. For example, more than a dozen state Medicaid programs are involved in a project (affiliated with the Oregon Health and Sciences University) assessing evidence about the relative safety and effectiveness of competing drugs in the same class. Similarly, the state of Washington has recently initiated a program to provide independent assessments of health technologies; a committee of physicians and other providers will review that evidence and make decisions about what treatments will be covered under the state's Medicaid program and other state-run health care programs. Oregon tried a broadly similar approach in its Medicaid program the 1990s (although controversies about the ranking of medical ser-

70. See Congressional Research Service, *Pay-for-Performance in Health Care*, CRS Report RL33713 (December 12, 2006).

vices according to their overall cost-effectiveness ultimately limited the impact of that experiment).[71]

Potential Effects on Research and Development. If public and private insurers incorporated the results of comparative effectiveness research into their coverage and payment policies, the types of new medical technologies that were supplied could be affected. Developers of new drugs, devices, and procedures would have clearer incentives than currently exist to generate products and services that yielded substantial health gains relative to current treatments or that could replicate the benefits of current care at a lower cost. Either outcome would tend to improve the efficiency of the health sector.

One potential concern is that the results of comparative research might enable the manufacturers of products with patent protection (such as prescription drugs) to charge higher prices, if their product was shown to be superior. But those same results would put downward pressure on the prices of competing products, which in turn could dampen the incentive to increase the price of the "winner." What is more, research that could establish the superiority of a specific product—rather than comparing broadly different treatment options—is more likely to be undertaken by the private sector, so the added impact of any federally sponsored studies in such circumstances might be modest. Overall, during the period of patent protection, prices of products found to be clinically more effective might rise, and those found to be less effective might fall relative to the current situation in which less is known about the comparative effectiveness of different products. The result would be relative prices that more appropriately reflected the relative values of products in terms of patients' health outcomes. Such a situation would signal producers to place a greater emphasis on developing products with greater clinical effectiveness.

Perhaps a greater concern is that extensive use of information about comparative effectiveness would discourage medical innovation and thus reduce the flow of new products and treatments—but the types most likely to be forgone are those that would have modest expected benefits or poor prospects for demonstrating cost-effectiveness. A particular concern may involve poorly constructed studies, which could provide inaccurate information about the relative merits of treatments and thus, in turn, skew research incentives; that possibility reinforces the importance of having new studies use rigorous methodologies. Overall, greater emphasis on using rigorous data about comparative effectiveness would seem likely to alter incentives for product development in ways that improved the efficiency of the health sector both at a point in time and over time.

71. See Thomas Bodenheimer, "The Oregon Health Plan—Lessons for the Nation," *The New England Journal of Medicine*, vol. 337. no. 9 (August 28, 1997), pp. 651–655; and Jonathan Oberlander, "Health Reform Interrupted: The Unraveling of the Oregon Health Plan," *Health Affairs,* Web Exclusive (December 19, 2006), pp. w96–w105.

Estimated Effects of a Recent Proposal

The near-term effects on health care spending that expanded federal research on comparative effectiveness could have are illustrated by CBO's estimate regarding a provision in legislation that was recently passed by the House of Representatives. Section 904 of H.R. 3162, the Children's Health and Medicare Protection Act of 2007, would do the following:

- Establish within AHRQ a Center for Comparative Effectiveness Research, which would fund research comparing the clinical effectiveness of treatments—using clinical trials, systematic reviews, observational studies, medical registries, and other methods. The center would develop methodological standards for conducting studies of comparative clinical "value" but would not fund studies that analyzed treatments' cost-effectiveness.

- Create a commission to oversee the center's activities, consisting of the Director of AHRQ, the Chief Medical Officer of CMS, and up to 15 additional members who would have relevant expertise and would represent clinicians, patients, researchers, insurers, and employers. The Comptroller General of the United States, in consultation with certain Members of Congress, would appoint those 15 members to multiyear terms.

- Provide authority to spend $300 million from 2008 to 2010 and $375 million per year thereafter, funded primarily by an annual per capita fee of about $2 imposed on private health insurance premiums that would start in 2011.

The provision would not change any of Medicare's or Medicaid's rules about which procedures and treatments were covered or how much was paid for them. Thus, any

impact that the resulting research would have on federal spending for health care would have to come primarily from changes such research induced in doctors' patterns of practice or patients' choices of treatments. Those changes—encouraged in some cases by private health insurers—would primarily affect private health spending, but some changes in treatment patterns would also be likely for enrollees in public programs because doctors tend to treat their patients in a similar manner regardless of their source of insurance.

To a lesser extent, some federal savings might also occur through changes in coverage that could be implemented under current law (although CBO did not make explicit assumptions about what those changes would be). For example, if research on comparative effectiveness determined that a service covered by Medicare did not confer any health benefits for certain types of patients or involved risks that outweighed the expected benefits, under its current coverage policies CMS would have clear authority to decide not to cover that service for those patients.

As discussed, evaluating the precise effect of new research is difficult because it is hard to know which studies will be undertaken and what they will find, but CBO estimates that such research would probably reduce spending for health care somewhat. Any impact of a given research study is likely to be felt over many years, so the change in spending in any given year would reflect the cumulative effects of past studies. Little evidence is available with which to estimate the precise magnitudes of the annual effects, although one comprehensive review of the issue indicated that additional information about the effectiveness of treatment options could "succeed in improving health care while paying for its own research-related costs through targeted health system cost reductions."[72] In estimating the effects of section 904, CBO assumed that the annual federal savings on health care would eventually reach a point at which they roughly equaled the annual outlays for research on comparative effectiveness—a process that would take about a decade.

Under H.R. 3162, budget authority for the Center for Comparative Effectiveness Research would be $1.1 billion over the 2008–2012 period and $2.9 billion over the 2008–2017 period. Because spending those funds would take some time, CBO estimates that outlays would amount to about $600 million over five years and $2.4 billion over 10 years. Direct spending by the federal government—mostly for Medicare and Medicaid—would be reduced by $0.1 billion over the 2008–2012 period and $1.3 billion over the 2008–2017 period. (Those amounts would constitute a very small fraction of cumulative federal outlays for those programs—less than one one-hundredth of 1 percent.) Thus, the net effect of enacting section 904 would be to increase federal direct spending by $0.5 billion over five years and $1.1 billion over 10 years, CBO estimates.

The impact on total spending on health care in the United States would be about five times as large as the effect on federal outlays, CBO estimates. Some of that effect would be seen in lower costs for providing health insurance to workers—costs that are excluded from income and payroll taxes. In turn, some of those savings on private insurance premiums would go to increase the taxable compensation of workers; by itself, that change would lead to a small increase in expected federal revenues. At the same time, the new fees on health insurance that would be used to finance the research would generate corresponding increases in health care costs for workers, which would tend to reduce taxable compensation modestly. Overall, those indirect effects of section 904 on revenues would be small.

72. Office of Technology Assessment, *Identifying Health Technologies that Work: Searching for Evidence*, p. 6.

CONGRESS OF THE UNITED STATES
CONGRESSIONAL BUDGET OFFICE
WASHINGTON, DC 20515

INSIDE MAIL

CER Work Group

July 2, 2009

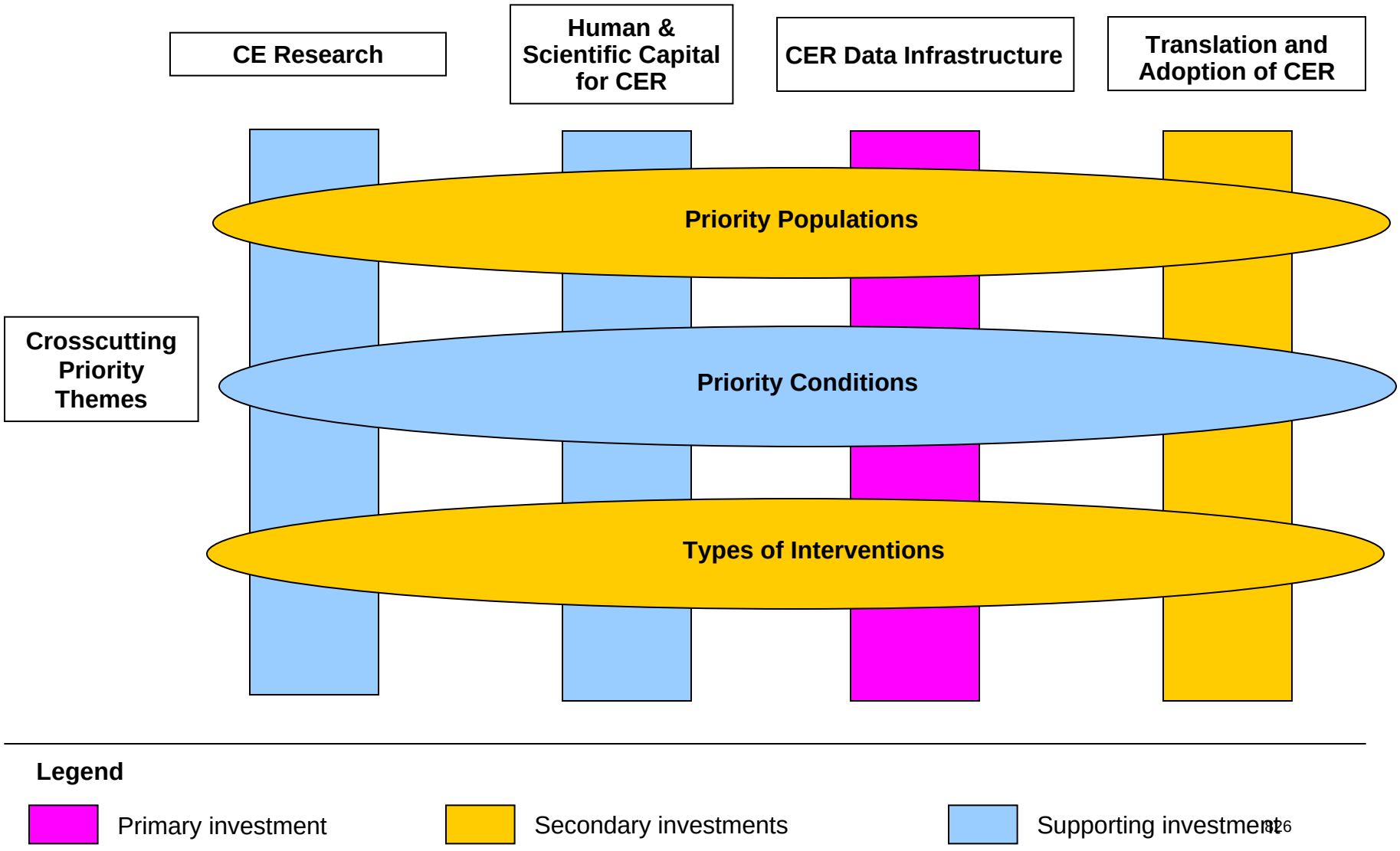
AGENDA

- Review several new proposals
- Discuss Potential Subcategories of strategic framework for evaluation
- Survey Results
- Discuss individual/groups of proposals
  - By evaluation criteria
  - General

New Proposals

- Patient-centered dissemination strategies
- Provider-centered dissemination strategies
- Expand Chronic Care Model networks and create registry for patients with multiple chronic conditions
- Creation of all-payer, all-claims database

Strategic Framework



Subcategories for Investment

- Infrastructure
  - Longitudinal claims data
  - Distributed Data Networks
  - Patient Registries
  - Surveys
- Dissemination and Translation
  - Federal delivery system
  - Non-Federal delivery system

Subcategories for Investment

- Interventions
  - Medications
  - Medical and Assistive Devices
  - Procedures and Surgeries
  - Diagnostic Testing
  - Behavioral Change
  - Delivery System Strategies
  - Prevention Strategies

Infrastructure

- LONGITUDINAL CLAIMS DATABASE-Research database that links claim data for single patients over a long period of time
- DISTRIBUTED DATA NETWORK-funding for patient registry networks, health information exchanges, and clinical EHR data networks for CER purposes
- PATIENT REGISTRY-databases that collect clinical data on patients with a specific disease or on a specific test or procedure
- SURVEYS-large scale surveys of patients or providers about health or health system related topics

Dissemination and Translation

- FEDERAL PROVIDER SYSTEMS-Support implementation of CER findings into practice in provider systems run by the Federal government, e.g., DoD, VA, IHS

- NON-FEDERAL PROVIDER SYSTEMS-Support communication, translation, and dissemination of CER to consumer, providers, and other decision makers outside of the Federal system

Interventions

-MEDICAL AND ASSISTIVE DEVICES (e.g., comparing rehabilitative devices)

-PROCEDURES AND SURGERIES (e.g., evaluating surgical options or surgery versus medical management)

-MEDICATIONS (e.g., comparing the effectiveness of 2 drugs from different classes on a specific disease)

-DIAGNOSTIC TESTING (e.g. comparing imaging modalities for evaluating certain types of cancer)

-BEHAVIORAL CHANGE (e.g., developing and assessing smoking cessation programs)

-DELIVERY SYSTEM STRATEGIES (e.g., testing two different discharge process care models on readmission rates

-PREVENTION (e.g., comparing two interventions to prevent or decrease obesity)

Minimum Threshold Criteria

- Included within statutory limits of Recovery Act and the Council's definition of CER
- Potential to inform decision-making by patients, clinicians, and other stakeholders
- Responsiveness to expressed needs of patients, clinicians, and other stakeholders
- Feasibility of research topic (including time necessary for research)

Prioritization Criteria

- Potential impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm)
- Potential to evaluate comparative effectiveness in diverse populations and patient sub-groups and engage communities in research
- Uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- Addresses need or gap unlikely to be addressed through other organizations
- Potential for multiplicative effect (e.g. lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government)

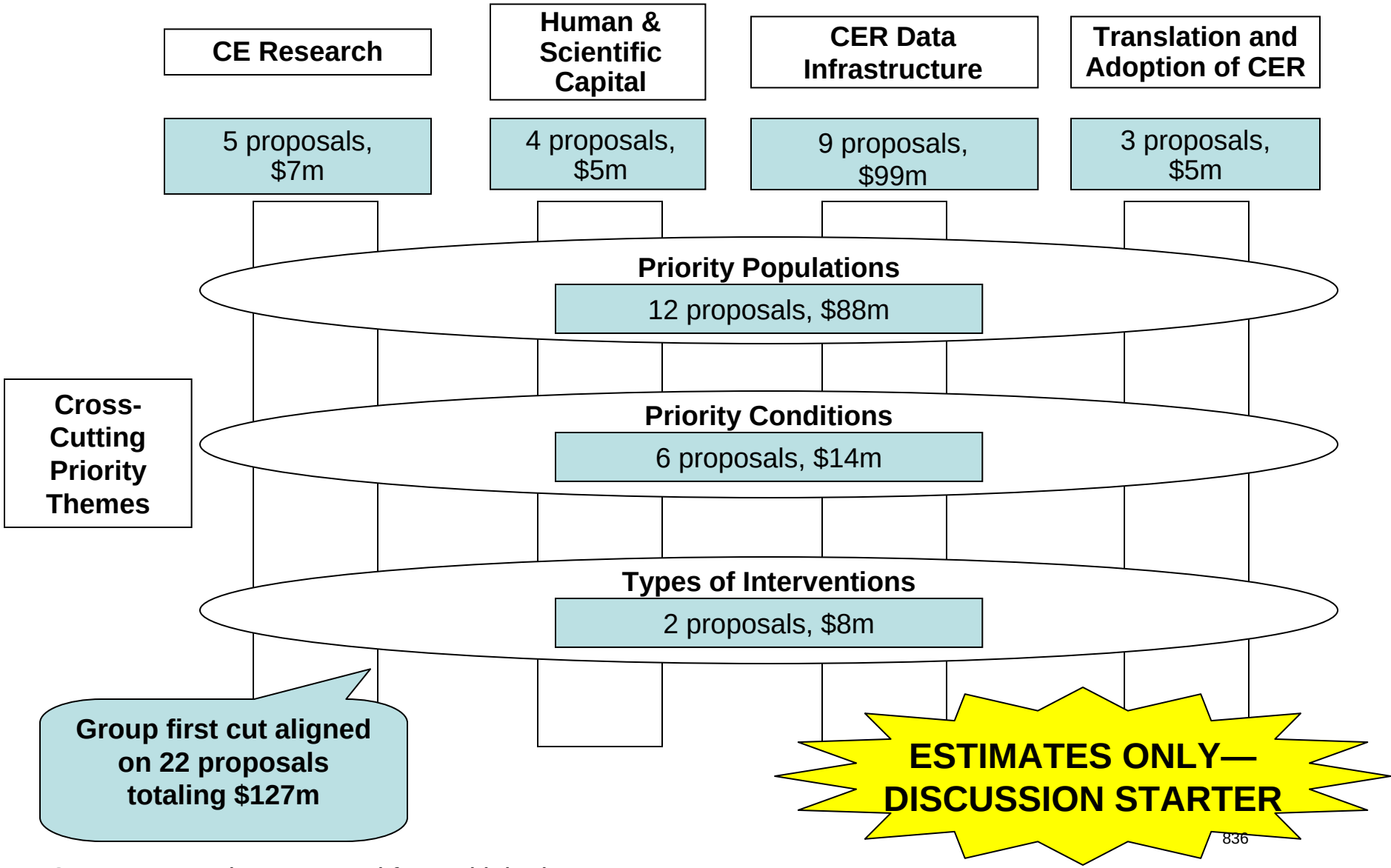
Potential Criteria for Overall Portfolio

- Balance across the strategic framework
- Balance of time to impact (short term vs. long term gains)
- Cost
- Balance of mechanisms (e.g. contracts, grants, public-private partnerships)
- Number of FTEs

Survey results summary

- 25 complete responses
- Group believes all 4 types of infrastructure investments are important, with maximum funding going towards longitudinal claims databases
- Efforts in translation and dissemination should be balanced across Federal and non-Federal provider systems, being sure to include public health providers in both groups
- The group agreed with the Council's recommendations that devices, procedures, diagnostics, behavioral change, delivery systems strategies, and prevention were the top priority types of interventions
- Majority agreed that 22 projects totaling ~$130m should receive requested funding or more than the requested funding
- Projects selected roughly correlate with Council recommendations, though relatively fewer investments in translation and more in research
- New proposals for this week and next address results of subcategory prioritization

Summary of "survey portfolio"



Note: Some proposals accounted for multiple times

Survey portfolio

Linked HHS longitudinal claims and clinical data sets
CER research standards
Clinicaltrials.gov expansion
HHS Summit meeting
Longitudinal ICD registry
Enhance availability and use of Medicare data to support CER
Medicaid analytic extract (MAX) data repository for CHIP and Medicaid populations
Data mining efforts
Centers of Excellence for Cultural and Linguistic Competency in Healthcare
Integration of Primary Care and Behavioral Health Services for Racial and Ethnic Minority Populations, Technical Assistance Center
Supporting Dissemination and Use of Behavioral Health Comparative Effectiveness (CE) Research in "Real World" Settings
Pediatric Research in Office Settings (PROS)
Depression Intervention and Comparative Effectiveness

Depression Intervention and Comparative Effectiveness
Expanding and Evaluating the Health Information Technology to Improve Care within the IHS
Using HIT to Examine HIS Medical and Pharmacy Utilization and Related Costs for Diabetes and other Co-Morbidities
Comparative Effectiveness of combinations of therapies for treating diabetes and associated co-morbidities in AIAN.
Comparing Effectiveness and Costs of Intervention models to Prevent and Treat Diabetes within Indian Communities
Advanced Pharmacy Practice Model
Compare effectiveness of locally applied models of health epidemiology in American Indian and Alaska Native communities
Develop a toolkit for comparing effectiveness of new models for managing and caring for patients with chronic health conditions
Developmental activities in support of a strategy for expanding CER
Clinically Enhanced State Data for Analysis and Tracking of Comparative Effectiveness Impact

Group's "Pick 5" portfolio (number of votes)

Group believes this proposal should request more funds—how much more? What should they go towards?

- Linked HHS longitudinal claims (11)
- Enhance availability for Medicare data to support CER (8)
- Medicaid MAX expansion (8)
- Distributed data networks (7)
- Surveys measuring impact on provider practice (6)

Proposals with a plurality of “Do not fund” recommendations

- Orthopedic joint registry (42% DNF)
- HIV/AIDS Oral Health (42% DNF)
- Developmental activities in support of a CER strategy (35%)
- Reducing Oral Health Disparities (47%)

Group was strongly divided on this proposal

How can we strengthen these proposals? What do they need to get over the line?

Proposals to fund for “less than the requested amount”

•10. Partnership in applied CE science for medical products (PACES) ($13)

•12. Accelerating Adoption of CER results by Providers & Patients ($45)

•13. Centers of Excellence for Cultural and Linguistic Competency in Healthcare ($5.5)

•14. Integration of Primary Care and Behavioral Health Services for Racial and Ethnic Minority Populations, Technical Assistance Center ($4)

•15. Use of Public Health Outreach Workers (PHOW) to Improve Clinical Outcomes. ($6.5)

•17. Effective Use of Regionalized Emergency Care Delivery ($2)

•18. Pediatric Emergency Care Applied Research Network (PECARN) ($8.8)

•21. The Health Center Consortia and Comparative Effectiveness ($4)

•22. Health Center Controlled Networks: Infrastructure Models for CER ($7.9)

•23. ADAP HIV/AIDS Drug Registry and HIV Care Models and CAREWare ($6.8)

•34. Informing clinical and public health approaches to chronic disease prevention using the foundation and the network of the prevention research centers (PRCs) ($20)

•35. Performing high-quality new synthesis of CER through the Guide to Community Preventive Services ($30)

•37. Assessing and Accelerating Implemenation Stragegies in AHRQ Networks ($17.8)

•40. Surveys Measuring the Impact of Comparative Effectiveness Research Findings on Medical Provider Practice and Patient Outcomes ($30.1)

Why should these proposals receive less funding? What is the right size? What should be cut?

BACKUP-Raw survey results

Evaluate each of the following subcategories for CER Data Infrastructure, 1 being should not receive funding, 6 being should receive maximal funding.

Answer Options	1	2	3	4	5	6	Response Count
Longitudinal claims databases	0	2	3	2	6	12	25
Distributed data networks	0	2	3	4	10	6	25
Patient registries	0	1	4	5	8	6	24
Surveys	4	3	1	7	6	1	22
Other	0	0	0	0	0	1	1

Evaluate each of the following subcategories for CER Translation and Dissemination, 1 being should not receive funding, 6 being should receive maximum possible funding.

Answer Options	1	2	3	4	5	6	Response Count
Federal provider systems	0	2	1	4	8	9	24
Non-federal provider systems	0	1	3	5	7	7	23
Other	0	0	0	1	0	0	1

Evaluate each of the following subcategories for Types of Interventions, 1 being should not receive funding, 6 being should receive maximum possible funding.

Answer Options	1	2	3	4	5	6	Response Count
Drugs	1	1	2	8	5	8	25
Medical and assistive devices	0	1	6	5	7	6	25
Procedures and surgeries	0	0	2	7	7	9	25
Diagnostic testing	0	1	1	7	10	6	25
Behavioral change	0	0	3	4	10	6	23
Delivery system strategies	0	0	4	2	7	11	24
Prevention	0	2	1	3	5	14	25
Other	0	0	0	0	0	0	0

Answer Options	Do not fund	Fund for less than the requested amount	Fund for the requested amount	Fund for greater that the requested amount	Response Count
1. Distributed clinical data networks ($40)	0	9	8	2	19
2. Linked HHS longitudinal claims and clinical data sets	1	2	12	4	19
3. CER research standards ($2)	2	5	9	3	19
4. Clinicaltrials.gov expansion ($2)	0	6	10	3	19
5. HHS Summit meeting ($0.3)	4	4	6	4	18
6. Orthopedic joint registry ($4)	8	5	3	3	19
7. Longitudinal ICD registry ($4)	2	4	10	3	19
8. Enhance availability and use of Medicare data to	1	5	6	9	21
9. Medicaid analytic extract (MAX) data repository for	0	7	9	5	21
10. Partnership in applied CE science for medical	3	9	7	0	19
11. Data mining efforts ($4.5)	2	5	7	3	17
12. Accelerating Adoption of CER results by Providers &	0	13	3	3	19
13. Centers of Excellence for Cultural and Linguistic	6	6	6	1	19
14. Integration of Primary Care and Behavioral Health	4	6	8	1	19
15. Use of Public Health Outreach Workers (PHOW) to	3	11	5	0	19
16. Supporting Dissemination and Use of Behavioral	2	7	9	1	19
17. Effective Use of Regionalized Emergency Care	3	9	6	1	19
18. Pediatric Emergency Care Applied Research	1	11	6	1	19
19. Pediatric Research in Office Settings (PROS) ($1.9)	2	7	9	1	19
20. Depression Intervention and Comparative	3	5	11	0	19
21. The Health Center Consortia and Comparative	4	9	6	0	19
22. Health Center Controlled Networks: Infrastructure	4	9	5	0	18
23. ADAP HIV/AIDS Drug Registry and HIV Care Models	6	7	5	0	18
24. HIV/AIDS Oral Health ($0.5)	8	5	3	3	19
25. Expanding and Evaluating the Health Information	2	3	14	0	19
26. Using HIT to Examine HIS Medical and Pharmacy	4	5	10	0	19
27. Comparative Effectiveness of combinations of	1	4	11	3	19
28. Comparing Effectiveness and Costs of Intervention	1	4	11	3	19
29. Advanced Pharmacy Practice Model ($4)	5	4	10	0	19
30. Compare effectiveness of locally applied models of	3	6	8	2	19
31. Develop a toolkit for comparing effectiveness of	2	2	12	3	19
32. Enhancing clinicaltrials.gov ($4)	4	5	7	3	19
33. Developmental activities in support of a strategy for	7	3	7	3	20
34. Informing clinical and public health approaches to	2	12	4	2	20
35. Performing high-quality new synthesis of CER	6	8	5	1	20
36. Reducing Oral Health Disparities:Fluoride and	9	3	5	2	19
37. Assessing and Accelerating Implemenation	1	10	8	0	19
38. Clinically Enhanced State Data for Analysis and	0	9	9	1	19
39. Clinicall Enhanced All Payer All Claims (APAC) Data	0	9	9	1	19
40. Surveys Measuring the Impact of Comparative	4	9	5	2	20

Please select the five proposals you believe are most deserving of funding.

Answer Options	Response Percent	Response Count
1. Distributed clinical data networks	29.2%	7
2. Linked HHS longitudinal claims and clinical data sets	50.0%	12
3. CER research standards	12.5%	3
4. Clinicaltrials.gov expansion	16.7%	4
5. HHS Summit meeting	8.3%	2
6. Orthopedic joint registry	12.5%	3
7. Longitudinal ICD registry	12.5%	3
8. Enhance availability and use of Medicare data to	33.3%	8
9. Medicaid analytic extract (MAX) data repository for	33.3%	8
10. Partnership in applied CE science for medical	4.2%	1
11. Data mining efforts	16.7%	4
12. Accelerating Adoption of CER results by Providers &	12.5%	3
13. Centers of Excellence for Cultural and Linguistic	12.5%	3
14. Integration of Primary Care and Behavioral Health	16.7%	4
15. Use of Public Health Outreach Workers (PHOW) to	16.7%	4
16. Supporting Dissemination and Use of Behavioral	12.5%	3
17. Effective Use of Regionalized Emergency Care	4.2%	1
18. Pediatric Emergency Care Applied Research	4.2%	1
19. Pediatric Research in Office Settings (PROS)	4.2%	1
20. Depression Intervention and Comparative	8.3%	2
21. The Health Center Consortia and Comparative	4.2%	1
22. Health Center Controlled Networks: Infrastructure	4.2%	1
23. ADAP HIV/AIDS Drug Registry and HIV Care Models	0.0%	0
24. HIV/AIDS Oral Health	4.2%	1
25. Expanding and Evaluating the Health Information	0.0%	0
26. Using HIT to Examine HIS Medical and Pharmacy	0.0%	0
27. Comparative Effectiveness of combinations of	4.2%	1
28. Comparing Effectiveness and Costs of Intervention	4.2%	1
29. Advanced Pharmacy Practice Model	4.2%	1
30. Compare effectiveness of locally applied models of	0.0%	0
31. Develop a toolkit for comparing effectiveness of	4.2%	1
32. Enhancing clinicaltrials.gov	8.3%	2
33. Developmental activities in support of a strategy for	4.2%	1
34. Informing clinical and public health approaches to	12.5%	3
35. Performing high-quality new synthesis of CER	12.5%	3
36. Reducing Oral Health Disparities:Fluoride and	12.5%	3
37. Assessing and Accelerating Implemenation	16.7%	4
38. Clinically Enhanced State Data for Analysis and	4.2%	1
39. Clinicall Enhanced All Payer All Claims (APAC) Data	4.2%	1
40. Surveys Measuring the Impact of Comparative	29.2%	7

FEDERAL COORDINATING COUNCIL FOR
COMPARATIVE EFFECTIVENESS RESEARCH

REPORT TO THE PRESIDENT AND THE CONGRESS

JUNE 30, 2009

US DEPARTMENT OF HEALTH AND HUMAN SERVICES

Federal Coordinating Council for Comparative Effectiveness Research

Report to the President and the Congress

June 30, 2009

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EXECUTIVE SUMMARY

Across the United States, clinicians and patients confront important health care decisions without adequate information. What is the best pain management regimen for disabling arthritis in an elderly African-American woman with heart disease? For neurologically impaired children with special health care needs, what care coordination approach is most effective at preventing hospital readmissions? What treatments are most beneficial for patients with depression who have other medical illnesses? Can physicians tailor therapy to specific groups of patients using their history or special diagnostic tests? What interventions work best to prevent obesity or tobacco use? Unfortunately, the answer to these types of comparative, patient-centered questions in health care is often, "We don't really know."

Thousands of health care decisions are made daily; patient-centered comparative effectiveness research focuses on filling gaps in evidence needed by clinicians and patients to make informed decisions. Physicians and other clinicians see patients every day with common ailments, and they sometimes are unsure of the best treatment because limited or no evidence comparing treatment options for the condition exists. As a result, patients seen by different clinicians may get different treatments and unknowingly be receiving less effective care. Patients and their caregivers search in vain on the Internet or elsewhere for evidence to help guide their decisions. They often fail to find this information either because it does not exist or because it has never been collected and synthesized to inform patients and/or their caregivers in patient-friendly language. When they do find information, it may be informed by marketing objectives, not the best evidence.[1]

Due to astonishing achievements in biomedical science, clinicians and patients often have a plethora of choices when making decisions about diagnosis, treatment, and prevention, but it is frequently unclear which therapeutic choice works best for whom, when, and in what circumstances. The purpose of comparative effectiveness research (CER) is to provide information that helps clinicians and patients choose which option best fits an individual patient's needs and preferences. It also can inform the health choices of those Americans who cannot or choose not to access the health care system.[2] Clinicians and patients need to know not only that a treatment works on average but also which interventions work best for specific types of patients (e.g. the elderly, racial and ethnic minorities). Policy makers and public health professionals need to know what approaches work to address the prevention needs of those Americans who do not access health care. This information is essential to translating new discoveries into better health outcomes for Americans, accelerating the application of beneficial innovations, and delivering the right treatment to the right patient at the right time.

Examples of successful CER include summaries of evidence from the Agency for Healthcare Research and Quality (AHRQ) on numerous conditions, such as prostate cancer and osteoporosis, as well as the National Institutes of Health (NIH) diabetes prevention trial that demonstrated lifestyle change was superior to metformin and placebo in preventing onset of type 2 diabetes. Additionally, the Veterans Affairs (VA) COURAGE trial demonstrated that patients treated with optimal medical therapy alone did just as well as patients who received percutaneous coronary intervention plus medical therapy in preventing heart attack and death. These exemplars show the power of CER to inform patient and clinician decisions and improve health outcomes.

Patients increasingly and appropriately want to take responsibility for their care. Therefore we have a responsibility to provide comparative information to enable informed decision-making. This patient-

[1] Lee TH, Brennan TA. N Engl J Med. 2002;346:529-531.
[2] Green LA, et al. N Engl J Med. 2001; 344:2021-5.

centered, pragmatic, "real world" research is a fundamental requirement for improving care for all Americans.

Comparative effectiveness differs from efficacy research because it is ultimately applicable to real-world needs and decisions faced by patients, clinicians, and other decision makers. In efficacy research, such as a drug trial for the U.S. Food and Drug Administration (FDA) approval, the question is typically whether the treatment is efficacious under ideal, rather than real-world, settings. The results of such studies are therefore not necessarily generalizable to any given patient or situation. But what patients and clinicians often need to know in practice is which treatment is the best choice for a particular patient. In this way, comparative effectiveness is much more patient-centered. Comparative effectiveness has even been called patient-centered health research or patient-centered outcomes research to illustrate its focus on patient needs.

The American Recovery and Reinvestment Act (ARRA) provided $1.1 billion for comparative effectiveness research. The Act allocated $400 million to the Office of the Secretary in the U.S. Department of Health and Human Services (HHS), $400 million to the National Institutes of Health (NIH), and $300 million to the HHS Agency for Healthcare Research and Quality. It also established the Federal Coordinating Council for Comparative Effectiveness Research (the Council) to foster optimum coordination of CER conducted or supported by Federal departments and agencies. Furthermore, the legislation indicated that "the Council shall submit to the President and the Congress a report containing information describing current Federal activities on comparative effectiveness research and recommendations for such research conducted or supported from funds made available for allotment by the Secretary for comparative effectiveness research in this Act" by June 30, 2009.

Transparent, Open Process Seeking Public Input

From the outset, the Council recognized the importance of establishing a transparent, collaborative process for making recommendations and sought the input of the American people on this important topic. The Council held three public listening sessions, two in the District of Columbia and one in Chicago. The Council also received comments for two months on its public Web site. Importantly, the open process allowed the Council to hear from hundreds of diverse stakeholders who represent views across the spectrum. Many patients expressed their need for this type of research; one of the most emotional and moving testimonies came from the mother of a child with a seizure disorder in Chicago who had struggled to find the best treatment for her child. A physician from the American Board of Orthopedics summarized many physicians' testimony by saying, "developing high quality, objective information will improve informed patient choice, shared decision-making, and the clinical effectiveness of physician treatment recommendations." The Council heard repeatedly at the listening sessions that the Federal Government must use this investment to lay the foundation for informing decisions and improving the quality of health care. In addition, the Council posted interim working documents for feedback, including the definition of CER, the prioritization criteria, and the strategic framework, and modified these based on the feedback. Comments from the listening sessions and via the Web site significantly influenced Council discussion and decisions. Indeed, this entire report is influenced by the public input—and Appendix A elaborates on the key themes that ran through the public comments.

Vision

The Council's vision for the investment in comparative effectiveness research focuses on laying the foundation for this type of research to develop and prosper so it can inform decisions by patients and clinicians. This research is critical to transforming our health care system to deliver higher quality and more value to all Americans. The Council specifically focused on recommendations for use of the

Office of Secretary (OS) funds to fill high priority gaps that were less likely to be funded by other organizations and therefore represent unique opportunities for these funds.

Early in the process, the Council set the following objectives consistent with ARRA:

1. Develop a definition, establish prioritization criteria, create a strategic framework, and identify priorities that lay the foundation for CER.

2. Foster optimum coordination of comparative effectiveness research conducted or supported by relevant Federal departments.

3. Formulate recommendations for investing the $400 million appropriated to the HHS Office of Secretary as part of this Report to Congress.

Definition and Criteria
The Council first established a definition, building on previous definitions, for comparative effectiveness research:

> Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in "real world" settings. The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.
>
> - To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations and sub-groups.
> - Defined interventions compared may include medications, procedures, medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies.
> - This research necessitates the development, expansion, and use of a variety of data sources and methods to assess comparative effectiveness and actively disseminate the results.

The Council needed explicit criteria to make recommendations for priorities. Therefore, the Council's second step was to establish minimum threshold criteria that must be met and prioritization criteria.

Minimum Threshold Criteria (i.e. must meet these to be considered):

- Included within statutory limits of Recovery Act and the Council's definition of CER
- Potential to inform decision-making by patients, clinicians, or other stakeholders
- Responsiveness to expressed needs of patients, clinicians, or other stakeholders
- Feasibility of research topic (including time necessary for research)

The prioritization criteria for scientifically meritorious research and investments are:

- Potential impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm)

- Potential to evaluate comparative effectiveness in diverse populations and patient sub-groups and engage communities in research
- Uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- Addresses need or gap unlikely to be addressed through other organizations
- Potential for multiplicative effect (e.g. lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government)

Importance of Priority Populations and Patient Sub-Groups
One important consideration for comparative effectiveness research is addressing the needs of priority populations and sub-groups, i.e., those often underrepresented in research. The priority populations specifically include, but are not limited to, racial and ethnic minorities, persons with disabilities, children, the elderly, and patients with multiple chronic conditions. These groups have been traditionally under-represented in medical research.

In addition, comparative effectiveness should complement the trend in medicine to develop personalized medicine—the ability to customize a drug and dose based on individual patient and disease characteristics. One of the advantages of large comparative effectiveness studies is the power to investigate effects at the sub-group level that often cannot be determined in a randomized trial. This power needs to be harnessed so personalized medicine and comparative effectiveness complement each other.

Strategic Framework
After completing the draft definition and criteria for prioritization of potential CER investments, the Council recognized the need to develop a strategic framework for CER activity and investments to categorize current activity, identify gaps, and inform decisions on high-priority recommendations. This framework represents a comprehensive, coordinated approach to CER priorities. It is intended to support immediate decisions for investment in CER priorities and to provide a comprehensive foundation for longer-term strategic decisions on CER priorities and the related infrastructure. At the framework's core is responsiveness to expressed needs for comparative effectiveness research to inform health care decision-making by patients, clinicians, and others in the clinical and public health communities.

Types of CER investments and activities can be grouped into four major categories:

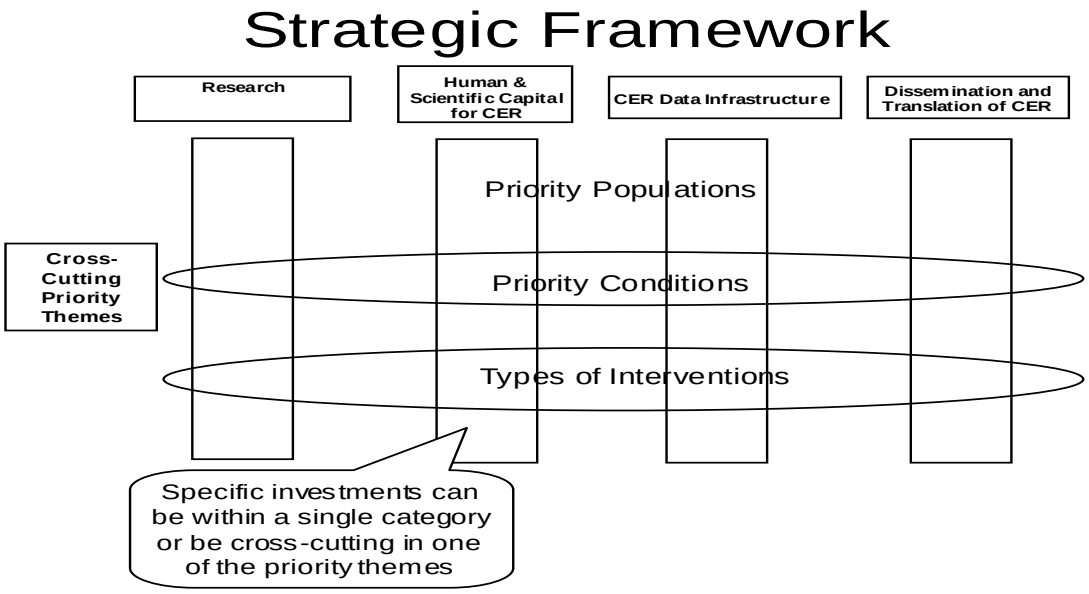
- **Research** (e.g., comparing medicines for a specific condition or discharge process A to discharge process B for readmissions)
- **Human and Scientific Capital** (e.g., training new researchers to conduct CER, developing CER methodology)
- **CER Data Infrastructure** (e.g., developing a distributed practice-based data network, longitudinal linked administrative or Electronic Health Record (EHR) databases, or patient registries)
- **Dissemination and Translation of CER** (e.g., building tools and methods to disseminate CER findings to clinicians and patients and translate CER into practice)

Furthermore, investments or activities related to a specific theme can cut across one or more categories and may include research, human and scientific capital, CER data infrastructure, and/or translation and adoption. These themes could include:

- **Conditions** (e.g., cancer, heart failure)
- **Patient populations** (e.g., elderly, minorities, children, persons with disabilities)
- **Type of intervention** (e.g., devices, behavioral change, delivery system)

Together, these activities and themes make up the "CER Strategic Framework" (Figure A)

Figure A



CER Inventory and Priority-Setting Process

The Council also conducted an inventory of CER and data infrastructure to help identify gaps in the current CER landscape. Maintaining that inventory and ongoing evaluation of government and private sector (where possible) CER investments and programs across these activities and themes is critical to this framework's value for decision-making. The first draft Federal Government inventory of CER and data infrastructure is included in this report, but it is critical to note that evaluation of current activities and the identification of gaps in order to inform priority-setting must be iterative and continue in the future.

As noted above, the Council's priority-setting process was informed by public input, and that input had a substantial influence on how the Council formulated its framework and priorities for CER. CER is an important mechanism to improve health and continued public input is vital for agenda setting.

Priority Recommendations

In developing its recommendations for how to invest the OS ARRA funding of $400 million, the Council sought to respond to patient and physician needs for CER, to balance achieving near-term results with building longer-term opportunities, and to capture the unique value that the Secretary's ARRA funds could play in filling gaps and building the foundation for future CER. The Council recommended that, among the four major activities and three cross-cutting themes in the CER

framework, the primary investment for this funding should be data infrastructure. Data infrastructure could include linking current data sources to enable answering CER questions, development of distributed electronic data networks and patient registries, and partnerships with the private sector.

Secondary areas of investment are dissemination and translation of CER findings, priority populations, and priority types of interventions. The priority populations identified that could be the focus of cross-cutting themes were racial and ethnic minorities, persons with disabilities, persons with multiple chronic conditions (including co-existing mental illness), the elderly, and children. CER will be an important tool to inform decisions for these populations and reduce health disparities. High-priority interventions for OS to consider supporting include medical and assistive devices, procedures/surgery, behavioral change, prevention, and delivery systems. For example, behavioral change and prevention have the potential to decrease obesity, decrease smoking rates, increase adherence to medical therapies, and improve many other factors that determine health. Delivery system interventions, such as comparing different discharge and transitions of care processes on hospital readmissions, community-based care models, or testing the effect of different medical home models on health have substantial potential to drive better health outcomes for patients.

The OS funds may also play a supporting role in research and human and scientific capital. Because the Council anticipates that AHRQ, NIH, and VA will likely continue to play a major role in these essential activities for the CER enterprise, OS funding would likely only fill gaps in these areas.

Longer-Term Outlook and Next Steps
This report and an Institute of Medicine report funded by the Department will inform the priority-setting process for CER-related funding. The most immediate next step will be the development of a specific plan, to be submitted by July 30, 2009, from the Secretary of Health and Human Services for the combined $1.1 billion of ARRA CER funding. In addition, an annual report from the Council is required under the ARRA legislation.

It will be important for this funding both to accomplish short-term successes and to build the foundation for future CER. The CER activity and investments should be coordinated across the Federal Government and avoid duplicative effort. In addition, the funding should complement and link to activities and funding in the private sector to maximize the benefits to the American people.

Clinicians, patients, and other stakeholders greatly need comparative effectiveness research to inform health care decisions. One private citizen unaffiliated with any health care group summarized, “It is more important than ever to engage in robust research on what treatments work and what do not. Doing so empowers doctors and patients, and helps make our practice of medicine more evidence-based.”

This is a unique opportunity to invest in the fundamental building blocks for transformation of health care in the United States to improve the quality and value of health care for all Americans. Physicians and patients deserve the best patient-centered evidence on what works, so Americans can have the highest quality care and achieve the best possible outcomes.

I. INTRODUCTION

The American Recovery and Reinvestment Act of 2009 (ARRA), Pub. L. 111-5, made available to the Department of Health and Human Services $1.1 billion for comparative effectiveness research (CER). Of this amount, $300 million was allocated to the Agency for Healthcare Research and Quality (AHRQ), $400 million to the National Institutes of Health (NIH), and $400 million was allocated to the Office of the Secretary (OS) for disbursement.

These and all Federal agencies distributing ARRA funds must do so in accordance with all nondiscrimination and equal opportunity statutes, regulations, and Executive Orders that apply to the distribution of funds under the Recovery Act. Agencies that grant funds also must ensure that their recipients comply with Title VI of the Civil Rights Act of 1964 (prohibiting race, color, and national origin discrimination), Section 504 of the Rehabilitation Act of 1973 (prohibiting disability discrimination), Title IX of the Education Amendments of 1972 (prohibiting sex discrimination in education and training programs), the Age Discrimination Act of 1975 (prohibiting age discrimination in the provision of services), and a variety of program-specific statues with nondiscrimination requirements.[3]

ARRA provides further guidance on how funds appropriated to the Office of the Secretary are to be allocated:

> … the funding appropriated in this paragraph shall be used to accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that: (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions; and (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data.

Section 804 of ARRA authorizes the establishment of the Federal Coordinating Council for Comparative Effectiveness Research (the Council). The Council is composed of senior Federal officials with responsibility for health-related programs. Most of the members are physicians and many have research expertise. The members represent not only the Department of Health and Human Services but also the Department of Veterans Affairs and the Department of Defense. Members of the Council come from a broad range of backgrounds, including the Office of Minority Health, the Office on Disability, community health centers, mental health, HIV and other infectious diseases, prevention, and others. The Council's purpose is to coordinate comparative effectiveness research and related health services research across the Federal Government with the intent of reducing duplication and encouraging the complementary use of resources. The Council is also charged with advising the President and Congress on strategies to address the infrastructure needs for CER within the Federal Government and organizational expenditures for CER by relevant Federal Departments and agencies.

[3] Memorandum from the Acting Assistant Attorney General for Civil Rights. 4 March 2009

The 15-member Council was announced by HHS via website on March 19, 2009, and has been meeting regularly since then.[4] One of the Council's responsibilities is to submit to the President and Congress an initial report describing current Federal activities on comparative effectiveness research and recommendations for CER conducted or otherwise supported from the $400 million made available for CER to be allocated by the Secretary. This report meets that requirement.

Rationale for Comparative Effectiveness Research

When patients ask clinicians about the evidence supporting one treatment choice, diagnostic plan, or prevention modality over another, the answer too often is that the evidence is unclear. Even when evidence exists, it is often from a trial that may not apply to the specific patient and/or situation under consideration, such as an elderly African-American woman with multiple comorbidities. When specific evidence is lacking, clinicians have to rely on their clinical experience to make the best treatment decisions possible. Nevertheless, these decisions can result in less than optimal, and sometimes inappropriate, treatment choices.

Due largely to government and scientific leadership accompanied by astonishing achievements in biomedical science, clinicians and patients often have a plethora of choices when making decisions about diagnosis, treatment, and prevention. Total investment in health services research, which includes CER, accounts for only 1.5 percent of medical research expenditures.[5] The Recovery Act greatly increased funding for CER and the prominence and important of such research. The purpose of CER is to provide information that helps clinicians and patients choose which option best fits an individual patient's needs and preferences. The amazing biomedical discoveries made in the United States to date can now support CER to routinely compare commonly used therapies or test which interventions work best for particular patients. This information is essential to translate new discoveries into better health outcomes for Americans.[6] We must generate this knowledge to be able to deliver the right treatment to the right patient at the right time. Patients increasingly and appropriately want to take responsibility for their care; therefore, we have an obligation to provide the comparative information that enables informed decisions.

No standardized Federal definition of comparative effectiveness research existed prior to the Council's definition. However, several government entities had developed individual definitions for CER. For example, the Congressional Budget Office has described comparative effectiveness research as "rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients." The Institute of Medicine refers to comparative effectiveness as "the extent to which a specific intervention, procedure, regimen, or service does what it is intended to do when it is used under real world circumstances." The Council's definition builds on these concepts and highlights key aspects of the ARRA CER provisions. The Council defined CER broadly, asserting that it is patient-centered, "real world" research that can help patients, clinicians, and other decision makers

[4] See Appendix D for Council membership.
[5] Moses III H, Dorsey EK, Matheson DHM, et al. Financial Anatomy of Biomedical Research. JAMA 2005; 294:1333-42
[6] Dougherty, D, Conway PH. The "3 T's" Roadmap to Transform U.S. Health Care: The "How" of High Quality Care. JAMA. 2008 May 21;299(19):2319-21

assess the relative benefits and harms of strategies to prevent, diagnose, treat, manage, or monitor health conditions and the systems in which they are made.[7] This definition will form the foundation of the common Federal definition.

The Department of Health and Human Services' ARRA appropriation for CER is a significant investment. CER and activities that support CER have been undertaken by a wide range of stakeholders both inside and outside the public sector. However, despite diverse activities across the Federal Government,[8] funds exclusively appropriated for CER have until now been funded under authorized by section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 for the Agency for Healthcare Research and Quality, which the Agency makes available for projects through its Effective Health Care Program. Since 2005, Congress has appropriated a total of $125 million for the program, including $50 million for comparative effectiveness in FY 2009.

The ARRA funding reflects the heightened interest in CER among the nation's clinicians, patients, policy makers and researchers and broader recognition of its potential to improve outcomes that matter to patients, including morbidity, mortality, and quality of life. CER has the ability to assess these very patient-centered outcomes in a comprehensive way. Furthermore, patients increasingly play an active role in their health care and expect to be active participants in decisions about their health care. These interests are rooted in the strong desire for better evidence upon which to make clinical and other health-related decisions at a time of heightened focus on the quality and variability of care delivered.

A health system guided by better information about "what works" would have benefits for all who have a stake in the nation's health system. Consumers and patients would develop more confidence that the increasingly complex array of treatments and interventions could be tailored to meet their individual needs; health professionals would have more certainty that their clinical decisions were evidence-based and serving patients well. Consequences of the lack of such information include wide geographic variations in treatments typically received for specific conditions and, with these variations, sizeable differences in related health care spending not accompanied by proportional differences in outcomes.

Noted medical author Dr. Atul Gawande recently summarized this issue, "In situations where the right thing to do is well established, physicians from high- and low-cost cities make the same decisions. But in cases where the science is more unclear, some physicians pursue the maximum possible amount of testing and procedures; some pursue the minimum. And what kind of doctor they are depends on where they came from. In case after uncertain case, more was not necessarily better… We will need to do in-depth research on what makes the best systems successful… and disseminate what we learn. Congress has provided vital funding for research that compares the effectiveness of different treatments, and this should help reduce uncertainty about which treatments are best. But we also need to fund research that compares the effectiveness of different systems of care—to reduce our uncertainty about which systems work best for communities. These are empirical, not ideological, questions."[9] This variation in care

[7] See Chapter 3 for the Council's definition of CER.
[8] See Chapter 6 for a comprehensive listing of CER activities across the Federal Government.
[9] Atul Gawande. "The Cost Conundrum." The New Yorker. June 1, 2009.

documented by Wennberg[10], Fisher[11] and others, means that Americans in one part of the country who are seeing particular clinicians may get vastly different care with potentially worse outcomes than Americans somewhere else. The health system can no longer produce highly variable results and tolerate low quality and inefficiency. The care delivered should be based on evidence and best practices, not on which physician a patient was referred to or where a patient lives. The Council believes that bringing to bear careful research across the continuum of care, from prevention, to diagnosis, to treatment, to delivery systems, will yield improved care for both individuals and for populations.

Current Comparative Effectiveness Research Landscape

In order to inform recommendations for comparative effectiveness research, the Council conducted an inventory of current CER activity. Section 6 summarizes CER activity in the Department of Health and Human Services, the Department of Veterans Affairs and the Department of Defense. Several examples of these activities are discussed below.

AHRQ has an established CER program as described above. As an example, an AHRQ Comparative Effectiveness Review in 2008 examined treatments for localized prostate cancer. There are a number of treatment options available for prostate cancer, each with its own potential for risks and benefits, so it is important that men understand what is known about the effectiveness of these treatments. Key findings from the report included:

- There is a lack of comparative studies across major modalities of treatment (e.g. surgery, radiation, watchful waiting).
- There were no randomized trials evaluating cryotherapy, laparascopic or robotic prostatectomy, primary androgen deprivation therapy, high-intensity focused ultrasound (HIFU), proton beam therapy, and intensity modulated radiation therapy (IMRT). While these therapies have become increasingly of interest for men considering treatments for prostate cancer, it is impossible to evaluate whether these therapies are more or less effective than other options.
- Of men who had surgery, those undergoing a radical prostatectomy were less likely to experience urinary incontinence and other complications if the operation was done by an experienced surgeon in a hospital that does many of the procedures.

NIH has funded numerous comparative trials with huge implications for the practice of medicine. For example, the Diabetes Prevention Program was a major multicenter trial to evaluate the comparative effectiveness of intensive lifestyle changes (diet and exercise), a pill for diabetes (Metformin), or a placebo in preventing the onset of type 2 diabetes in adults with pre-diabetes. This landmark trial found that while both lifestyle changes and Metformin reduced the risk of developing diabetes compared to a placebo, lifestyle changes were significantly more effective than Metformin. This effect was seen in men and women, and in all ethnic groups. With the increasing incidence of pre-diabetes in this country, the results of this trial were critical in informing patients and physicians about prevention strategies for diabetes. Similarly, the BARI

[10] Wennberg J, Gittelsohn A. Small area variations in health care delivery. Science. 1973; 182:1102-8.
[11] Fisher ES, Wennberg J. Health Care Quality, Geographic Variations, and the Challenge of Supply-Sensitive Care Perspectives in Biology and Medicine. 2003; 46(1): 69-79

2D trial compared optimal medical management with revascularization for preventing premature death in Type 2 diabetes and found medical management to deliver equivalent outcomes.[12]

VA also has a very strong history of conducting CER. For example, the COURAGE trial, published in 2007, compared the effectiveness of percutaneous coronary intervention (PCI, or angioplasty) plus optimal medical therapy with optimal medical therapy alone in the prevention of heart attack or death in veteran patients with stable heart disease. The results showed that patients treated with optimal medical therapy alone did just as well as patients who received PCI plus medical therapy. This trial can inform patients and clinicians about the most efficient use of PCI in patients with stable angina.

In addition to Federal activities, state level, private sector, and non-profit sector CER efforts are currently underway across the country.[13,14] For example, 14 states participate in the Drug Effectiveness Review Project (DERP), based at the Center for Evidence-Based Policy (EPC) at the Oregon Health & Science University. The project is a collaboration between the Oregon EPC and the Oregon Center for Evidence-Based Policy. Together, they produce evidence-based reviews of the comparative effectiveness and safety of drugs in many drug classes, and then make this information publicly available.

Large insurers and health organizations such as Aetna, CIGNA, UnitedHealthcare, and Humana have developed the capacity to conduct evidence reviews in-house. These payers may also commission external studies from entities such as the Blue Cross and Blue Shield Association Technology Evaluation Center, which has been conducting evidence-based technology assessments for more than thirty years. Pharmaceutical, biotechnology, and medical device companies may sponsor studies that share some of the attributes of CER. In the non-profit sector, organizations synthesize and publicize CER, rather than generating new evidence. For example, Consumers Union relies on DERP reports to provide information for its *Best Buy Drugs* Web site.

Although there are a number of institutions, both public and private, involved in CER, a number of challenges remain unaddressed. Much of the CER underway is fragmented, and not aligned with a common set of priorities or definition of what constitutes CER. Databases and patient registries that are invaluable for comparative effectiveness analysis are similarly fragmented and often limited in numbers of patients or of variable or unknown data quality. Some resources, such as privately maintained claims databases and Medicare claims data, are difficult for researchers to access due to licensing and cost issues. Furthermore, there are a number of gaps in the content of the research being conducted. Studies often do not include participants of sub-groups, such as racial minorities or people with disabilities, and generally focus on therapeutics at the expense of other types of interventions (e.g., devices or the delivery system). Many effective interventions for improving health are likely to involve prevention and community

[12] BARI 2D study group et al. N Engl J Med. 2009; 360(24):2570-2.

13 Academy Health. A First look at the Volume and Cost of Comparative Effectiveness Research in the United States. Available at: http://www.academyhealth.org/files/FileDownloads/AH_Monograph_09FINAL7.pdf. Accessed June 17, 2009.

[14] The following paragraphs draw on information contained in an environmental scan prepared by the Lewin Group for the Federal Coordinating Council on Comparative Effectiveness Research.

intervention, but these areas are currently understudied. CER should identify interventions that yield the most health improvement and represent the best value wherever and however the interventions are delivered.

The OS ARRA funds are a unique opportunity to address some of these gaps. The following box summarizes gaps in CER landscape:

Major Gaps in CER Landscape

- **Coordination across the CER framework**
  - Substantial CER assets exist across the Federal Government, but coordination is necessary to capture their full value
- **Research**
  - Many comparative, patient-centered research questions remain unanswered
- **Human and Scientific Capital**
  - CER methods development needed
  - Limited trained researchers for conducting CER
- **CER Data Infrastructure**
  - Fragmented data
  - Data sources limited in terms of clinical robustness of data and longitudinal data capture
  - Data capture and feedback loop at point of care often lacking
- **Dissemination and Translation of CER**
  - Suboptimal dissemination and translation of CER findings to patients and clinicians
  - Limited linkages between CER findings and directly improving patient outcomes
- **Priority populations**
  - Limited information on many priority populations and sub-groups
- **Priority Interventions**
  - Less information on certain comparative interventions such as behavioral change, procedures, devices, delivery system strategies, and prevention

<u>Opportunity Provided by ARRA Funds</u>

Within this context of national and international activity, the ARRA CER funds offer an extraordinary opportunity to complement ongoing research in the public and private sectors by establishing a solid infrastructure for future CER. Such investments could include development of data and methods, training of researchers who could accelerate the conduct of future studies, and rapid dissemination of results to patients and clinicians. For example, enhancing existing data resources and learning better how to maximize their utility could expand the types of questions addressed as well as identify high-impact opportunities for research. In addition, ARRA's investment in CER coincides with expected increases in the adoption of health information technology to improve health care quality and safety. That technology also offers the promise of including care delivery in the conduct of research (what some have termed a "learning health care system") and offering a platform for rapid dissemination of results to the

point of care to inform physician and patient decisions.[15] The field of CER is not entirely new, but increased availability of clinical electronic data resulting from diffusion of information technology demands improved methods and a cadre of researchers ready to take advantage of these expanding data resources.

As CER becomes a more integrated resource for health care decision-making, we must assure public trust by ensuring the privacy and security of health information and by maintaining access to appropriate care options. CER should not be used as a sole criterion for denying or awarding care or as justification for making care choices based on cost without consideration of effectiveness, safety, and convenience for an individual patient. CER has the potential to offer tremendous benefits to Americans so long as we apply its conclusions appropriately and protect the individual health information that informs it.

The Council believes that there is much to be learned about how research results can be incorporated into the everyday practice of medicine and inform consumer health care choices. The Council's hope is that ARRA funding has the potential to form a firm base for the Federal Government's future investments in CER and lay the foundation for a productive CER enterprise that improves care for all Americans.

II. VISION AND COUNCIL OBJECTIVES

Comparative effectiveness research has the potential to catalyze a patient-centered transformation of the U.S. health care system. By equipping patients and clinicians with the information needed to make joint medical decisions, and by optimizing the system in which the patient/clinician team makes these decisions, CER can improve the quality, safety, and value of care delivered while increasing patient satisfaction.[16] By passing ARRA, Congress recognized this vision and the need for CER, and also highlighted the need for an unbiased, cross-functional Council to "foster optimum coordination" of the Federal Government's CER efforts.

Given the Council's distinct role and the unprecedented resources available to the Secretary, the Council has a unique opportunity to begin working toward this vision for CER. The Council sees the following as potential accomplishments at the end of the ARRA funding period:

1. Establishment of a process for CER priority-setting that maximizes the value of Federal investments in CER through responsiveness to patient and other stakeholder needs, transparency, and effective coordination.

2. Development of a robust, foundational infrastructure for CER.

3. Implementation of a strategy to support rapid, systematic dissemination of CER results to empower patients, clinicians, and other stakeholders to make more informed decisions and increase the quality of care.

[15] Conway PH, Clancy C. Transformation of Health Care at the Front Line. JAMA. 2009 Feb 18;301(7):763-5.
[16] Naik AD, Peterson LA. The Neglected Purpose of Comparative Effectiveness Research. NEJM. 2009 May 7; 360(19):1929-31.

To accomplish this vision, the Council outlined three specific, near-term objectives that build on those established in ARRA:

1. Develop a definition, establish prioritization criteria, create a strategic framework, and identify priorities that lay the foundation for CER.

2. Foster optimum coordination of comparative effectiveness research conducted or supported by relevant Federal departments.

3. Formulate recommendations for investing the $400 million appropriated to the HHS Office of Secretary as part of this Report to Congress.

III. COMPARATIVE EFFECTIVENESS RESEARCH DEFINITION AND CRITERIA

One of the first activities of the Council was to build on previous definitions of comparative effectiveness research, including IOM, CBO, and others, to develop a definition of comparative effectiveness research for the Council. After much discussion and sharing with the public for feedback, the Council established the following definition.

Definition

Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in "real world" settings. The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.

- *To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations and sub-groups.*
- *Defined interventions compared may include medications, procedures, medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies.*
- *This research necessitates the development, expansion, and use of a variety of data sources and methods to assess comparative effectiveness and actively disseminate the results.*

The definition above is not meant to exclude randomized trials; however, these trials would need comparator arms other than placebo and be representative of populations seen in "real world" practice.

Once a definition was established, the Council drafted threshold criteria for consideration and prioritization criteria for comparative effectiveness research and related investment. These criteria were posted on a public Web site, feedback was received, and modifications were made. The following are the current Council criteria.

Prioritization Criteria for Comparative Effectiveness Research Related Investments

Minimum Threshold Criteria (i.e. must meet these to be considered):

- Included within statutory limits of Recovery Act and FCC definition of CER
- Potential to inform decision-making by patients, clinicians, or other stakeholders
- Responsiveness to expressed needs of patients, clinicians, or other stakeholders
- Feasibility of research topic (including time necessary for research)

The prioritization criteria for scientifically meritorious research and investments are:

- Potential impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm)
- Potential to evaluate comparative effectiveness in diverse populations and patient sub-groups and engage communities in research
- Uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- Addresses need or gap unlikely to be addressed through other organizations
- Potential for multiplicative effect (e.g. lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government)

This definition and criteria guided the Council as it considered potential priority recommendations for the OS funds and will guide AHRQ and NIH in allocating their CER funds.

IV. IMPORTANCE OF PRIORITY POPULATIONS AND SUB-GROUP ANALYSIS

As the United States has grown in its diversity, there has remained a persistent under-representation of women, the elderly, persons with disabilities, and racial and ethnic minorities in clinical and other research studies. While the NIH has a policy of inclusion of women and racial and ethnic minorities in all NIH-funded clinical trials,[17] the majority of research conducted in the U.S. does not require the inclusion of these and other priority populations. The lack of adequate representation of important patient populations in many research studies presents a major challenge in applying the results of these studies to important populations and sub-groups. In recognition of this fact, the ARRA legislation notes that "research conducted with funds appropriated shall be consistent with Departmental policies related to the inclusion of women and minorities." This criterion is critically important for ensuring that information gained from comparative effectiveness research improves the quality of care for all Americans.

[17] http://grants.nih.gov/grants/funding/women_min/guidelines_amended_10_2001.htm

Indeed, focused attention is needed on priority populations,[18] including racial and ethnic minorities, individuals with disabilities, children, persons with multiple chronic conditions, and the elderly, not only because of their under-representation in current research but also because of the increased disease burden and health disparities faced by these sub-groups.

The following sections highlight some of the challenges facing our health system as it relates to priority populations. Disparities in health care and health outcomes for these populations persist, affecting an ever-increasing proportion of residents of the United States. Also outlined are some of the research challenges that exist for priority populations, followed by recommendations to address these issues.

Growth in Priority Populations

Priority populations not only account for a large proportion of current health services utilization, but their numbers are growing; their need for health care services will likewise continue to grow. The most recent U.S. Census Bureau data reveal that over 100 million people living in the United States belong to a racial or ethnic minority group; this equates to 34 percent of the total U.S. population, and these minorities will likely become the majority of the U.S. population within 30 years.[19] Similarly, the number of elderly Americans is growing, with that segment of the population expected to increase from 35 million today to 71 million by 2030— or nearly 20 percent of the overall U.S. population. The population over the age of 85 is projected to grow from 5.3 million today to 21 million by 2050.

Health Disparities

A number of important reports have highlighted disparate disease prevalence, progression, and health outcomes for racial and ethnic minorities, elderly Americans, individuals with disabilities people of low socioeconomic status, people with mental illness, and others.[20,21] In this context, health disparities are defined as significant gaps or differences in the overall rate of disease incidence, prevalence, morbidity, mortality, or survival rates in the priority population as compared to the health status of the general population.[22] For example, African-American women are 34 percent more likely to die from breast cancer, even though they are diagnosed with the disease 10 percent less frequently than white women; Hispanics in the U.S. are 50 percent more likely than whites to suffer from diabetes; and the incidence of diabetes among

[18] Priority populations are defined in Sec. 901 of the Healthcare Research Act of 1999, S. 580.
[19] U.S. Census Bureau. Minority Population Tops 100 Million: Press Release. Available at: *http://www.census.gov/Press-Release/www/releases/archives/population/010048.html*. Accessed April 1, 2009.

[20] Report of the Secretary's Task Force on Black and Minority Health. U.S. Department of Health and Human Services. 1985.

[21] Brown ER et al. Racial and Ethnic Disparities in Access to Health Insurance and Health Care. UCLA Center for Health Policy Research and the Henry J Kaiser Family Foundation. 2000.

[22] Minority Health and Health Disparities Research and Education Act of 2000. Public Law 106-525. November 20, 2008.

Native Americans is more than twice that for whites. Elderly Americans also face particular health challenges, from greater susceptibility to multiple chronic conditions to a lower likelihood of obtaining preventive treatments, including mammograms and immunizations. In addition, approximately 42 percent of individuals over the age of 65 report a functional limitation.[23]

Persons with Disabilities

According to the 2007 Institute of Medicine report *The Future of Disability in America,* from 40 to 54 million people in the United States have disabilities. These numbers will grow considerably in coming decades as baby boomers age and as new medical interventions extend the lives of young persons with significant impairments who would once otherwise have died. Although rates are lower in children, disability prevalence is rising at younger ages. According to figures from the National Health Interview Survey, childhood disability has risen by 350 percent during the last 40 years, with the largest increase occurring during the past decade.

Across the lifespan, disabilities are clinically and functionally heterogeneous, encompassing diverse cognitive, sensory, physical, and mental health impairments. Traditionally patients with disabilities have been excluded from clinical trials, yet they have the same risk for diseases as non-disabled persons. Future clinical trials should exclude persons with disabilities only if there are clear and compelling reasons to do so.

Comparative effectiveness research relating to persons with disabilities is important in a number of areas.

First, research would be beneficial about the most effective interventions to prevent or mitigate disability and the disabling effects of chronic diseases. All research including comparative effectiveness research relating to disability should include outcome measures that address functional abilities, people's abilities to participate in daily activities, and quality of life. This is critical as the world's population is growing older at a very fast pace and this has serious implications due to expected increasing rates of chronic conditions. Moreover, with the advances in science and technology, lifespan has increased considerably; this is also true for persons with disabilities.

Second, future comparative effectiveness research should look into community-based models of care for persons with disabilities. Following the Supreme Court *Olmstead v. L.C. ex rel. Zimring, 527 U.S. 581 (1999) Decision*, traditionally institutionalized individuals with disabilities or those at risk of being placed in institutions are increasingly being cared for in their own homes and/or communities. Underscored by the *Olmstead Decision* states now have to consider civil rights when developing their programs. Effective care coordination/care management is critical to help persons with disabilities live independently in their communities with added years of quality life. Care coordination/care management is even more important for those individuals with multiple chronic conditions, which are often associated with certain levels of disability. While care

[23] Federal Interagency Forum on Aging-Related Statistics. Older Americans 2008: Key Indicators of Well Being. Federal Interagency Forum on Aging-Related Statistics. Washington, DC: U.S. Government Printing Office. March 2008

coordination/care management is the current state-of-the-art, it is still considered to be in its early stage. This represents a major opportunity for building the infrastructure to support future CER studies. In addition, because the definition of care coordination varies according to settings and models of care, its effectiveness has not been clearly established, particularly as it relates to the role support services play and how better integration of health and support services can lead to improved health outcomes for persons with disabilities and reduced health care costs for our nation.

Third, persons with disabilities are at increased risk for developing secondary conditions that are associated with their primary disabling condition. For example, without preventive measures, individuals with spinal cord injuries may acquire a number of adverse health conditions, including cardiovascular disease, genitourinary tract disorders, depression, obesity, and pressure sores. Comparative effectiveness studies should determine which interventions are most likely to prevent secondary conditions or ameliorate their consequences.

Fourth, studies should investigate the comparative effectiveness of rehabilitation interventions to restore or maintain functioning or minimize its loss. For example, much more research is needed to identify effective speech-language, physical, and occupational therapy interventions. This research could include a comparison of conventional treatments to newer interventions or a comparison of various systems of care. More research is also required about various assistive devices, medical equipment, and technologies, including technologies addressing sensory deficits, communication impairments, and physical and motor limitations.

Fifth, comparative effectiveness studies of therapeutic and preventive interventions need to address explicitly the needs of children with disabilities and be sensitive to the developmental stage of the child. For many children with disabilities and complex health care needs, the transitions through adolescence and into adulthood are complicated by the absence of comprehensive care programs that fully address their needs. Comparative effectiveness studies should examine different care models to determine which ones offer the best care coordination and generate the greatest patient and family satisfaction and health outcomes.

Other underrepresented populations

Children represent another group that can benefit tremendously from comparative effectiveness research. Evidence cannot simply be extrapolated from adults to the pediatric population. There is a dearth of information to inform decisions by children and their families, especially since outcomes, such as quality of life and functioning, are often more subtle. In addition, comparative preventive interventions (e.g. for obesity) will often have the most long-term effects if started in the pediatric population.

At the other end of the continuum, the elderly represent another group for which there exists little information about best care practices. As our population ages, knowledge about the best and most effective treatments for this group will become essential. Other important areas of focus for the elderly include home health care strategies and optimal approaches to delivery of care within nursing facilities.

Veterans and service members often have many conditions for which CER could be informative. They have a number of special considerations in deployment-related illness such as post traumatic stress disorder, traumatic brain injury, exposures, infectious diseases, disabilities and others. CER provides a vital opportunity to glean additional information necessary for clinicians to make informed decisions about particular veterans needs and information to assist veterans in their participation in care decisions.

Finally, research to compare the effectiveness of prevention strategies, treatments, diagnostics, and care delivery for patients with multiple chronic conditions is essential. Again, as our population ages, patients increasingly have several comorbidities which may impact their response to treatment. The majority of clinical research to date excludes such patients, so the applicability of "standard" treatments to this population is unclear. A physician advising a 45-year-old woman with asthma and HIV about treatment for breast cancer simply does not have the evidence necessary to factor her comorbidities into her patient's treatment decision. By utilizing varied and robust research methodologies, CER affords the opportunity to target treatments and other interventions to improve the quality of life and overall health of this important group of patients.

Personalized Medicine and Patient Sub-groups

The need to identify and address the needs of emerging patient sub-groups, and indeed the very concept of sub-categories of conditions to which medical products are applied, is expected to change and grow as our understanding of genomics and molecular medicine increases and becomes an integral part of health care. Better understanding of an individual's genomic and other individual biological characteristics will enable us to recognize and respond to human variability with a new degree of specificity. Understanding biological differences at the molecular level promises a significant leap in our ability to use and develop medical technologies more effectively, targeting interventions at more defined groups of individuals with greater precision. This potential, sometimes referred to as personalized medicine, has strong bearing on comparative effectiveness research.[24] Many drugs prescribed in the United States today are effective in fewer than 60 percent of treated patients. This is not a fault of the drugs, but reflects the variability of metabolism or other factors from person to person.[25]

Unfortunately, it remains common medical practice to follow a trial-and-error approach in selecting medical interventions for patients to achieve a satisfactory therapeutic outcome. In the case of breast cancer, for example, while chemotherapy can be an important positive treatment for some patients, we have few tools today to successfully predict which patients will benefit– and the result is that many women who are treated with chemotherapy today are receiving treatments that may not be effective for their condition.

Personalized medicine aims to make medical care more precise and effective. Increased understanding of our individual genomic profiles and other individual biological characteristics

[24] Willard HW: Organization, Variation and Expression of the Human Genome as a Foundation of Genomic and Personalized Medicine. In *Genomic and Personalized Medicine. Volume 1.* Edited by Willard HW and Ginsburg GS. London: Academic Press; 2009:4-21.
[25] Spear BB, Heath-Chiozzi M, Huff J. Trends Mol Med. 2001 May; 7(5):201-4.

will enable us both to use more effectively the therapies we have now and to identify significant areas where research and development of new products may be needed. Pharmacogenomics, the use of genetic information or other biomarkers to assist in accurate medical therapy decision-making, is expected to be a hallmark of this approach.

CER can be an important partner in helping to bring about this new level of medical effectiveness, personalization, and innovation. At the same time that CER is being used to identify which interventions and strategies work best on average, it can also help to identify different responses by different groups of patients. In some cases, different existing therapies may be identified as most effective for specific sub-groups. In other cases, CER may help to identify significant sub-groups for whom effective therapies do not yet exist. CER may also help steer research efforts toward the development of products and strategies for areas of significant need.

Research Challenges

Multiple research challenges exist for priority populations. Examples include a need for increased diversity in research populations, expanded data sources for evidence-based studies in diverse populations, enhanced collection of racial and ethnic health data, a better understanding of the effectiveness of interventions in the context of comorbidities, and a greater focus on implementation research.

Generalizations that result from comparative effectiveness research that fail to consider sub-groups and individual differences may have limited applicability. Currently there are gaps in knowledge about whether specific treatment strategies work across different sub-groups under a variety of circumstances. Recognizing that there might be variations in the effectiveness of specific interventions in the elderly, racial and ethnic minorities, individuals with disabilities, and other priority populations is key to designing evidence-based strategies to successfully improve the quality of care that is delivered. Infrastructure investments that capture priority populations and patient sub-groups will be critical to overcoming these challenges.

Strategies to Strengthen Comparative Effectiveness Research for Priority Populations

In light of the aforementioned challenges, comparative effectiveness research presents an opportunity to be more inclusive of minorities, the elderly, persons with disabilities, and other priority populations. This feature of CER is especially true in the context of conducting specific studies that take into account health conditions and linguistic and cultural attributes in order to develop the most appropriate and effective interventions.

Investments in CER can be used to address the needs of priority populations by doing the following:

Evaluating and identifying interventions that are tailored for priority populations. To explore which interventions are most effective for addressing the needs of priority populations, specific studies are needed to look at interventions that target diseases with a high prevalence in racial and ethnic minority communities, the elderly, and individuals with disabilities. These

studies may need to simultaneously address several diseases/conditions, or assess combinations of interventions (e.g., behavioral and physical treatments/interventions) that are most effective in promoting desired outcomes for these populations. Studies examining care delivery interventions tailored for priority populations are also needed in order to ensure that care is delivered to these individuals through effective approaches that are targeted to their needs. To ensure effective communication with the priority populations both in conducting the research and implementing its results, investigators should ensure that those language and communication services are available for those with limited English proficiency or disabilities.

Creating and enhancing potential databases looking at interventions in priority populations. Successfully examining and evaluating a range of interventions that are effective for priority populations will require a broad range of potential data sources and infrastructure investments. In addition to traditional patient registries and systematic reviews, the inclusion of distributed data networks that utilize community-based infrastructure, such as Federally Qualified Health Centers, will be an important asset in broadening the tools to evaluate effectiveness in various priority populations. CER studies should routinely perform and report sub-group analyses to examine possible differences in effectiveness for important racial and ethnic groups, and should over-sample such groups whenever there is existing evidence to suggest differences in effects or outcomes in any priority population. Standardized reporting and analysis of priority population sub-groups will also permit pooling of research results across studies to explore sub-group differences.

In addition, efforts should be made to build capacity and infrastructure within traditionally underserved racial/ethnic communities to allow for standardization of data collection and to enable the seamless integration of such data with larger databases/systems currently in use by the research community. This will allow for more accurate downstream comparisons to pre-existing and future majority data sets, producing more comprehensive and reliable CER study results.

Finally, this infrastructure for CER in priority populations is particularly important for developing and implementing Clinical Preventive Services Guidelines and recommendations for the U.S. Preventive Services Task Force. According to the IOM, CER data on priority populations is often unavailable for developing guidelines, and what information is available is often insufficient for making conclusions on how to treat priority populations.

Increasing the number of community-based studies, including community-based participatory research (CBPR) studies. CBPR is defined as a collaborative research approach in which communities and researchers are equally involved in the design and conduct of research that is conducted in their communities. Successful and effective CBPR studies result in the development of research tools, strategies, and interventions that are effective in creating sustainable and positive behavior changes and outcomes among priority populations within communities. Because CBPR studies are conducted with substantial input from the community, interventions are typically tailored to fit the needs and characteristics of the community. Furthermore, communities become “owners” of the research, which results in sustainable research outcomes.

Increasing cultural competency. Understanding the linguistic, cultural, social, and environmental attributes of priority populations is essential in designing interventions and promoting strategies that are effective in addressing the needs of these populations. Specifically, doing so allows for the development of culturally and linguistically appropriate interventions. For example, an obesity/diabetes intervention involving diet and/or physical activity would require an understanding and assessment of the populations' cultural attributes (e.g., food preferences), social attributes (e.g., competing family and work demands), and environmental attributes (e.g., access to 'healthy' foods and safe walkways) that support or inhibit adhering to a diet and/or physical activity intervention.

Building workforce capacity. Racial/ethnic minorities, individuals with disabilities, and women are underrepresented in the research and medical communities. The lack of a diverse and linguistically competent scientific workforce adds to disparities in research development, service delivery, and quality of care. Initial CER investments in workforce capacity could create opportunities to engage researchers and providers from diverse backgrounds. For example, 90 percent of minority physicians educated at Historically Black Medical Colleges live and serve in minority communities. Hispanic-Serving Institutions (HSIs) also play a major role in educating Hispanics researchers. Approximately 49 percent of all Hispanic students attend an HSI. A special focus on priority populations could provide an avenue for engaging Historically Black Colleges and Universities and HSIs in the conduct of CER among priority populations.

Developing and implementing outreach strategies to various racial, ethnic, and health disparity populations for participation in research protocols. In order to strengthen CER, effective outreach strategies must be developed and implemented that will increase the participation of priority populations in clinical research protocols. Developing appropriate strategies to reach out to various priority communities requires an understanding of the history of these populations in research and the identification and recruitment of trusted community members who can champion the research benefits and inform communities about risks. Community health workers can be important partners in addressing and advocating for the needs and concerns of priority populations. In addition, clinicians and providers will need to be educated on the benefits and implications of CER and the utilization of evidence-based interventions.

Dissemination, translation and adoption of research results is one of the biggest challenges within comparative effectiveness research, particularly as applied to priority populations, but also as applied to the population as a whole. The young science of implementation research focuses on the acceleration of translation of evidence into everyday care, and affords an opportunity to build a more coordinated approach to improving the quality of health care of priority populations. This is not a one-way transfer of knowledge. Racial and ethnic minorities, persons with disabilities, children, and the elderly, can offer insights into how best to engage their communities. Active listening and thoughtful planning of the dissemination process can create better health outcomes for all Americans.

Making CER investments that are responsive to the needs of priority populations and sub-groups is critical to ensuring that the benefits of CER reach those with the greatest needs. Such

investments, however, can also benefit the population as a whole by validating new strategies and approaches for comparative research and implementation.

V. STRATEGIC FRAMEWORK FOR CER

There are countless opportunities for action and investment in CER. Many Federal, state, and private institutions are already involved in CER and have made choices about which of these activities and investments to pursue. After completing the draft definition and criteria for prioritization of potential CER investments, the Council recognized the need to develop a strategic framework for CER activity and investments to categorize current activity, identify gaps, and inform decisions on high-priority recommendations.

This framework represents a comprehensive, coordinated approach to CER priorities. It is intended to support immediate decisions for investment in CER priorities and to provide a comprehensive foundation for longer-term strategic decisions on CER priorities and the related infrastructure. At the framework's core is responsiveness to expressed needs for comparative effectiveness research to inform health care decision-making by patients, clinicians, and others in the clinical and public health communities. The framework will be supported by detailed inventories of Federal CER activities and research/data infrastructure, and a priority-setting approach. This organizing framework fosters consideration of the balance of activities and priority themes, focuses on the most pressing needs expressed by patients and clinicians, and allows for identifying and addressing gaps in the current landscape of CER.

CER activities and investments made by the government or other institutions can be grouped into four major Core Categories:

- **Research** includes activities or investments in primary research or meta-analysis. Organizations involved in this group of activities may be funding research, conducting research themselves, or helping to establish a common set of research priorities to create momentum around the most critical research topics.

- **Human and Scientific Capital** includes activities or investments that enhance the United States' capacity for CER by expanding and strengthening relevant research skills or by advancing CER approaches and methodologies. Organizations involved in this group of activities may be directly involved in training and workforce development, developing new CER methods, validating results of CER, or driving consensus on valid approaches to CER.

- **CER Data Infrastructure** includes activities or investments that develop, build, or maintain data infrastructure, systems, or tools. These investments could include the creation of new research data sets and repositories, aggregation of existing data sources, development of new tools to query and analyze existing data sets, or creation of standards for new data collection.

• **Dissemination and Translation of CER** includes activities or investments that disseminate CER findings and put them into practice. Activities and investments range from dissemination and distribution of CER information to improving processes and outcomes in health care and public health delivery systems through CER translation and adoption.

Table 1
Example Activities in Each Major Category

Activity	Examples
Research	Comparing outcomes of treatments or care delivery for a specific condition
Human & Scientific Capital	Training new researchers to conduct CER or developing CER methodology and standards
CER Data Infrastructure	Developing a distributed practice-based data network, linked administrative or EHR databases, or patient registries
Dissemination and Translation of CER	Building tools and methods to disseminate findings and translate CER into practice to improve health outcomes for patients

Furthermore, investments or activities focused on a specific priority theme can cut across these categories. The potential themes include:

•**Conditions.** Organizing investments and activities around a condition or disease state is common in research and reflects the organization of medical practice. Focusing on a single disease state across all four major categories of activity (e.g., funding primary CER in oncology, developing new methodologies for CER in palliative care settings, expanding the Surveillance, Epidemiology, and End Result database (SEER), and partnering with an academic cancer center to pilot CER implementation strategies) could result in significantly improved patient-centered outcomes in that disease area.

•**Patient populations.** While clinical research is relevant to the patient population it is designed to address, it often provides little information relevant to patient groups not typically enrolled in clinical studies. In private-sector-funded trials, this often includes the elderly, racial and ethnic minorities, children, and persons with disabilities. The NIH, however, already requires that all publicly funded trials include appropriate numbers of women and racial and ethnic minorities. Cross-cutting activities and investments that facilitate studies responsive to the needs of these populations can ensure that all Americans benefit from CER.

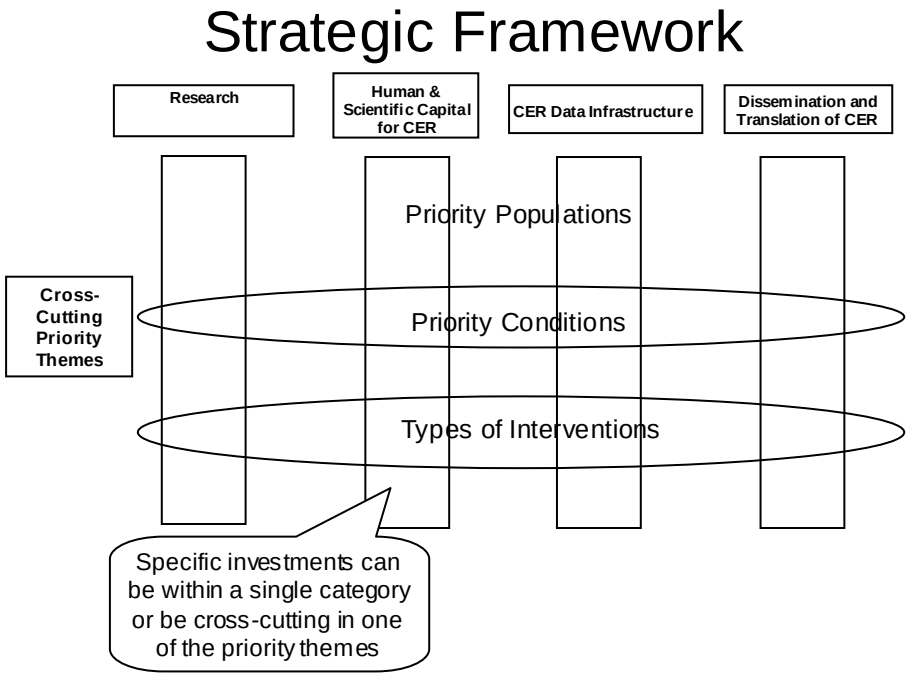
•**Type of intervention.** Several potential areas of focus emerge from studying interventions by type. In defining CER, the Council specifically included the following types of interventions: medications, medical and assistive devices, procedures, behavioral change, diagnostic testing, and delivery system strategies. Each of these has unique opportunities for coordinated investment in data infrastructure, research, building

research capacity, and translation. In addition, one could focus on interventions at a stage of the disease (i.e., prevention, diagnosis, treatment, and management).

Together, these activities and themes make up the CER strategic framework (Figure 1).

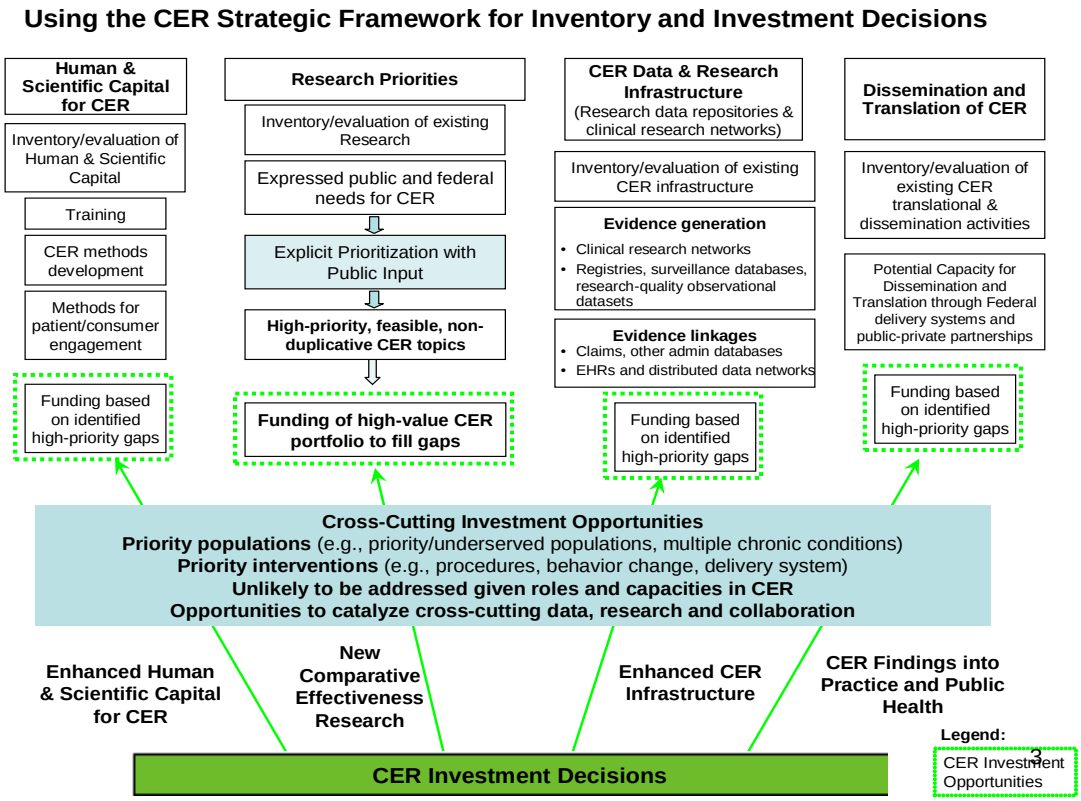
Agencies or organizations that are engaged in CER will often make investments in one group of activities or across multiple groups within a cross-cutting theme. The pattern of activity and investment for a single organization highlights its strategy. For example, a medical information database company may concentrate its CER activities in data infrastructure, whereas the National Cancer Institute is involved in multiple types of activities with a focus on cancer. When patterns of activity for the most critical agencies and organizations involved in CER are viewed in aggregate, the CER framework reveals gaps in CER activities and investments. These gaps are potential areas of opportunity and impact for the Secretary's ARRA funds. As such, the framework is useful for determining what investments are appropriate for ARRA funds and for future Federal investments in CER, as well as for codifying the ongoing activities of Federal agencies involved in comparative effectiveness research.

Figure 1



Creating and maintaining an inventory summarizing current and past Federal efforts across the CER framework is critical to its value in decision-making. This inventory of Federally-sponsored CER activities will also be a critical component of future Council annual reports. This process of inventory-taking, gap analysis, and establishing priorities for investment should be iterative. The process for developing the inventory and aligning findings from that process with CER investment decisions is outlined in Figure 2.

Figure 2



Overall, the CER framework is a useful strategic and analytic tool to help organize ongoing CER activities of Federal agencies, to facilitate development of a strategy for the Secretary's ARRA investments, and to continually monitor progress in CER across the different dimensions of the framework.

VI. CURRENT CER INVENTORY AND CER DATA INFRASTRUCTURE

The following CER inventory and data infrastructure was collected for the first time and on a very short timeline. The counts of CER studies are based primarily on electronically accessible sources, informed in part by interviews of senior agency staff. Attributes of the research reported here (study designs, types of interventions studied, etc.) were determined from study summaries or abstracts rather than inspection of full-text reports of these studies.

As described below, providing a high-confidence estimate of the number of Federally-funded CER studies underway for a given fiscal year is not currently feasible. Prospective identification of CER studies using keywords or other "tagging" in one or more readily searchable electronic databases would enable tracking of completed and ongoing CER. Therefore, this preliminary inventory is informed by a convenience sample and should be viewed as a rough estimate of what will be an iterative process going forward.

Although ARRA is the first coordinated Federal CER effort, several Federal agencies have been conducting comparative effectiveness research and maintaining data and infrastructure for CER. Most of this activity has been conducted independently within the given agency. The agencies most active in CER include AHRQ, NIH, and the Veterans Health Administration (VHA). But many other agencies conduct or have resources related to CER to a lesser degree, such as comparative effectiveness research studies, related data infrastructure, or the potential to be effector arms for research dissemination and translation. Finally, it is important to note that this inventory does not include CER conducted by private or not-for-profit organizations.

CER Inventory

Table 2 provides information about the numbers of studies for these agencies. There is no standard, systematic means of reporting on CER studies and funding across Federal agencies. It is not possible at this time to estimate the total number of primary or secondary CER studies conducted by the Federal Government. Other than AHRQ, by virtue of its dedicated Effective Health Care Program, agencies have limited ability to track CER studies and spending, reflecting that CER is a relatively new field of inquiry, has no standard definition, and is not "tagged" or readily searchable in biomedical or health services research databases. AHRQ tracks its funding and number of studies by fiscal year. Funding for CER studies for AHRQ ranges from 12 million to 35 million per fiscal year since FY 2006, with 12-18 studies funded per year. Estimates for the number of CER studies and funding for DoD and VHA are approximations per year rather than specific numbers for particular years. For example, DoD estimates its funding to be approximately $125,000 to $500,000 per year for 5-10 studies per year; the VHA estimates are 50 million to 70 million per year for 40-50 studies per year.

As part of its large portfolio of biomedical research, the CER funded by NIH makes that agency the single largest sponsor of primary comparative effectiveness research. These studies are difficult to identify, however, as they are not "tagged" or otherwise readily searchable as CER in such databases as ClinicalTrials.gov or CRISP (Computer Retrieval of Information on Scientific Projects, a database of biomedical research funded by NIH).

For purposes of this pilot inventory, a keyword search of ClinicalTrials.gov yielded an initial set of 1,800 NIH-funded trials during the years 2006-2009 that were candidates for CER. Subsequently, in cooperation with NIH, a sample set of 463 NIH CER studies for 2008 was identified, starting with a new searching process under development by NIH to track CER studies and spending.[26]

[26] NIH recently developed an initial process involving a keyword searching software algorithm based on consensus among several experts regarding which studies from among those funded by NIH qualify as CER. NIH applied this algorithm to all studies funded by NIH in 2008, which yielded more than 800 studies with a score above a certain threshold—tagging them as potential CER. Inspection of all of the records of all of these studies in CRISP by staff supporting the Coordinating Council identified the set of 443 that appeared to qualify as CER. This set of 443 does not necessarily represent the full set of CER studies funded by NIH in 2008.

Table 2: Estimated CER Grant/Study Counts FY 2006 – FY 2009[1]

Agency	CER Grants/Studies FY2006-FY 2009 (YTD)
AHRQ	144
DoD	25
VHA	96
NIH[2]	463

[1]As of June 2009, based on review of agency/department websites and agency/department generated lists

[2]NIH is in process of cataloging CER. This primarily represents FY 2008.

CER studies conducted or sponsored by VHA and DoD often focus on the particular populations they serve. These include CER studies involving patient groups that fall within designated U.S. priority populations (e.g., the elderly, racial and ethnic minorities, patients with multiple chronic conditions, persons with disabilities).

The main findings from analyses of Federal CER for fiscal years 2006-2009 include the following:

- In this initial compilation, the inventory of CER that could be confirmed independently for those agencies that perform or sponsor it was generally comparable to the inventory as described in interviews with agency staff. The main exception was NIH, where the volume of CER is acknowledged to be large yet remains to be quantified.
- Other than that for AHRQ, agency budgets for CER are not well defined. Agency staff typically described rough percentages of total research budgets or approximate ranges of annual expenditures on CER, but generally could not cite budget amounts allocated to CER (e.g., by Federal fiscal year).
- Excluding AHRQ, which could cite studies in its Effective Health Care program as at least a core set of CER, agency staff could not specify the number of CER studies conducted per year or other period. Three main factors account for this. First, there has not been a standard definition of CER. Second, while agencies may have a sense of expenditures or relative emphasis of CER, individual studies are typically not titled, given keywords, or otherwise "tagged" in a manner for identification as CER. Third, the time frame for CER study counts is not standardized; some agencies provided counts in terms of studies underway during a given year, others provided counts of studies initiated in a given year. Thus, providing a high-confidence estimate of the number of Federally-funded CER studies underway for a given fiscal year is currently not feasible. Clear identification of CER studies, particularly prospectively, would better enable tracking of completed and ongoing CER.
- Combined Federal CER is broadly distributed across study types (i.e., primary versus secondary studies). The volume of primary CER sponsored by NIH, particularly Randomized Controlled Trials (RCTs) and other trials, accounts for the largest general type of CER.
- The greatest concentrations of Federal CER are systematic reviews by AHRQ, RCTs by NIH, and RCTs by VHA (Table 3).

- Most AHRQ CER comprises secondary research (i.e., systematic reviews and other syntheses) and VHA supports secondary research through its Evidence-based Synthesis Program. Otherwise there is little emphasis on secondary research. Moreover, mathematical modeling is infrequently used in Federal CER (Table 3).
- Most primary research is done through RCTs (Table 3).
- Without careful inspection on a trial-by-trial basis, reliable detection of "practical" (or "pragmatic") trials among the primary CER studies is not possible. As a group, the VHA trials appear to have more such "practical" characteristics than trials sponsored by other agencies.
- Relative to the RCT volume from NIH and VHA, the use of observational analyses, including those involving large patient-level databases, is relatively infrequent.
- The locus of research varies by agency. All CER funded by VHA and most by DoD is intramural. Most CER funded by AHRQ is extramural. Although NIH conducts some intramural primary research, most CER is done extramurally.
- The interventions studied most often in Federal CER are pharmacologic, which account for the majority of the interventions studied by AHRQ and NIH. These are followed by studies of the health care delivery system, led by VHA, and behavioral interventions (which are often compared to pharmacologic interventions), led by NIH and VHA (Table 4).
- Roughly 86 percent of the CER studies in this sample across agencies focus on at least one priority disease/condition. The leading categories among these are depression and other mental health disorders, substance abuse, cardiovascular disease, and diabetes (Appendix C).
- The distribution of priority diseases/conditions studied by DoD and VHA largely reflects the respective populations they serve. For DoD, they are cancer, functional limitations and disability, and depression and other mental health disorders. For VHA, they are cardiovascular disease, and depression and other mental health disorders (Appendix C).

Table 3: Estimated Types of CER by Agency/Department

Study Type[1]	AHRQ	NIH[2]	DoD	VHA	Total
Primary Research					
Randomized Controlled Trial	11%	79%	0%	77%	60%
Practical/Pragmatic Controlled Trial[3]	3%	1%	16%	1%	2%
Other Non-Randomized Controlled Trial	2%	2%	32%	0%	3%
Observational Study (natural experiment)	1%	2%	0%	4%	2%
Observational Study (Prospective/Registry)	4%	3%	16%	6%	4%
Observational Study (Retrospective)	9%	5%	6%	4%	6%
Secondary Research					
Systematic Review	58%	0%	13%	0%	14%
Meta-Analysis	3%	0%	0%	0%	1%
Mathematical Model	4%	3%	3%	3%	3%
Research Training	n/a[4]	0%	13%	0%	1%
Other Capacity Building	n/a[4]	0%	0%	1%	0%
Other	2%	2%	0%	3%	2%

[1] Some studies include more than one study design, totals may not equal 100% due to rounding.
[2] NIH 2008 (based on sample of 443 studies) plus NIH multi-year (based on 30 studies across years).
[3] Rough estimate given no standard definition for pragmatic trial.
[4]AHRQ has been heavily involved in development of human and scientific capital for CER. It provides career development (K) grants for CER as well as a T and R grant for CER capability building. It also has funded numerous methodology studies for CER. These will be more fully quantified in the completed inventory.

Table 4: Estimated Types of Interventions Included in Studies

Study Intervention Type[1]	AHRQ	NIH[2]	DoD	VHA	Total
Pharmacologic Treatment	35%	68%	24%	10%	34%
Biologic Treatment	1%	1%	10%	4%	4%
Alternative Medicine	2%		8%	1%	2%
Medical Device/Equipment	17%	6%	0%	7%	11%
Surgical Procedure	11%		3%	9%	9%
Behavioral Intervention	11%	24%	11%	24%	16%
Public Health Intervention	2%	1%	17%	3%	3%
Delivery System	11%		19%	41%	20%
Other	10%		8%	1%	2%

[1] Some studies include multiple types of interventions and may not total 100% due to rounding
[2] NIH multi-year. Will need to be updated once inventory based on types of NIH interventions is complete.

The involvement of priority populations in CER sponsored by Federal agencies is varied. While several studies do not explicitly focus on a priority population, investigators sometimes report on analyses of one or more specific sub-groups:

- About half of CER studies across these Federal agencies involve a priority population, with nearly 60 percent of VHA studies doing so. Many studies focus on more than one population group. In part consistent with their respective missions, the agencies exhibit different distributions of emphasis on priority populations.
- Among those studies that do involve priority populations, those involving patients requiring chronic care, and those who are elderly are the most common. While no studies specifically indicate a focus on low-income groups, such individuals often comprise some of the patients studied, including the elderly, those with multiple chronic conditions, and minority groups.
- Studies vary as to whether there is sufficient representation of one or more priority groups in the study population to enable sub-group analysis, even if the study does not focus on a priority population as a principal objective. Particularly at AHRQ, in cases where studies do not have as their primary focus a priority population, sufficient numbers of members of priority groups may not be present for sub-group analyses, especially in the case of systematic reviews.
- Future iterations of the inventory will need to drill down on the representation of priority populations in studies.

CER Data Infrastructure

Substantial Federal and private sector infrastructures exist that could be used to identify potential CER priorities, to support the conduct and improve the productivity of CER, and to enable the translation of research findings into actionable information. However, the current infrastructure for CER is fragmented, and it is not coordinated or mobilized in a way that would enable providing coherent and targeted support for CER.

Patient-level Databases and Databases to Support Researchers

Federal agencies support or have access to substantial patient- and person-level databases that could support CER. Additional databases in the private sector can also deliver specialized content for CER. For example, these Federal and private sector databases can support or enable:

- Analyses preparatory to CER, such as.:
    - Disease prevalence and burden to help determine priority areas for comparative effectiveness research.
    - Utilization and distribution (e.g., geographic) of alternative interventions to help identify variations in practice and candidate interventions for CER.

    - Patient characteristics, socioeconomic attributes, comorbidities, and so forth, to determine the availability of certain patient populations for clinical trials, registries, and other person-level studies.
- Observational studies and retrospective data analysis (e.g., mining data from natural experiments).
- Support for prospective studies, including efficient development of registries and objective collection of treatment detail.

Important considerations for investing in and applying patient/person level databases to CER include:

- Potential to link to other databases that enrich the person/patient view, such as databases containing socioeconomic characteristics of individuals and mortality information (e.g., the Social Security Deathmaster or the CDC National Death Index).
- Potential to link databases that contain clinical information to those with transactional information (e.g., linking claims databases that have chemotherapy detail on cancer patients to electronic health records or registries for the same patients that have clinical data such as cancer stage, histology, and patient status).
- Research readiness of the databases (e.g., requiring minimal time on the part of the researcher to learn database attributes and develop special programs for data clean-up and access).
- Requirement to maintain security and privacy for any personally identifiable health information.

Appendix C lists some key patient-level databases with potential applications for CER. Among the ones available through Federal agencies are the major administrative databases maintained by CMS, the medical records databases at VHA, targeted databases maintained by AHRQ and NIH focused on service areas (e.g., HCUP on hospital-based care), and the NIH's SEER cancer registry.

Key private sector databases for CER include large administrative databases with longitudinal health care detail on millions of patients, and consolidated databases on EHRs. To the extent that these repositories can be linked (for which many have the potential), they can be highly valuable assets for CER, particularly because they account for commercially insured populations that are not captured in Federal and state databases.

In supporting research activities, the following Federal data infrastructure assets can speed communication among researchers and expedite identification of researchers with special skills:

- AHRQ: the DEcIDE Network, the CERTs (Centers for Education & Research on Therapeutics), and group of EPCs (Evidence-Based Practice Centers).
- NIH Clinical Translational Research Awards (CTSAs) recipients.
- CDC: Evaluation of Genomic Applications in Practice and Prevention (EGAPP) workgroup.
- HRSA research networks: Pediatric Research in Office Settings (PROS) and Emergency Medical Services for Children (EMSC) groups, among others

- SAMHSA: National Child Traumatic Stress Network
- VA Research Center of Excellence

Other databases for supporting researchers include:

- ClinicalTrials.gov (Federally and privately supported clinical trials).
- MEDLINE/PubMed (biomedical journal literature), HSRProj (Health Services Research Projects in Progress).
- CRISP (biomedical research funded by NIH, including clinical trials and other studies).
- Disease-oriented databases, surveys and Web sites, including the Longitudinal Studies on Aging (NCHS and NIA) and the Cardiovascular Health Study (NHLBI).
- Survey of Mental Health Organizations, General Hospitals Mental Health Services, and Managed Care Organizations (SAMHSA).
- Numerous CDC disease and research data assets and sites, including the NCHS surveillance systems, cancer registries, and vaccine registries.

None of these databases with actual or potential applications in CER were developed for the explicit purpose of comparative effectiveness research. Furthermore, they generally have not been organized or indexed to enable searching for CER. For example, careful record-by-record inspection of such research study databases as ClinicalTrials.gov and CRISP is required to identify CER. In order to assess current gaps and support translation and adoption of CER findings efficiently, these databases would require "tagging" of records or related searching functions that would enable accurate identification of CER.[27]

Dissemination and Translation Infrastructure

A few agencies, notably AHRQ, VHA, NIH, and SAMHSA, have capacities to translate CER into actionable information for practitioners, patients, and other target audiences. The VHA's capabilities for translation and adoption are inherent in its integration of research and patient care at VHA treatment centers. Additional agencies also have capabilities for disseminating information to segments of consumers and practitioners. All of these agencies have the potential to influence adoption of CER findings.

There are, however, minimal formal mechanisms to disseminate and translate CER from research agencies such as AHRQ and NIH into the delivery system side of HHS (e.g., HRSA, IHS, SAMHSA, CMS QIO's). In addition, given the current expansion of CER and the increased emphasis on achieving impact from its findings, the current dissemination and translation capacity of the relevant agencies involved in CER is likely to be insufficient for achieving CER's potential.

Some of the key elements that can be leveraged in a comprehensive and articulated CER dissemination and translation strategy are outlined below.

[27] In MEDLINE, for example, indexing tags for particular "publication types," such as Randomized Controlled Trial, Clinical Trial, Phase III, Meta-Analysis, and Review, would readily enable searching for journal articles that report such studies.

- AHRQ
  - CER methods guides, tools, and resources made available via the AHRQ Web site, Web conferences, public service announcements, advertising campaigns, online audio guides available to public, and other means for informing consumers, clinicians, and policymakers.
  - The John M. Eisenberg Clinical Decisions and Communications Science Center, which focuses on translation of research to various target audiences.
  - AHRQ Publications Clearinghouse.
  - AHRQ dissemination partnerships, including with health professional societies, patient advocate groups, and non-profit organizations focused on particular diseases/conditions.
  - Effective Health Care Program Stakeholder Group, which helps to identify important information gaps, ensure transparency, and provide feedback on reports.

- CDC
  - Information to monitor the adoption of CER recommendations and to track the effects from changes in clinical practices and policies on the following process and outcomes measures: clinical management of specific conditions, including the use of medications and other specific services, and intermediate health-related outcomes, such as test results; incidence and prevalence of specific conditions; personal behaviors, health status, and functioning; and births and deaths.
  - Public use data from NCHS surveys available through the CDC/NCHS Web site and internal confidential data available for researchers through the NCHS Research Data Centers.
  - NCHS/CDC reports, including Data Briefs and E-Stats, and other analyses available through the Web site, and articles in the peer-reviewed literature.
  - Dissemination by and with collaborators, including sponsors of specific data collection and analysis.

- DoD
  - Searchable publication libraries, including the Military Health System Publication Search.
  - DeployMed Research Link, which informs Service members, researchers, health care providers, military leaders, and others about DoD and other Federally funded medical research related to deployments since 1990.

- NIH
  - Clinical and Translational Science Awards (CTSAs), which are NIH-funded academic centers that translate research into practice.
  - Nation Cancer Institute's Physician Data Query, an online database that summarizes study results in prevention, screening and management of cancer in versions appropriate for physicians and for patients.
  - Research databases, including MEDLINE/PubMed, HSRProj, CRISP, and ClinicalTrials.gov.

- Public health campaigns, such as Red Dress (women's heart health) and Small Steps Big Rewards (weight loss).
- NIH Consensus Development Conference program, which summarizes knowledge about a variety of clinical and public health interventions.

- VHA
  - QUERI (Quality Enhancement Research Initiative) program for enhancing the uptake of evidence within VHA.
  - Periodic research summaries and issues briefs for senior VHA clinical and policy leaders, and related research results disseminated to researchers.
  - CME programs for nurses and other health professionals that incorporate recent research findings.
  - Print and online patient education tools, including the MyHealthE Vet Web site, for dissemination to patients.
  - Point-of-service decision-support tools and reminders to clinicians within the VHA EHR system guiding practice toward the most effective treatment, including a Web portal for clinicians to access clinical practice guidelines.

- SAMHSA
  - National Registry of Evidence-based Programs and Practices (NREPP) and the Technical Assistance Centers can serve as translation vehicles. NREPP is a searchable online registry of approximately 140 mental health and substance abuse interventions and targeted outcomes; it provides quality of research and "readiness for dissemination" ratings.
  - The Addiction Technology Transfer Center (ATTC) Network is comprised of 14 Regional Centers and a national office which facilitates alliances among providers, administrators, and recovery and treatment communities, and connects them to the latest research and information through activities such as skills training, academic education, online and distance education, conferences, workshops, and publications.
  - The National Centers for the Application of Prevention Technologies (CAPT) work to bring research to practice by assisting States/Jurisdictions and community-based organizations in the application of the latest evidence-based knowledge to their substance abuse prevention programs, practices, and policies.
  - The SAMHSA Health Information Network (SHIN) provides a one-stop, quick access point that connects the behavioral health workforce and the general public with the latest information on the prevention and treatment of mental and substance abuse disorders.

- FDA
  - Web site provides news and other information to physicians and consumers on drugs, biologics, and devices.

- Office of Public Health and Science (OPHS)
  - Comprises 12 core public health offices and the Commissioned Corps, some of whom work with population and community-based networks to disseminate health information (e.g., Office of Disease Prevention and Health Promotion, Office of

Minority Health (OMH), Office on Women's Health). OMH, for example, has cooperative agreements and other partnerships to disseminate research findings (though not CER to date) to minority populations.

- Office of the National Coordinator for Health Information Technology (ONC)
  - Efforts to develop and implement a nationwide, interoperable health information technology infrastructure could provide a means for incorporating CER into decision-support systems for clinicians and other applications in health care.

- HRSA
  - Among multiple dissemination vehicles, the AIDS Education and Training Centers Program and the Ryan White HIV/AIDS Program support a network of 11 regional centers and more than 130 associated sites that conduct targeted, multidisciplinary education and training programs for health care providers treating people living with HIV/AIDS.
  - HRSA's Maternal and Child Health Bureau disseminates information using cooperative agreements with professional organizations and academic institutions, and funds grants for continuing education to academic centers across the country, specifically for the purpose of translating research into practice.

There is virtually no capacity to track the impact of CER dissemination, translation, and adoption activities. As a result, this limits the ability to measure the impact of CER and to conduct research on effective approaches. Claims databases could be one resource for tracking changes in practice over time and their impact.

Human and Scientific Capital

The future workforce engaged in CER should include experts from a wide array of disciplines, including biostatistics, epidemiology, mathematics, economics, and ethics. To date, however, there has been little focus on human and scientific capital infrastructure for CER. The principal exception is the close affiliation of certain AHRQ activities involving academic centers and other organizations, including the DEcIDE network, CERTs, EPCs, the Eisenberg Center, and various awards to researchers. AHRQ funding of DEcIDE network members and EPCs supports research trainees at those organizations. AHRQ also provides career development (K) grants focusing on generation of new scientific evidence and analytic tools that enable the prioritization of evidence-based services and goals for patients with multiple comorbidities.[28] In addition, AHRQ has sponsored other scientific and methodological activities, including development of methods guides, training seminars, and related events (e.g., at AcademyHealth and other professional conferences), and various workshops and support materials on MEPS, HCUP, and other data sets.

NIH provides significant training opportunities that could incorporate CER, including support for medical students interested in research, clinical fellowships, workshops for researchers, training grants, and consensus conferences. The CTSA program at NIH provides translational development support at academic and other research centers, some of which addresses evidence-

[28] http://grants.nih.gov/grants/guide/notice-files/NOT-HS-08-004.html.

based medicine approaches, if not CER in particular. The NIH K30 Clinical Research Curriculum Awards support training in design of clinical research projects, hypothesis development, biostatistics, epidemiology, disease mechanisms, medical technology, human genetics, and the legal, ethical, and regulatory issues related to clinical research.[29]

Although DoD has an extensive training and professional education infrastructure, it does not focus on CER.

A small number of training programs at academic centers focus on areas that address methodologies and study designs related to CER. Among these are the Clinical Research Training (CREST) program at Boston University, which provides training in clinical research that includes epidemiology, clinical epidemiology, health services research, biobehavioral research, and translational research,[30] and the Duke Clinical Research Training Program, which provides training in quantitative and methodological principles of clinical research, including research design, research management, medical genomics, and statistical analysis.[31]

Several agencies draw on the considerable scientific and methodological expertise resident in the FDA, but there is little emphasis on comparative effectiveness research at that agency. These informal links to scientific expertise could be formalized; also, specific CER expertise could be housed in selected agencies with an expectation of a cross-agency role. FDA expertise would be of exceptional value in, for example, understanding the respective merits of alternative study designs for assessing efficacy vs. effectiveness and for collecting and assessing adverse event data, strengths and limitations of using surrogate endpoints and other biomarkers in CER, incorporation of genomics and other aspects of personalized medicine into CER. Phase III and phase IV studies could also generate evidence on comparative effectiveness, as well as on other scientific and methodological aspects of CER.

Despite the promise of "practical" or "pragmatic" trials for CER, methodological gaps and threats to internal and external validity remain. Real world trials must deal with confounders, including confounding by indication and presence of comorbidities, selection bias, and other factors that impede the assessment of cause and effect. Focused research to improve the validity of practical trials and interpretation of their findings could enhance the use of these study designs.

Further development of mathematical modeling approaches and retrospective data analysis capabilities would also provide alternative means of analyzing comparative effectiveness, as well as generating viable research hypotheses and providing input for designing primary and secondary CER.

Gaps in the Current CER Landscape and Investment Opportunities

The inventories of CER and CER data and research infrastructure reveal gaps and other challenges for achieving the potential of comparative effectiveness research.

[29] http://grants.nih.gov/training/K30.htm.
[30] http://www.bumc.bu.edu/clinepi/crest/general-info/
[31] http://crtp.mc.duke.edu/content.asp?page=about

Coordination across the CER framework: Substantial CER assets exist across the Federal Government, but coordination is necessary to capture their full value. Several challenges exist in achieving this:

- Prior to this report, there was no standardized Federal definition for CER; aligning organizations around this definition will be necessary for identifying, cataloging, and disseminating CER in a coordinated manner
- Difficulty in setting national CER priorities.
- Structural barriers that limit collaborations among agencies.
- Limited coordination with private sector CER efforts. This includes lack of integration of existing data sets across payers, suboptimal development of CER data infrastructure, an inability to track populations and treatments across payers, and suboptimal translation and adoption of CER findings.
- Unrealized benefits of stakeholder involvement. Greater involvement of stakeholders (e.g., patient advocates, health professionals, researchers, technology manufacturers, payers) in CER processes can help to achieve the goals of CER, including more informed priority setting, input on certain aspects of study design (e.g., identification of important subgroups and patient-centered outcomes), and identification of target audiences for CER and strategies to reach them.

Research: Despite the comparative effectiveness research to date, there are many unanswered questions.

- Those who sponsor and design clinical trials continue to face challenges in tradeoffs between internal validity of CER for causal effects of interventions on outcomes and external validity of CER to heterogeneous patient groups and routine health care settings.
- Increased emphasis on well-conducted pragmatic trials could increase acceptance of CER findings.
- May research questions for important clinical health care decisions remain unanswered

Human and scientific capital: Due in part to the increasing interest in comparative effectiveness research, continued investment in human and scientific capital for the field is needed.

- Greater investment is needed in developing education and training programs to support the development of professional talent, the development of methods for linking and using databases for CER, the development of new methodologies for pragmatic trials, effective translation and adoption of CER findings into practice, modeling approaches for CER, and evaluation of the impact of CER
- More methods work is needed to advance the state-of-the-art for pragmatic trials and to provide training for using these study designs.
- Recent growth in training for the related fields of health technology assessment, outcomes research, and health economics, among others, has helped to yield a cohort of researchers who are well-positioned to become more expert in CER, along with

educational curricula and materials that can be adapted for training in comparative effectiveness research.

CER Data Infrastructure: The scope and scale of CER requires data infrastructure that may outstrip current capabilities.

- Current data sources are fragmented and limited in terms of clinical robustness and longitudinal data capture.
- An evolving inventory of CER data infrastructure is needed to track the capacity of this infrastructure and provide a basis for its further development; this inventory should include observational databases, registries, claims and other administrative data, pharmacy and laboratory data, adverse events registries, EHR networks, and other health information technology.
- In addition to one or more inventories, greater understanding is needed regarding the strengths and limitations of these data sources, and areas for their further development. An example of a relevant resource is the 2007 *Registries for Evaluating Patient Outcomes: A User's Guide*, produced by the AHRQ's DEcIDE Research Center.
- Investment in linking such data sources is more likely to be realized by establishing clear information policies and technical standards, standardized terminology, improved platform capability, novel search algorithms, mechanisms to maintain patient privacy, and controls to access data, and by reducing and coordinating data processing times.[32]
- There are few searchable electronic inventories or related databases of CER and CER infrastructure. While sources like ClinicalTrials.gov, CRISP, MEDLINE, and HSRProj contain information about completed and ongoing CER, but they are not presently configured or linked to serve the needs of CER.
- Absence of an inventory of CER limits the ability to assess the magnitude and nature of the current portfolio of completed and ongoing CER, to identify CER on particular topics, and to inform priority-setting for CER.
- A comprehensive inventory of CER infrastructure would improve the ability to conduct CER and to allocate resources to develop the national capacity to conduct CER.

CER Dissemination and Translation: Many findings to date from CER have not yet been fully integrated into clinical practice or made accessible to patients in easy-to-understand language.

- Certain effective dissemination avenues are in place, including among some of the agencies engaged in CER. Except for AHRQ, however, these agencies are not yet oriented to CER and do not adequately extend beyond dissemination alone to translation and adoption of CER into practice.
- Tools and mechanisms to support clinicians and patients in incorporating available CER information are lacking. This information needs to be delivered to the front line of care where health decisions are made and results measured.

[32] See, for example: Diamond CC, Mostashari F, Shirky C. Collecting and sharing data for population health: a new paradigm. Health Aff (Millwood) 2009;28(2):454-66.

Priority populations and other sub-groups: At present, the agencies have largely separate approaches to addressing these groups. A better-coordinated Federal approach is needed to address priority populations and priority conditions, including sub-groups with multiple chronic conditions.

- Greater attention on designing studies with sufficient power to discern treatment effects and other impacts of interventions among patient sub-groups (e.g. accounting for heterogeneity of treatment effects) will better serve clinical decision-making, enabling more individualized, patient-specific care.
- Improved partnerships with Federal grantees serving priority populations, such as Community Health Centers, will enhance their engagement with CER.
- Improved access to and utilization of Federally sponsored databases that include priority populations can significantly enhance the inclusion of sub-groups into CER.

Types of interventions: To date, CER has been disproportionately focused on pharmacologic treatments rather than the full spectrum of intervention types. This likely derives in part because of the relative emphases of the research agendas of agencies that sponsor CER and the focus of the private sector is primarily on new drugs and biologics. The emphasis on pharmacologic treatments has meant fewer resources for other interventions, including behavioral, procedures, prevention, and delivery system interventions, that can have major impacts on health outcomes.

VII. PRIORITY-SETTING PROCESS

The Council actively sought public input throughout this process, and this input significantly influenced all Council decisions. To help guide the Council's deliberations on the definition, framework, and priorities for comparative effectiveness research, the Council held three listening sessions and solicited additional public comments online. The Council heard from over 300 stakeholders representing health care associations; consumer, community, and advocacy organizations; academia and think tanks; patients; providers; hospitals and hospital systems; payers; pharmaceutical companies; foundations, public health entities; and private sector companies engaged in the health care field. One U.S. Senator also submitted comments.

Several respondents honed in directly on the reason why investments in CER are important. One person, for example, said that CER is crucial to reforming the practice of medicine to increase the quality, safety, value, and effectiveness of what providers bring to patients on a daily basis. Other respondents addressed a wide range of interrelated issues, including priorities for the research agenda, collaboration, infrastructure development, research methodology, transparency, care delivery, cost, and knowledge transfer. Many patients expressed their need for this type of research; one of the most emotional and moving testimonies came from the mother of a child with a seizure disorder in Chicago who had struggled to find the best treatment for her child. A physician from the American Board of Orthopedics summarized many physicians' testimony by saying, "developing high quality, objective information will improve informed patient choice, shared decision-making, and the clinical effectiveness of physician treatment recommendations."

The public input has been extremely valuable in informing the Council's deliberations, and many of the major thematic threads that run thought the public comments are reflected in the strategic framework, focus, and recommendations for priorities for OS CER funds. Details about what the public had to say are contained in Appendix A.

The Council also conducted a first draft inventory of CER and data infrastructure (outlined above) to help identify gaps in the current CER landscape. For the Office of Secretary funding recommendations, the Council proceeded through structured deliberations informed by public input, developed an inventory of current activities, established prioritization criteria and a strategic framework, and discussed the unique role for OS funds to fill gaps and build the foundation for future CER. In the future, the Council should continually and actively engage stakeholders inside and outside the government, including patients, providers, payers, employers, industry, academia, and others. This critical component of the priority-setting process could take the form of even more active participation by external stakeholders in the future.

VIII. PRIORITY RECOMMENDATIONS FOR OFFICE OF SECRETARY CER FUNDS

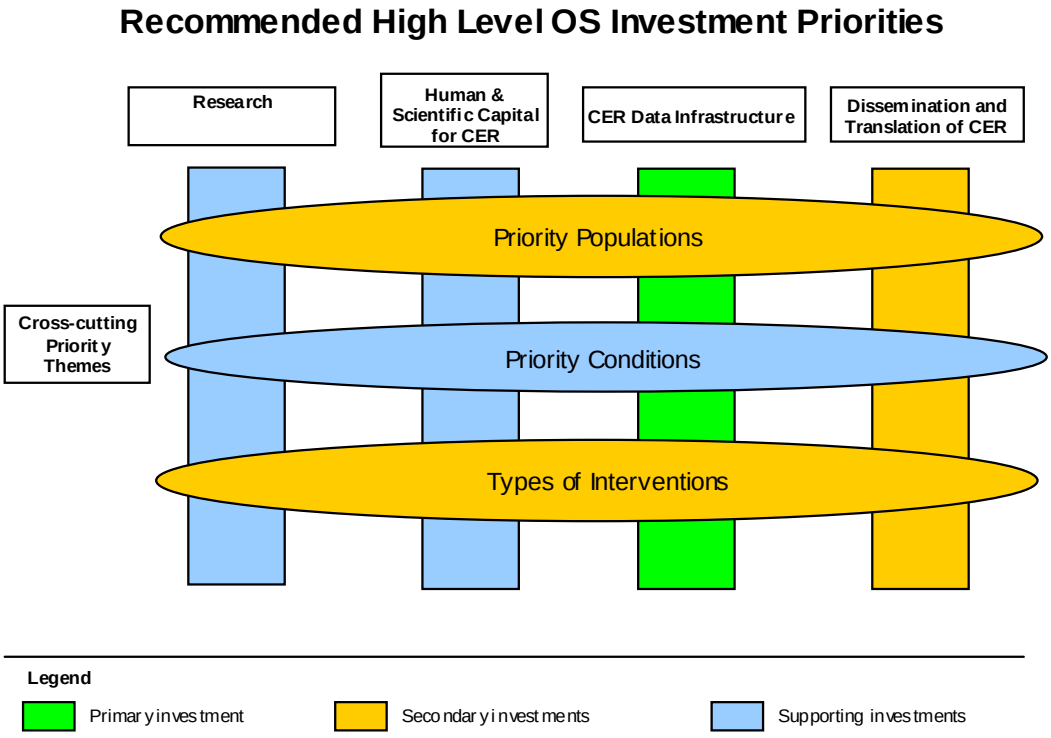
Using the strategic framework for CER discussed in Section V, and taking into consideration the unique role that OS funds can play in addressing high priority gaps, the Council developed a recommended high-level investment strategy for the use of the OS ARRA funds. The strategy has three different levels of priority recommendations for OS fund investments in the Core Activities and Cross-cutting Priority Themes in the CER framework (Figure 3).

- **Primary investment.** This area of investment should represent a large portion of the OS funds. It best fulfills the full range of prioritization criteria and requires scaled investment in order to be successful. The Council recommends that CER Data Infrastructure be the primary investment.

- **Secondary investments.** These areas should also receive significant investment. They are as critical to success in CER as the primary focus, but individually may require a smaller amount of funding to be successful. The Council recommends that Dissemination and Translation of CER, Priority Populations, and Priority Types of Intervention be secondary investments.

- **Supporting investments.** These areas should not be the major focus of OS funding as they do not fulfill the prioritization criteria as well as primary and secondary investments, but some funding may be necessary to support and enable investments in higher priority areas and fill identified gaps. The Council recommends that Human and Scientific Capital, Research, and Conditions receive supporting investments. It is important to note that these recommendations pertain only to OS funds; AHRQ, NIH, and VA have a history of significant investments in Research, Human and Scientific Capital, and Conditions.

Figure 3



The Council believes that this strategy and distribution of investments will best position the Secretary to:

- Respond to patient and physician demand for CER.
- Balance achieving near-term results with building longer-term opportunities.
- Capture the distinctive value of the Secretary's ARRA funds.

While it is the responsibility of the Office of the Secretary to operationalize this strategy, the Council's rationale for these recommendations is designed to help guide the Secretary in making specific investment decisions. The Council based its rationale for each level of investment in the strategy on the prioritization criteria described above, as well as representative examples of investment in each area proposed through the public comment process and by Federal agencies.

Primary investment

CER data infrastructure development is the most distinctive opportunity for OS ARRA funding. It requires a large, up-front infusion of capital to be successful that is unlikely to come from any source other than OS ARRA funds, making it ideal for this funding mechanism. It has broad potential impact, with the ability for resulting research to address conditions and populations captured in the primary data. Given the absence of comprehensive databases and data evaluation

tools (See Section VI), there is significant demand from the patient, clinical, and public health communities for new, expanded data infrastructure and data access to support decision-making. Finally, investments in data infrastructure have the potential to generate significant additional investments in two ways. First, some of these investments could take the form of public-private partnerships. Second, data infrastructure is a tool that, once developed, will result in new research conducted and/or funded by entities such as biomedical research organizations, payers, foundations, and health care providers.

The Council received proposals on a number of potentially promising initiatives related to data infrastructure, including but not limited to:

- Building, expanding, and linking longitudinal administrative claims databases.
- Linking administrative data with EHR-based or registry data.
- Expanding high-impact patient registries, (e.g., collaborations with specialty organizations, SEER).
- Distributed data networks populated by EHRs in practice and provider settings.
- Expanding analysis of FDA and private sector data on drug and device trials and safety.

As the Office of the Secretary identifies specific opportunities in data infrastructure, the Council recommends that it consider most carefully those that:

- Expand access to existing resources, especially those currently managed by Federal agencies.
- Create scaled platforms by leveraging existing data and capabilities in the private sector.
- Capitalize on linkages between health IT investments and the potential for CER infrastructure to develop evidence to inform decision-making.
- Ensure that infrastructure is responsive to needs of patients, providers, and other decision-makers—and not driven by what is most feasible.

The Council appreciates the relationship and need for coordination between CER and health IT (e.g. through a distributed network of EHRs) investments. As the Secretary develops HHS's full portfolio of ARRA investments, it will be critical to consider both CER and health IT holistically, not as policy silos, recognizing that success in CER is largely dependent on success in health IT and vice versa.

With all data infrastructure investments, the government will need to ensure data security and privacy. Protecting security and privacy is key to maintaining the public's trust.

Secondary investments
Secondary investments include a core area of investment—Dissemination and Translation of CER—and two cross-cutting themes—Priority Populations and Types of Intervention.

Dissemination, translation and adoption of CER is about realizing the benefits that comparative effectiveness research has to offer both patients and providers. While the breadth and depth of the near-term impact depends on what types of pilot programs the OS supports, the lessons and tools for translation developed by those pilots will be relevant to all.

The lack of reliable success in disseminating findings from CER in ways that translate into better health outcomes highlights the uncertainty and difficulty of this enterprise. However, dissemination and translation is essential to improving outcomes for patients and the link between evidence production and how best to get this information to physicians and patients in a way they understand is critical to capitalizing on the CER investment. Despite important efforts by the Federal Government, especially AHRQ, NIH, VA and DoD, the majority of current funding goes to building evidence as opposed to ensuring that the existing evidence base is utilized in patient care and health systems management. This creates a unique role for OS ARRA funding. Investments in dissemination and translation programs also have the potential to generate additional investments, especially from providers, if private institutions elect to implement similar efforts or partner with the Federal Government on translation efforts.

There are a wide range of potential dissemination, translation and adoption programs that the OS could support, including:

- Investing in dissemination and translation of CER findings throughout the Federal delivery system.
- Dissemination and translation through partnerships with provider and/or patient organizations.
- Decision support and shared decision-making tools to provide information to clinicians and patients at the point of care.
- Developing standards for communication tools for patients and providers, (e.g., a patient-friendly simple scoring system).
- Partnering with an existing consumer media channel (e.g., Internet search engine or health information site) to expand patient access to existing CER data.
- Creating a National Patient Library with a primary focus on providing evidence to patients in easy-to-use and understandable formats.

The Council recommends that the Office of the Secretary consider the following in making investments in dissemination and translation:

- Investing in better understanding the most effective methods to disseminate and translate research findings to improve patient outcomes.
- Identifying opportunities both to develop tools for translation and to pilot implementation of these tools.
- Partnering with provider organizations in Federal agencies, as well as in states and the private sector.
- Accounting for potential surrogate decision-makers (e.g., families) and the context for decisions in patient-focused tools.
- Ensuring that programs address a specific need articulated by the implementing organization or the partner to ensure success and the sustainability of dissemination activities.
- Focusing on developing standards for communication.
- Increasing understanding of the most effective methods to disseminate findings to clinicians and patients to inform decision-making

From an operational perspective, investments in the cross-cutting themes are somewhat distinct from investments in the core areas. Whereas funding for a core area might go to a project or organization focused on a specific activity, funding for a cross-cutting theme requires multiple coordinated investments and activities to be successful. Investments in these themes could cover some or all of the four core activities: research, data infrastructure, human and scientific capital, and dissemination and translation. These investments could involve a coordinated investment across HHS or the Federal Government, or they could be focused in academic centers, integrated delivery system organizations, private industry, or other non-governmental entities. Collaborative efforts to inform and transform care will be essential to achieving meaningful impact across these cross-cutting themes.

Investments in specific populations, meanwhile, will help ensure that the benefits of CER are available to all. It can also focus CER efforts on populations with existing health disparities and worse outcomes. CER has the potential in some populations, such as racial and ethnic minorities, to fill critical gaps that, historically, efficacy research has left unaddressed.

The Council identified several populations for whom the Secretary should consider allocating CER funds:

- Racial and ethnic minorities
- Persons with disabilities
- Elderly
- Children
- Patients with multiple chronic conditions

Investment in specific types of interventions in a cross-cutting manner also presents a unique opportunity for the nation's health system. The Council has identified six specific interventions for the Secretary to consider that address large and varied populations, resulting in high potential impact, are areas of high clinical uncertainty, and are not being adequately addressed by other entities. They are:

- Medical and assistive devices (e.g., comparing rehabilitative devices).
- Procedures and surgery (e.g., evaluating surgical options or surgery versus medical management).
- Diagnostic Testing (e.g. comparing imaging modalities for evaluating certain types of cancer)
- Behavioral change (e.g., developing and assessing smoking cessation programs).
- Delivery system strategies (e.g., testing two different discharge process care models on readmission rates or testing two different medical home models on preventing hospital admissions and improving quality of life).
- Prevention (e.g., comparing two interventions to prevent or decrease obesity, comparing strategies for reaching populations that do not access the health care system with prevention efforts).

Furthermore, the Council recommends that the Office of the Secretary consider the following in making investments in the cross-cutting themes of priority populations and types of interventions:

- Focusing on immediate, specific patient needs that can generate results.
- Concentrating on areas with cross-cutting gaps in research, data infrastructure, scientific capital, and/or translation.
- Building on promising systems and practices already in place, both within the government and in the private sector, and measuring results when scaled up and disseminated.
- Strongly encouraging coordination across the government and with entities outside of the government.

Supporting investments

The Council recommends that the OS reserve some ARRA funding for Research, Human & Scientific Capital, and the Conditions cross-cutting theme. Because these investments and topics are the major foci of CER activities at NIH and AHRQ, both of which will likely utilize ARRA funds administered by those organizations for these purposes, they do not represent distinctive investment for OS funds. However, there will likely be targeted investments in these areas that could support other OS ARRA efforts, such as training new researchers in CER methods or addressing gaps not addressed elsewhere in the Federal Government.

In making these targeted investments, the Council recommends the Office of the Secretary consider:

- Focusing on areas that maximize the value of the Secretary's investments in other areas.
- Avoiding duplication of efforts with other agencies.

For all of the above investments, the Council recommends that the Office of the Secretary consider the portfolio of investments and where synergies exist to leverage one investment into multiple areas. For example, a data infrastructure investment that can also be used for a cross-cutting priority theme would be of higher value than an investment that has more limited applications. Doing so will help to ensure that the funds allocated to the Office of the Secretary for CER will have a significant positive impact on the quality of patient care in the near term, and lay the foundations for continued improvements going forward.

IX. LONGER-TERM OUTLOOK AND NEXT STEPS

Outlook

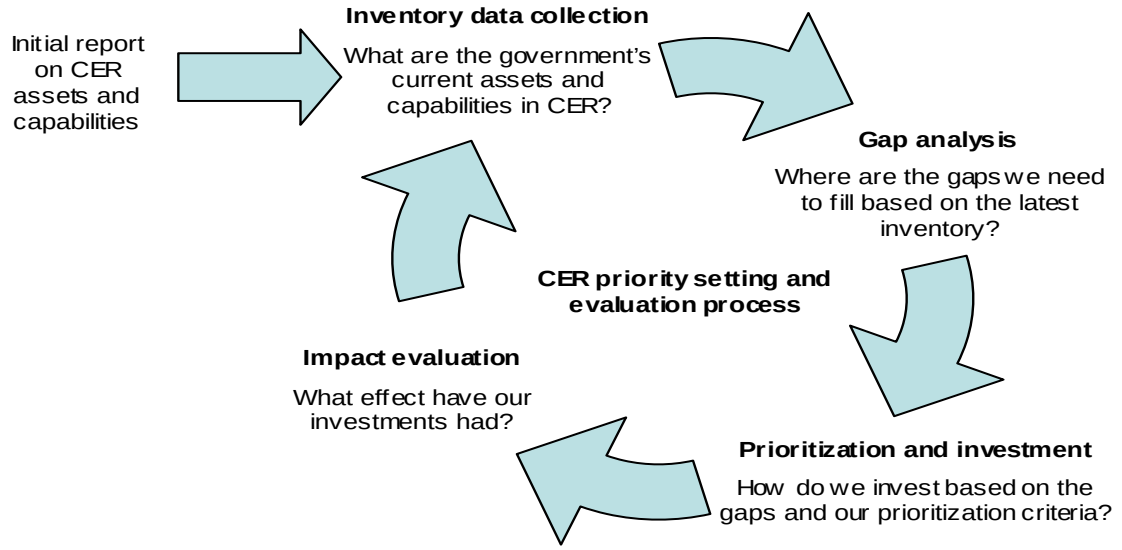
The CER investment strategy recommended in the previous section, if implemented successfully, has the potential to further a number of elements of the Council's vision for improved patient care. In the near term:

- CER dissemination and translation pilots in Federal health care delivery systems could help empower patients and their physicians to make better decisions now about their care and serve as models for expansion to private delivery systems. Moreover, a time limited investment can support establishment of a systematic strategy for translating the products of all relevant research to benefit patients served by Federal programs.
- An increased emphasis on CER for priority populations could ensure that all will benefit from comparative effectiveness research.
- Improved access for researchers to existing Federal data sources, and development and enhancement of distributed data networks and patient registries outside of the Federal Government, could jump-start a new wave of CER in the areas that matter most to patients.
- The inventory of Federal activities in CER will help reduce duplicative or uncoordinated investments among Federal agencies and help create transparency for patients.

It will be essential that a continuous cycle of CER priority-setting and evaluation of impact take place. The four critical steps in this cycle are inventory of CER and infrastructure; gap analysis; priority setting; and evaluation of impact. Figure 4 below depicts this process.

Figure 4

Continuous Evaluation of CER Inventory, Gaps, Priority Setting, and Impact



The strategic framework for comparative effectiveness research is intended to lay the groundwork for longer-term initiatives, such as innovative public-private partnerships to build data infrastructure and conduct CER. The goal of this investment is to generate some near-term results and momentum for the future. This strategy allows the government to facilitate the building of needed infrastructure, to expand access to existing infrastructure, and to demonstrate proof of concept for implementation efforts. These efforts are only a first step, however, to

achieving the vision of improved quality, safety, efficiency, equity, and patient satisfaction through improved medical decision-making and an optimized health care delivery system.

Indeed, a number of clear issues and challenges will remain for Federal CER efforts in the near term:

- **Listening and Engaging External Stakeholders.** While the Council has worked to create transparency and responsiveness in this process to date, it is critical that it continue to have a patient-centered focus going forward. In the future, the Federal Government may want to consider the options of listening and engaging stakeholders with representatives including, at minimum, patients, providers, payers, employers, and industry representatives, to guide CER or broader patient-centered outcome efforts.
- **Continued Coordination.** The Council laid the groundwork for coordination, but coordination will need to become embedded across the Federal Government. As the government makes investments in CER, there must be a mechanism in place to track and coordinate these investments and avoid duplication of efforts.
- **Building Scientific and Human Capital.** To maximize the potential benefit of investments in CER, the nation needs more researchers trained in the applicable research methods and further development of these methodologies. This presents both a short-term and a long-term challenge.
- **Maintaining Gains.** These investments represent only the beginning of CER efforts. New research findings will need to be disseminated and successful translation of evidence efforts should be expanded. New databases and data sets need to be maintained and kept current, and the catalog of Federal activities and data infrastructure in CER needs to become a living document.
- **Building Leverage.** The Federal Government is now a major funding source of CER, but the private sector still represents a majority of the investment in biomedical research. The government needs to find innovative ways of partnering with the private sector to leverage government investments and help private-sector investment better serve patients.
- **Keeping it Current.** There are no widely accepted and applied common standards or approaches for periodically re-evaluating CER to ensure that previous conclusions still hold. A system must be developed to ensure that the conclusions from CER remain valid over time.

These issues reflect both the fact that comparative effectiveness research remains in its infancy and that it must be seen as a continuous and iterative process that needs to constantly evolve based on the changing needs of the patient.

Next steps

There are a number of next steps leading to the Secretary's integrated strategy and spend plan for CER on July 30, 2009, and several requirements for the success in implementation of that strategy. Most importantly, it is critical to the success of CER and health care transformation that the plan is coordinated across the ARRA CER funding allocated to the Secretary, AHRQ, and NIH. Furthermore, the Secretary should develop the plan as part of HHS's broader portfolio of ARRA investments, not as a stand-alone program.

The following steps are needed to finalize the CER operational strategy prior to the July 30 deadline mandated by Congress:

- Integrate IOM and Council strategic recommendations and leverage the investments, resources, and capacity identified through the initial inventory effort.
- Provide more specific recommendations for a portfolio of initiatives for all of HHS' ARRA funds within the framework outlined in the report.
- Define metrics for evaluating success.
- Coordinate the submission of the CER ARRA spend plans to ensure that they cover the gaps in CER outlined in this report, and that the agencies' investments leverage the strengths of each agency and are complementary, not duplicative.
- Maintain transparency and engagement with the public.

The Federal Government will need to continue its work to coordinate CER investments and assure Americans that these resources are being invested wisely. ARRA required that the Council submit an annual report regarding its activities and recommendations concerning the infrastructure needs, organizational expenditures, and opportunities for better coordination of comparative effectiveness research by relevant Federal departments and agencies. The first annual report will likely be in June 2010.

Comparative effectiveness research is being considered as a key piece of health reform, and the Federal Government must demonstrate its capability to coordinate that investment, achieve impact, and measure the results. This report outlines the priorities and path forward. Now the Federal Government must make progress and deliver results for the American people.

APPENDICES

Appendix A. LISTENING SESSIONS AND PUBLIC COMMENT SUMMARY

Overview

In order to help guide the Council's thinking on the definition, framework, and priorities for comparative effectiveness research (CER), the Council held three listening sessions (April 14 in Washington, D.C.; May 13 in Chicago; and June 10 in Washington, D.C.) and solicited public comments through the hhs.gov/recovery Web site. The response was strong:

- 92 panelists testified
- Greater than 300 individuals and organizations submitted comments

A breakdown of the responses by stakeholder type reveals that over half of the comments came from three groups: health care associations; academia and think tanks; and consumer, community-based, and advocacy organizations. Additional comments were received from patients, providers, payers, hospital and health systems, pharmaceutical companies, foundations, public health entities, and private sector companies in the health care field. One U.S. Senator also submitted comments.

More important than the diversity of respondents is what individuals and organizations had to say. Respondents provided a wide range of opinions and offered recommendations on everything from stakeholder participation to how to prioritize investments in CER to specific areas of focus to knowledge transfer and dissemination.

Without question, the public input has been extremely valuable in informing the Council's deliberations; many of the major thematic threads that run through the public comments are reflected in the Council's strategic framework, focus, and recommendations for priorities for OS CER funds. Of particular value to the Council was the opportunity to engage with panelists at the listening sessions. This back-and-forth discussion enabled Council members to refine their ideas and solicit further feedback.

Before summarizing the key themes, it is useful to note that several respondents honed in directly on the reason why investments in comparative effectiveness research are important—CER matters. For example, one respondent talked about the value of and application of CER for everyone's health and health care. Another talked about how funding is crucial to reforming the practice of medicine to increase the quality, safety, cost benefits, and real world effectiveness of what providers bring to patients on a daily basis.

One theme that wove through many of the comments was the need for greater collaboration among Federal agencies, among organizations at the Federal, state and local levels, and between the public and private sectors. One respondent stated that because expertise on comparative effectiveness research resides in both public and private entities, every effort should be made to encourage public-private collaboration in the design, conduct, analysis, and reporting of CER. This discussion about collaboration dovetailed with the question of stakeholder input, including the need to ensure that patients have a defined and central role in the CER process.

Key Themes

Some respondents recommended targeted research topics; these ranged from testing the total effects of medication on the frail elderly, to testing the efficacy of a diet to treat children with seizures, to informing prescribing physicians on a wide range of what does and doesn't work well for patients with various complex conditions.

Most of the comments, however, fell into several broad categories—prioritizing the agenda, infrastructure development, research methodology and conduct, care delivery, knowledge transfer, cost, and health disparities and personalized medicine. A number of key themes and specific comments are summarized below.

Prioritizing the Agenda

A number of respondents tried to step back and look broadly at the question of how to prioritize the agenda for comparative effectiveness research and what criteria should guide decision-making in this arena. An overarching theme that echoed through many of these comments was the need to think big and look system-wide. One respondent stated that CER that is localized to a single disease may be less of a priority than questions that cross over diseases. Another talked about the need for CER to be undertaken for quality, efficiency, effectiveness, and other appropriate dimensions for health care delivery systems along the entire spectrum of systems integration, adding that the spectrum should include integrated delivery systems, multi-specialty group practices, single-specialty groups, "virtual" groups, and small medical practices.

A number of recommended areas of focus emerged. Many respondents talked about focusing on areas of major clinical significance and the greatest impact on health care delivery, including chronic conditions. One respondent specifically noted that CER on chronic diseases should focus on all relevant health care services, including medical and surgical procedures, diagnostics, and medical devices. Another respondent said that more attention is needed in the areas of post-acute and long-term care. Still others talked about the need for comparative effectiveness research on emergency care processes, and CER to evaluate regional differences in trauma care. A few people talked about studying the role of alternative treatments, including homeopathic treatments for chronic and acute disease states. Several respondents also talked about looking at conditions with the greatest impact on morbidity, and a few about doing research on conditions with the greatest impact on cost.

A few respondents discussed the need to ensure that the priorities of state and local jurisdictions be given consideration in evaluating various CER strategies. For example, one participant noted that many jurisdictions have on-going investigative agendas designed to improve program effectiveness that can be considerably amplified by Federal support, adding that such efforts would extend beyond purely clinical protocols to include the evaluation of public health, community-based, and behavioral strategies that may enhance the effectiveness of public programs.

One respondent suggested that significant resources be devoted to population-level interventions as well as patient-level effectiveness. Another respondent talked about the need for comparative effectiveness priority research areas to include critical cross-cutting research questions and cited

several examples (clinical decision-making, human-technology partnership, team coordination and continuity of care).

In addition, respondents talked about the need for Federal investments in CER to focus on health disparities and understudied sub-groups. Many of the respondents who addressed this topic talked about under-sampling of minorities in clinical trials and stressed the need for research that looks at the impact of various treatments on specific sub-groups, including women, minorities, people in rural communities, persons with disabilities, and children.

Infrastructure Development

A number of respondents honed in on the need to scale up the capacity to do comparative effectiveness research. As one respondent put it: "All healthcare reform proposals are predicated on the presumption that a robust and well-developed quality infrastructure exists. However, this is not uniformly the case."

Infrastructure capacity, as framed by the public comments, incorporates three components: human and scientific capital, organizational capacity, and data capacity.

Regarding human and scientific capital, respondents said that investments are greatly needed to enhance the skills, supply, and diversity of the research work force. One respondent pointed specifically to a dearth of researchers focused on mental health and substance abuse and treatment. Another respondent talked specifically about the need to increase the number of Hispanic health professional researchers, and suggested that HHS target Hispanic health professional, students, residents, and graduate students interested in serving in their communities.

Regarding organizational capacity, many of the comments focused on building capacity at the regional and local level. For example, one respondent talked about the role that health improvement collaboratives and chartered value exchanges can play in maintaining patient registries and other databases, and about using the information for performance reporting. A second respondent talked about the role that more community organizations could play in helping to address racial and ethnic health disparities were they to have the appropriate infrastructure and capacity.

The third critical subset of infrastructure development is data. A number of respondents talked about the need for both better data and access to data for comparative effectiveness research and decision-making. They urged the Council to access as much available data as possible, including clinical trials data, electronic health record systems, health care claims systems, administrative data, and Federal health data (including data from Medicare and Medicaid and that collected by the Veterans Health Administration). Respondents also talked about the need to invest in a coordinated effort to link public and private sector databases, as well as the need for standardized data available from the point of patient care.

Several respondents also talked about the value of registries, and the need to link data sets in order to provide valuable sources of data to examine appropriate use, effectiveness of care, cost of care, value-based health care, and other criteria. Another respondent stressed the need for

research that involves collaboration in different data environments and research that explores the use of different types of electronic health care data.

Research Methodology and Conduct

How should CER be undertaken? This is another theme that ran through many of the comments. Those who tackled this question addressed key issues that ranged from the enterprise level to guidance on study design. At the broader level, one respondent talked about the need for a broad Federal CER enterprise that spans treatment, prevention, promotion, and health-determinant interventions designed for both people and populations. Another respondent recommended adopting value of information principles and tools to prioritize CER investments on those studies where there is a greater likelihood that the research will lead to changes in practice. A third person spoke about the opportunity to fund research into "the science of CER" to build a foundation for this work.

Others talked about the scope of CER, noting that much of the research is conducted in single settings of care. One respondent, for example, noted that this poses a challenge for "generalizability," and suggested that many of the questions that remain unanswered relate to uncommon conditions or outcomes that have proven challenging to study. He recommended the use of multi-center research networks to address this issue.

Looking more closely at study design, one respondent noted that CER should continue to use a variety of study designs to generate evidence about the comparative effectiveness, comparative safety, and cost effectiveness of medical interventions. A second respondent talked about the limitations of randomized clinical trials, suggesting that the Council should also consider designs that are more common for evaluating comprehensive population-focused interventions, such as observational cross-sectional studies, quasi-experimental designs, and time series analyses. Another respondent stressed that clinical trial design and CER infrastructures must accommodate the goal of addressing health disparities. Another respondent pointed out that comparative effectiveness can at times be determined by assessing technology and using quantitative metrics rather than via an expensive and sometimes-lengthy clinical trial. A fourth respondent talked about the need to include utilization of laboratory services in order to effectively compare treatments and outcomes for major chronic disease cost drivers.

Several respondents also addressed the need for greater transparency throughout the process. They talked about the critical importance of transparency for reducing bias and rebuilding trust, and they recommended that researchers show results prior to adjustments as well as adjusted results. Respondents who tackled the issue of transparency also talked about the need to disclose in detail the methods and metrics used in any research. One respondent stressed that patients and providers need to know all the inputs that go into a research analysis so that they can weigh the costs, safety, and quality issues appropriately in each instance.

A corollary to transparency is addressing potential conflicts of interest. Respondents talked about the need to develop a strong and clear policy for conflicts of interest in both research and publishing, and suggested that funding decisions for CER should favor researchers and institutions that are focused on the public interest and do not have current conflicts. They also talked about the need for 100-percent disclosure and transparency at the outset of all conflicts by

individual researchers and institutions. One respondent specifically said that the ARRA expenditures on CER offer an opportunity to move to a platform where research funding is completely independent of other sources of funds in order to get to research that is independent, unbiased, untainted, and neither methodologically flawed nor influenced by industry.

Care Delivery

Several respondents pointed out that care delivery is critical, and that investments in CER are needed to look at how the health care delivery system should be organized and the best models for delivering care to patients. One respondent recommended that the Council invest in research that looks at optimal practice models for delivering patient care along with strategies for using information technology and clinical decision support tools to implement research findings into clinical practice. Another respondent suggested that CER is needed to look at the organization, design, and management of patient care. A third said that CER should focus on medical delivery systems and operations, resulting in information that can be leveraged to foster better clinical and cost outcomes.

Much of the discussion on care delivery was focused on people with one or more chronic conditions (e.g., diabetes). One respondent, for example, talked about the need for CER studies that compare current, more traditional models of chronic care delivery with team-based, patient-centers models that include patient education and self-care. Another respondent emphasized the need to focus research on the impact of non-medical services (e.g., providing housing) on cost-effective and clinical outcomes for chronically medically ill populations. A third person talked about CER around the role of support services (e.g., case management) in the health outcomes of people with HIV/AIDS; a fourth, about the need for CER on crisis residential services as an alternative to psychiatric hospitalization. Yet another respondent talked about the need to study the cost-effectiveness of community health worker interventions.

One respondent talked about the need to study care models that integrate primary and tertiary care. Another respondent suggested that there was a need for research into how to deliver care in a way that helps patients get the care they need, adhere to proposed treatment regimes, and prevent subsequent untoward effects of chronic diseases. Regarding adherence to treatment regimes, one respondent specifically noted that patient compliance is a seldom-accounted-for variable in CER, and he talked about the value of electronic verification devices to track compliance. Another respondent talked about the need to compare palliative care models to understand which processes of care and specific program interventions and models are the most effective.

One respondent noted that much of the literature on the impact of electronic medical records is anecdotal, and he expressed concern that people are considering the value of electronic health records without understanding the totality of what an effective system does for health care delivery. As a result, he urged that research be done to evaluate the comparative effectiveness of different types of EHR-mediated interventions. A second respondent likewise talked about the needs for research on how health information technology and EHR exchanges can be used to create more robust data sources and to help evaluate comparative effectiveness issues across a broader range of settings.

Knowledge Transfer

A number of respondents pointed out that all the data is meaningless if the information is not disseminated effectively. One respondent, for example, stressed that knowledge translation research must not be overlooked, while another respondent pointed out that both research and dissemination of research findings are essential to realizing the quality improvements and returns-on-investment that are integral to the success of comparative effectiveness research.

While respondents had different recommendations for how to approach knowledge transfer, there was a consensus that this work is critical. One respondent noted that the evidence base that is developed around clinical comparative effectiveness offers a substantial opportunity to improve value in health care if the information is disseminated and applied by physicians and patients. Others talked about the need to identify what approaches and incentives to dissemination and adoption are most effective (and under what circumstances), and when dissemination should target change at the organizational level, the community level, or the individual level. One respondent talked about cultural competence and health literacy research, and the need for both in order to change behaviors and improve lifestyles.

One respondent noted that while technology (including electronic health records) is one avenue for dissemination, other effective dissemination and translation techniques are also needed. She noted that while many strategies have been used to enhance the rate and extent of adoption of evidence-based best practices (including clinical guidelines, continuing education for health care professionals, patient education tools, and academic detailing), the approaches have not been well studied and the results are variable.

One respondent suggested that an independent body be established to disseminate comparative effectiveness research findings; others took the approach that everyone—including providers, payers, consumers, and employers—has a role to play in disseminating research results. Another respondent suggested creating a national citizens' advisory board to help HHS better understand the perspectives and values of the general public when designing and disseminating CER. Another respondent talked about the need not only to provide the evidence base for best disease prevention, health promotion, and/or clinical interventions, but also to look at how these findings can be implemented in "real-world, complex organizational settings."

Cost

Two distinct opinions about cost emerged: (1) that it should be a factor in comparative effectiveness research or (2) that it has no place in the discussion.

Those opposed to factoring cost into CER expressed concern that too often people put cost into a separate silo and make decisions without regard to efficacy, and they suggested that a focus on costs could lead to limiting access and benefits. For example, one respondent said that comparative effectiveness research should not be focused on looking for cheaper treatments, and it should not be the basis for coverage decisions. Another talked about the fear that CER results might impact physician reimbursement rates. Several respondents also expressed concern that CER could be used to restrict access to care, to deny coverage, or to reduce payments for interventions, thus undermining physician/patient decision-making and limiting patient access to treatment options.

On the flip side, other respondents felt equally strongly that cost was an integral component of informed decision-making. For example, one respondent said that information about costs enables understanding not only of the direct differences in terms of clinical outcomes but also of the value of interventions and whether they represent an efficient use of resources. Another respondent suggested that, if costs are not considered, the tradeoff in terms of lost health benefits would be too steep. Others stressed that a wide range of stakeholders—including employers, policymakers, and state and local public health departments—have said that they need cost information to make decisions.

Health Disparities and Personalized Medicine

Several respondents spoke about the related topics of the need to address health disparities within CER and support for the growth of personalized medicine. Inclusion of and attention to underrepresented sub-groups was spoken of as a means to address the problem of disparities in care. Others spoke of the importance of fostering the application of personalized medicine.

Respondents cited the need for more CER in the areas of preventive care, pediatric care and children's health, behavioral health interventions, addiction, mental disorders, and suicide prevention. One respondent pointed out that CER is needed to understand the cost and quality implications to the overall health system of continuing to under-treat conditions in systems that are siloed and distinct from mainstream health and health care. Another respondent specifically noted that the aim of personalized medicine and the mapping of the human genome is to achieve disease interventions much earlier (ideally at the point of preventing the disease from ever taking hold, he said).

One respondent stressed that CER must be mobilized to improve the health outcomes of various racial and ethnic minorities in order to close the gap that exists between the health status of some minority populations and other Americans. Others warned about relying on small, narrowly focused studies, suggesting that understanding and addressing health disparities requires a broader approach; conversely, respondents also cautioned against "one-size-fits-all" approaches that could decrease access to treatments. One respondent specifically talked about the need for research that examines health intervention outcomes across the lifespan, and for different minority and gender groups, in order to understand the effectiveness of interventions within and between population groups.

Several people talked about the need to design studies that appropriately include minority populations (see also *Prioritizing the Agenda*, above). For example, one respondent said that the design of studies must reflect the diversity of patient populations, including racial and ethnic diversity, and must communicate results in ways that reflect the differences in individual patient needs. Another respondent stressed that clinical trial design and CER infrastructures must accommodate the goal of addressing health disparities. There was also discussion more broadly about the need to build the infrastructure to address health disparities relating to people of color.

One respondent pointed to the dichotomy between studying populations and the promise of personalized medicine, asking: How can CER at a broad population level be balanced with the

goals and rapid scientific advancements in the area of personalized and stratified medicine in order to encourage the development of targeted therapies for sub-groups?

One respondent talking about personalized medicine recommended that CER studies include the evaluation of approaches to health care delivery and care management that foster the effective application of personalized medicine.

Appendix B: SUMMARY OF THE COUNCIL'S MEETINGS AND DELIBERATIONS

The following contains a summary of the Council's deliberations as they unfolded once the Council was officially convened.

April 10, 2009

The Council was presented with background information on comparative effectiveness research and briefed on CER activity at AHRQ, NIH, and VA. The Council also discussed the scope of their work and objectives.

Next, the Council began discussion of the components of the definition of CER and potential criteria for prioritization. The Council also discussed how CER and data infrastructure for CER might be categorized. Finally, the Council reviewed the timeline and discussed plan for listening sessions, including the first listening session on April 14, 2009.

April 22, 2009

The Council met to discuss what they had heard at the April 14 listening session. Members identified several key themes, including the need to outline a clear, well-delineated definition of comparative effectiveness research. They noted that participants had also talked about the need to prioritize methodology, and the fact that CER should be inclusive of all components of medical care.

Council members also noted that they had heard, loud and clear, that the Council's governance and processes must be transparent, and that the Council must incorporate input from all stakeholders to gain credibility and build trust.

Other themes that emerged from the listening session include the need to focus on patients and outcomes; the importance of incorporating diverse populations and multiple research methods; and the need for investments in infrastructure. Regarding the focus on patients and outcomes, Council members noted that participants had talked about the importance of considering patient input from the start and the fact that the results must be framed and disseminated in ways that are relevant to patients and providers. Regarding diverse populations, Council members observed that there was discussion about the need to include sub-groups with multiple chronic conditions, and the need more broadly to make CER relevant to sub-groups. Members also noted that participants had talked about the need to use a multitude of different research methodologies (not just randomized clinical trials), and to look at the Department of Veterans Affairs' experience using registries.

Regarding infrastructure, Council members observed that participants had stressed there was a need to expand, improve, and build on existing information and registries, and that perhaps this investment could lay the foundation for distributed data networks with the capability to answer many future CER questions. Members also noted that there had been discussion about the need to make data monitoring easier and more routine.

Finally, Council members talked about how they could tweak the listening session format to allow for a more robust conversation with participants.

May 1, 2009

The Council looked at the timetable for its work and the due dates for its key deliverable. The Report to Congress is due June 30, and the preliminary timetable builds in time for HHS and OMB clearance, comments, and suggested edits. The Council also briefly discussed the upcoming second listening session, slated for May 13 in Chicago.

Next, the Council briefly discussed the process for compiling the CER and data infrastructure inventories, and agreed that members would identify primary contacts in their division or agency who can work with the contractor to drive that process.

The Council's next goal was to arrive at consensus on a draft definition of comparative effectiveness research, prioritization criteria, and a categorization framework for CER. Once complete, the Council agreed to post the draft language on the hhs.gov/recovery Web site and to solicit public feedback.

To begin that work, the Council tackled the draft definition. There was considerable discussion about what the definition of CER should be. Members expressed the belief that the definition needed to be inclusive of the multiple stakeholders in the health care arena, including communities, and they also looked at what types of interventions should be called out. The Council ultimately came to consensus that they wanted a definition that was broad-based and inclusive, but that was not so detailed as to inadvertently narrow the scope of comparative effectiveness research.

The Council next turned its attention to the prioritization criteria. Before doing so, however, the Council first wrestled with the question of whether the criteria should be focused broadly or more narrowly targeted to provide guidance to the Office of the Secretary in allocating its Recovery Act funds. The Council generally felt that the criteria should be broad enough to allow the Council to make recommendations on overall funding and funding criteria.

Next, Council members discussed how to prioritize the CER criteria, including whether impact should be listed first, with feasibility and scientific merit second. One person spoke out about the need to keep the criterion on diverse populations and patient sub-groups within the top five. There was also discussion about whether knowledge gap was a criterion, or whether it should perhaps be wrapped into the criterion on impact.

The Council also looked at several potential frameworks for comparative effectiveness research, including categorization by type of CER investment, by patient sub-groups, by condition, and by type of intervention. The aim of developing a framework was to help categorize current CER activity and to identify gaps for potential future investments in CER. Council members also discussed CER centers, and agreed that Recovery Act funding could be used to support this work. One member suggested that the Council, at a future date, should discuss how to coordinate interest in CER centers across agencies.

Finally, the Council received a presentation on enhancing the inclusion of minority and other underserved populations in comparative effectiveness research. As a result, the Council agreed to establish a small workgroup co-led by NIH, AHRQ, the HHS Office of Minority Health, and the HHS Office on Disability. The workgroup will have two key tasks: (1) to develop recommendations for the inclusion of minority and other underrepresented populations in the expanded comparative effectiveness research agenda, and (2) to receive input from non-Federal groups on targeted actions.

May 8, 2009

The Council reviewed a revised definition of comparative effectiveness research and agreed to post the definition on the hhs.gov/recovery Web site on or about May 15.

Next, the Council resumed its discussion of the prioritization criteria. There was considerable discussion about whether "scientific rigor and validity" needed to be included in the threshold minimal criteria, with some members saying that it was implicit (and something already being done) and others expressing concerned about including a yes/no component to the threshold minimal criteria. The consensus of the Council was that scientific rigor and validity be included as part of a concept statement.

The Council then looked at a first draft outline of the Report to Congress. It included (1) Introduction, (2) Objectives, (3) Definition and Criteria, (4) Framework for CER, (5) Current CER and CER data infrastructure, (6) Recommendations for Priorities for OS CER Funds, and (7) Longer-term Vision and Opportunities.

Council members discussed a number of items that they believed needed to either be included or called out in the report, including concrete examples of what CER is and why it matters as well as a discussion about the full range of CER activities (and not just randomized clinical trials). There was also discussion about having a stand-alone section on high level priorities; the need to call out the roll of public/private partnerships; including a sub-section on the need for CER data to be synthesized and operationalized, along with some mechanisms for achieving this outcome; and the need to add language on sub-groups. Members also agreed to add a new section, Summary of the Listening Sessions, and to include a high-level Executive Summary.

Next, the Council began its discussion of CER priorities. To frame their discussion, members looked at four categories: primary research, dissemination of results, data infrastructure, and cross-cutting coordinated investments. One member asked, "What are the gaps that no one else can fill?" The Council agreed to continue its discussion at its next meeting.

May 22, 2009

The Council opened its meeting with a debrief from the May 13 listening session in Chicago. Members said they found the meeting both useful and exciting, and cited some themes they had heard that particularly struck them. These included the need to study chronic diseases (and to include sex, ethnicity, and race in the analysis); the idea of using theoretical models to assess how to approach a study (and to ensure the information is useable); the inclusion of mental health as a priority area; the importance of CER on pediatric populations; the importance of CER on prevention; and the need for training, and for starting to build the pipeline early.

The Council then briefly addressed next steps on the Report to Congress, including the fact that certain members would be assigned to draft specific sections of the report.

Next, the Council resumed its discussion of CER priorities where it had left off: looking at research, dissemination, data infrastructure, and cross-cutting investments. There was general consensus that OS funds should focus primarily on the latter three areas (as AHRQ and NIH are likely to make CER investments in research); there was also discussion about how to frame the priorities, including whether they should be framed around the type of CER investment or around types of diseases (e.g., people with multiple chronic illnesses, or people with disabilities and chronic illnesses). There was also specific discussion about the need to improve dissemination of research results—and a related topic, impacting practice. "If we just talk about dissemination," said one Council member, "we won't get anywhere. We need to look at the best methods for impacting practice."

There was also discussion around the question of how the Council should think about structuring its Report to Congress. At issue was whether the report should focus primarily on guidance to the HHS Secretary on how to allocate the $400 million in OS funds. In addition, the Council discussed the research time horizon, and whether ARRA monies could be used to fund projects that will have a time horizon longer than two years. One member suggested that one way to think about the question was to reframe it and ask, "Can we think about creating research centers that will be great resources into the future?"

Council members also stressed the need for the Council to address in its report the *process* for its deliberations and its recommendations, including making clear that CER investments are weighted to public health needs and responsive to the needs of decision makers. Council members suggested that some of the discussion about impacting practice might be linked to the discussion about data infrastructure investments.

May 29, 2009

The Council honed in on the details of the strategic framework for comparative effectiveness research, and the fact that it represents a comprehensive, coordinated approach to Federal investment in CER priorities that is intended to support immediate decisions for investments in CER priorities and to provide a comprehensive basis for longer-term CER investment decisions.

The Council discussed a framework that includes four major categories of activity (research, human and scientific capital, data and research infrastructure, and translation and adoption). The framework is designed to allow for investments within a single category or to cross-cut priority

themes. The Council agreed upon the categories. The Council's next step will be to determine the recommended mix among the major activities for OS funds.

The Council agreed to post on the hhs.gov/recovery Web site a copy of the broad framework diagram as well as a more detailed version to inform the public and to seek feedback on the strategic framework.

Next, the Council looked at some examples of the types of investments that might be made in the areas of infrastructure and translation and adoption. The idea of the discussion was to enable members to think about what types of projects might address gaps and further the CER enterprise.

The Council also looked briefly at an updated draft outline for the Report to Congress, and then members heard a presentation on three possible categories for investments in disability comparative effectiveness research.

June 5, 2009

The Council discussed the first draft of the Report to Congress. There was consensus that the Executive Summary needed to better frame the conversation around the value of CER to inform patients, clinicians, and other stakeholders. There was also discussion about setting out, early in the body of the report, why CER matters and how it matters to each stakeholder group. In addition, the Council agreed to add an additional appendix that contains a summary of its meetings and deliberations.

Next, the Council took up its recommendations for priorities. The discussion revolved around four key issues: the balance in spending priorities among the major activities versus cross-cutting themes; the distribution of spend priorities across the four major activities; what themes should be prioritized (and what the distribution of spend priorities should be across those themes); and whether the overall distribution makes sense vis-à-vis the prioritization criteria.

Regarding the distribution of spend priorities across the four major activities, Council members generally agreed that the majority of funding (e.g. 60 percent) should be spent on activities rather than themes. At the same time, there were lingering questions about the need to identify research gaps, implementation gaps, or both.

Regarding the distribution of spend priorities across the four major activities, the Council supported a breakdown that focuses the bulk of the funding in the areas of infrastructure (e.g. 60 percent) and translation (e.g. 20 percent). Members noted that there is a unique opportunity with ARRA funds to make significant investments in infrastructure.

Regarding potential priorities, members looked at draft lists of both priority populations and types of interventions. On the populations side, one Council member said that all of the proposed priority populations share in common that they have not traditionally been enrolled in clinical trials. There was also discussion about the need to include veterans as well as people with co-occurrence of mental health disorders along with physical comorbidities. On the interventions side, there was some discussion about the inclusion of delivery systems, and that

CER on delivery systems offers an opportunity to look at promising practices and how they might be scaled up and disseminated.

Finally, the Council was divided as to whether the bulk of OS funds should be used primarily for investments in populations or in interventions—or whether they should be equally important priorities.

June 12, 2009

The Council debriefed on what was heard in the third listening session. This generated enhancement to the common themes and some new information to be incorporated. The Council then revised the definition, threshold and prioritization criteria, and strategic framework based on the feedback from the session and the feedback received online. The Council then further discussed priority recommendations and the Report to Congress. The Council suggested edits for the Report prior to it going into clearance the next week.

Appendix C. PRELIMINARY DATA INFRASTRUCTURE AND CER BY CONDITION

The following is a preliminary inventory of examples of CER data infrastructure and CER by condition.

Person-Level Health Care Research Databases from First Inventory

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
US Federal							
Healthcare Cost and Utilization Project (HCUP)	AHRQ	Hospital information system	—	All	Y	N	Y
HIV Cost and Services Utilization Study (HCSUS)	AHRQ	Surevy & records abatsraction	2,864	HIV	Y	N	Y
AIDS Cost and Services Utilization Study (ACSUS)	AHRQ	Hospital information system	1,900	AIDS	Y	N	Y
National Vital Statistics	CDC	Surveillance program/registry data	—	All	n/a	N	N

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
National Vital Statistics—Natality	CDC	Surveillance program/registry data	4 million	All	n/a	N	Y
National Health Interview Survey	CDC	Survey	87,000	All	n/a	Y	Y
National Health and Nutrition Examination Survey	CDC	Survey	5,000	All	n/a	Y	Y
National Ambulatory Medical Care Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Hospital Ambulatory Medical Care Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Hospital Discharge Survey	CDC	Survey and records abstraction	n/a	All	N	Y	Y
National Nursing Home Survey	CDC	Survey and records abstraction	13,507	All	N	Y	Y
National Home and Hospice Care Survey	CDC	Survey and records abstraction	9,416	All	N	Y	Y
Chronic Condition Data Warehouse	CMS	Administrative claims database, enrollment data, health assessment data, prescription drug event data	45 million	All	Y	Y	Y
Hospice Standard Analytical File (Hospice SAF)	CMS	Administrative claims database	—	All	Y	Y	?

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Medicaid Statistical Information System Personal Summary File (MSIS Personal Summary File)	CMS	Administrative claims database, EMR/EHR system	—	All	Y	Y	Y
National Claims History (NCH) 100% Nearline Fil	CMS	Administrative claims database	—	All	Y	Y	?
MEDPAR Claims Data	CMS	Administrative claims database	—	All	Y	Y	Y
MMA Part D Claims Data	CMS	Pharmacy claims database	25 million	All	Y	Y	Y
Sentinel System	FDA	Surveillance program/registry data	N/A	n/a	N	Y	N
SEER (Surveillance Epidemiology and End Results)	NCI	Surveillance program/registry data	11.4 million	Cancer	Y	N	Y
SEER-Medicare database	NCI, CMS	Administrative claims database, Surveillance program/registry data	3.3 million	Cancer	Y	Y	N
Cancer Research Network (CRN)	NCI, AHRQ	Administrative claims database, EMR/EHR system	—	Cancer	Y	Y	N
Computerized Patient Record System (CPRS)	VA	EMR/EHR system	4.2 million	All	Y	N	N
Diabetes Epidemi-ology Cohort	VA	Surveillance program/registry data	> 4,800	Diabetes	Y	Y	Y

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Hepatitis C Registry	VA	Surveillance program/registry data	>60 K	Hepatitis C	Y	N	Y
Immunological Case Registry	VA	Surveillance program/registry data	>15 K	HIV	Y	N	Y
Dementia Registry	VA	Surveillance program/registry data	>150 K	Dementia	Y	N	N
National Surgery Quality Improvement Program	VA	Surveillance program/registry data	>1 Million	All major surgery	Y	Y	Y
Scientific Registry of Transplant Recipients (SRTR)	HRSA	Transplant registry and outcomes data		Organ specific	Y	Y	Y
Pediatric Emergency Care Applied Research Network (PECARN) CDMCC*	HRSA	Emergency medical services for children	800,000 + patients	Emergency Services to Children	Y	Y	Y
AIDS Drug Assistance Program (ADAP)	HRSA	Care Program Registry Data	—	HIV/AIDS	Y	Y	N
US Private Sector							
National Oncologic PET Registry (NOPR)	Academy of Molecular Imaging	Intervention program data	>100,000	Cancer	Y	Y	?
Cerner Health Facts Database	Cerner	EMR/EHR system	—	All	Y	Y	Y
GE Centricity	GE	EMR/EHR system	10 million	All	Y	N	Y

* Central Data Management and Coordinating Center

Database	Owner	Data Type	No. Lives	Disease Area	Data Linkable at Patient Level	Data Linkable to External Data	Researcher Ready
Ingenix Research Data Mart (RDM) Database	Ingenix	Administrative claims database	>39 million	All	Y	Y	Y
Premier Perspective Data Warehouse	Premier	Administrative claims database	—	All	Y	Y	Y
MarketScan Data Warehouse	Thomson-Reuters	EMR/EHR system	—	All	Y	N	N
International Databases							
General Practice Research Database (GPRD)	NHS (UK)	EMR/EHR system	> 3.6 million	All	Y	Y	Y
NHS Care Records Service (CRS)	NHS (UK)	EMR/EHR system	2 million	All	Y	N	Y
The Health Improvement Network (THIN)	INPS and EPIC (UK)	EMR/EHR system	—	All	Y	Y	Y

Priority Diseases/Conditions in CER

Priority Diseases/Conditions	AHRQ (n=178)	NIH (n=513)	DoD (n=26)	VHA (n=106)	Total (n=823)
Arthritis and non-traumatic joint disorders	6%	1%	0%	3%	**2%**
Cancer	10%	7%	23%	7%	**8%**
Cardiovascular disease, including stroke and hypertension	20%	10%	4%	23%	**13%**
Dementia, including Alzheimer's Disease	1%	1%	0%	3%	**1%**
Depression and other mental health disorders	8%	16%	8%	18%	**14%**
Developmental delays, attention-deficit hyperactivity disorder, and autism	4%	1%	0%	0%	**1%**
Diabetes mellitus	11%	11%	0%	8%	**10%**
Functional limitations and disability	8%	4%	15%	7%	**5%**

Infectious diseases including HIV/AIDS	3%	11%	0%	6%	**8%**
Obesity	1%	3%	0%	2%	**3%**
Peptic ulcer disease and dyspepsia	0%	0%	0%	0%	**0%**
Pregnancy, including preterm birth	1%	4%	0%	0%	**2%**
Pulmonary disease/asthma	5%	3%	0%	4%	**3%**
Substance abuse	2%	19%	0%	9%	**14%**
Other	20%	11%	50%	12%	**14%**

*Studies focusing on patients with more than one priority disease or condition are counted in applicable rows..
**NIH 2008 plus NIH multi-year sample.

Appendix D. COUNCIL LIST AND STAFF SUPPORT

1. Carolyn Clancy, MD	AHRQ
2. Peter Delaney, PhD, LCSW-C	SAMHSA
3. Ezekiel Emanuel, MD, PhD	OMB
4. Jesse Goodman, MD, MPH	FDA
5. Garth Graham, MD, MPH	Office of Minority Health
6. Anne Haddix, PhD	CDC
7. Deborah Hopson, PhD, RN	HRSA
8. David Hunt, MD	ONC
9. Michael Kilpatrick, MD	Dept of Defense
10. Joel Kupersmith, MD	Dept of VA
11. Michael Marge, Ed.D.	Office of Disability
12. Elizabeth Nabel, MD	NIH
13. James Scanlon, PhD	ASPE
14. Neera Tanden, JD	Office of the Secretary
15. Tom Valuck, MD, MHSA, JD	CMS

Executive Director: Patrick Conway, MD, MSc
Deputy Executive Director: Cecilia Rivera Casale, PhD

Alternates to the Council participating: Kelley Brix, Margaret Cary, Rosaly Correa-de-Araujo (replaced Michael Marge on Council June 12th), Elisabeth Handley, Lynn Hudson, Michael Millman

Contributors to Council and Report: Kate Goodrich, Lauren Hunt, John Poelman, Daria Steigman, Caroline Taplin, Jordan VanLare.

Appendix E. THE AMERICAN RECOVERY AND REINVESTMENT ACT STATUTE RELATED TO CER AND COUNCIL

Appropriations

For an additional amount for 'Healthcare Research and Quality' to carry out titles III and IX of the Public Health Service Act, part A of title XI of the Social Security Act, and section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, $700,000,000 for comparative effectiveness research: *Provided,* That of the amount appropriated in this paragraph, $400,000,000 shall be transferred to the Office of the Director of the National Institutes of Health ('Office of the Director') to conduct or support comparative effectiveness research under section 301 and title IV of the Public Health Service Act: *Provided further,* That funds transferred to the Office of the Director may be transferred to the Institutes and Centers of the National Institutes of Health and to the Common Fund established under section 402A(c)(1) of the Public Health Service Act: *Provided further,* That this transfer authority is in addition to any other transfer authority available to the National Institutes of Health: *Provided further,* That within the amount available in this paragraph for the Agency for Healthcare Research and Quality, not more than 1 percent shall be made available for additional full-time equivalents.

In addition, $400,000,000 shall be available for comparative effectiveness research to be allocated at the discretion of the Secretary of Health and Human Services ('Secretary'): *Provided,* That the funding appropriated in this paragraph shall be used to accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that: (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions; and (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data: *Provided further,* That the Secretary shall enter into a contract with the Institute of Medicine, for which no more than $1,500,000 shall be made available from funds provided in this paragraph, to produce and submit a report to the Congress and the Secretary by not later than June 30, 2009, that includes recommendations on the national priorities for comparative effectiveness research to be conducted or supported with the funds provided in this paragraph and that considers input from stakeholders: *Provided further,* That the Secretary shall consider any recommendations of the Federal Coordinating Council for Comparative Effectiveness Research established by section 804 of this Act and any recommendations included in the Institute of Medicine report pursuant to the preceding proviso in designating activities to receive funds provided in this paragraph and may make grants and contracts with appropriate entities, which may include agencies within the Department of Health and Human Services and other governmental agencies, as well as private sector entities, that have demonstrated experience and capacity to achieve the goals of comparative effectiveness research: *Provided further,* That the Secretary shall publish information on grants and contracts awarded with the funds provided under this heading within a reasonable time of the obligation of funds for such grants and contracts and shall disseminate research findings from such grants and contracts to clinicians, patients, and the general public, as appropriate: *Provided further,* That, to the extent feasible, the Secretary shall ensure that the recipients of the funds provided by this paragraph offer an opportunity for public comment on

the research: *Provided further,* That research conducted with funds appropriated under this paragraph shall be consistent with Departmental policies relating to the inclusion of women and minorities in research: *Provided further,* That the Secretary shall provide the Committees on Appropriations of the House of Representatives and the Senate, the Committee on Energy and Commerce and the Committee on Ways and Means of the House of Representatives, and the Committee on Health, Education, Labor, and Pensions and the Committee on Finance of the Senate with an annual report on the research conducted or supported through the funds provided under this heading: *Provided further,* That the Secretary, jointly with the Directors of the Agency for Healthcare Research and Quality and the National Institutes of Health, shall provide the Committees on Appropriations of the House of Representatives and the Senate a fiscal year 2009 operating plan for the funds appropriated under this heading prior to making any Federal obligations of such funds in fiscal year 2009, but not later than July 30, 2009, and a fiscal year 2010 operating plan for such funds prior to making any Federal obligations of such funds in fiscal year 2010, but not later than November 1, 2009, that detail the type of research being conducted or supported, including the priority conditions addressed; and specify the allocation of resources within the Department of Health and Human Services: *Provided further,* That the Secretary, jointly with the Directors of the Agency for Healthcare Research and Quality and the National Institutes of Health, shall provide to the Committees on Appropriations of the House of Representatives and the Senate a report on the actual obligations, expenditures, and unobligated balances for each activity funded under this heading not later than November 1, 2009, and every 6 months thereafter as long as funding provided under this heading is available for obligation or expenditure.

Sec. 804. Federal Coordinating Council for Comparative Effectiveness Research

(a) ESTABLISHMENT— There is hereby established a Federal Coordinating Council for Comparative Effectiveness Research (in this section referred to as the 'Council').

(b) PURPOSE— The Council shall foster optimum coordination of comparative effectiveness and related health services research conducted or supported by relevant Federal departments and agencies, with the goal of reducing duplicative efforts and encouraging coordinated and complementary use of resources.

(c) DUTIES— The Council shall—

(1) assist the offices and agencies of the Federal Government, including the Departments of Health and Human Services, Veterans Affairs, and Defense, and other Federal departments or agencies, to coordinate the conduct or support of comparative effectiveness and related health services research; and

(2) advise the President and Congress on—
(A) strategies with respect to the infrastructure needs of comparative effectiveness research within the Federal Government; and
(B) organizational expenditures for comparative effectiveness research by relevant Federal departments and agencies.

(d) MEMBERSHIP—

(1) NUMBER AND APPOINTMENT— The Council shall be composed of not more than 15 members, all of whom are senior Federal officers or employees with responsibility for health-related programs, appointed by the President, acting through the Secretary of Health and Human Services (in this section referred to as the 'Secretary'). Members shall first be appointed to the Council not later than 30 days after the date of the enactment of this Act.

(2) MEMBERS—
(A) IN GENERAL— The members of the Council shall include one senior officer or employee from each of the following agencies:
(i) The Agency for Healthcare Research and Quality.
(ii) The Centers for Medicare and Medicaid Services.
(iii) The National Institutes of Health.
(iv) The Office of the National Coordinator for Health Information Technology.
(v) The Food and Drug Administration.
(vi) The Veterans Health Administration within the Department of Veterans Affairs.
(vii) The office within the Department of Defense responsible for management of the Department of Defense Military Health Care System.
(B) QUALIFICATIONS— At least half of the members of the Council shall be physicians or other experts with clinical expertise.
(3) CHAIRMAN; VICE CHAIRMAN— The Secretary shall serve as Chairman of the Council and shall designate a member to serve as Vice Chairman.

(e) REPORTS—

(1) INITIAL REPORT— Not later than June 30, 2009, the Council shall submit to the President and the Congress a report containing information describing current Federal activities on comparative effectiveness research and recommendations for such research conducted or supported from funds made available for allotment by the Secretary for comparative effectiveness research in this Act.

(2) ANNUAL REPORT— The Council shall submit to the President and Congress an annual report regarding its activities and recommendations concerning the infrastructure needs, organizational expenditures and opportunities for better coordination of comparative effectiveness research by relevant Federal departments and agencies.

(f) STAFFING; SUPPORT— From funds made available for allotment by the Secretary for comparative effectiveness research in this Act, the Secretary shall make available not more than 1 percent to the Council for staff and administrative support.

(g) RULES OF CONSTRUCTION—

(1) COVERAGE— Nothing in this section shall be construed to permit the Council to mandate coverage, reimbursement, or other policies for any public or private payer.

(2) REPORTS AND RECOMMENDATIONS— None of the reports submitted under this section or recommendations made by the Council shall be construed as mandates or clinical guidelines for payment, coverage, or treatment.

Title VIII—Departments of Labor, Health And Human Services, and Education, and Related Agencies

200 INDEPENDENCE AVENUE, S.W.

WASHINGTON, DC 20201

Federal Coordinating Council for Comparative Effectiveness Research

April 10, 2009

Agenda

1. Comparative Effectiveness Research Background and Draft Initial Framework
2. Comparative Effectiveness Research Current Portfolios and Future Plans for CER
   - AHRQ
   - NIH
   - VA
3. Listening Session Format
4. Other

Statute Description of Comparative Effectiveness Research

- Statute states that CER funding “shall be used to accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that:
  - (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions;
  - (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data.”

Potential Interventions in CER

- Interventions include at least the following:
  - Medications
  - Devices
  - Surgery or procedures
  - Behavioral interventions (e.g. for smoking)
  - System interventions (e.g. discharge process A vs. B from hospital) – included in CER? how to scope?
- Reasonable to compare across categories at times (e.g. medication vs. surgery or behavioral intervention vs. medication)

Outcomes

- Examples of Potential Outcomes
  - Clinical endpoints (e.g. mortality and morbidity, adverse events)
  - Surrogate endpoints (HbA1c, biomarkers)
  - Functional endpoints (e.g. quality of life, symptom severity, and patient satisfaction)
  - Economic outcomes (e.g. cost effectiveness)

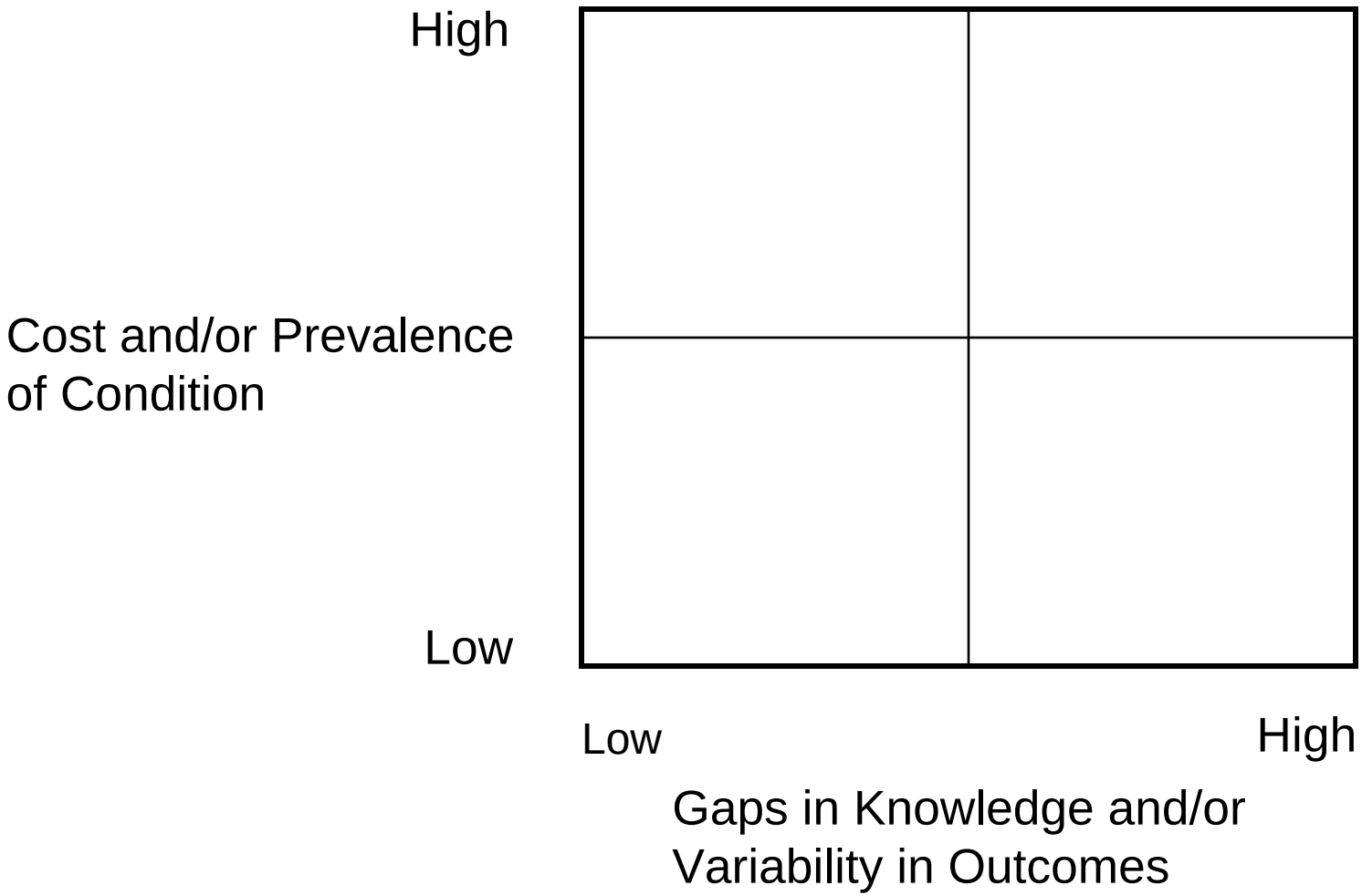
Objectives for FCC

1. Foster optimum coordination of comparative effectiveness research conducted or supported by relevant Federal departments.
2. Formulate recommendations for how best to spend the $400 million appropriated to Office of Secretary as part of Report to Congress.

Need:

1. Framework for setting priorities
2. Categorization scheme for current CER and to help evaluate future investments to fill gaps

Draft Simplified Framework for Priority Setting



Other Criteria for Priority Setting

- Responsiveness to expressed needs / preferences of patients and clinicians
- Potential to evaluate comparative effectiveness in diverse patient populations and patient segments
- Cost of investment versus potential importance of finding(s)
- Balance of short term and longer term projects
- Other?

Types of CER Investment

- Consistent with ARRA
  1. Comparative Effectiveness Research
  2. Investment in electronic clinical data networks and registries that support evaluation of outcomes and comparative effectiveness
- Strategic issue for Council: Balance of investment in specific research projects vs. investment in electronic data resources

Major Categories of CER

Category	Advantage	Disadvantage
Evidence Synthesis/Systematic Reviews	• Least time and cost • Summary of Evidence	• Need substantial primary evidence to summarize
Observational Studies - Administrative data - Electronic clinical data	• Less time and cost than RCT's • Evaluate large volumes of data so easier to investigate patient subgroups (e.g. elderly Hispanic patients) • "Real world" effectiveness	• Potential for bias in results
Randomized Controlled Trials	• No selection bias so most definitive results	• Sometimes exclude or fail to have sufficient numbers to inform decisions for patient sub-populations • Expensive and long time

Example Potential Priority Investments and Time Needed for Results

Potential Investment	Relative Time Horizon
R1: Evidence Synthesis	Short
R2: Observational studies	Medium
R3: RCT	Long
I1: Linked administrative data	Short
I2: Electronic Clinical Data Networks	Medium - Long
I3: Patient Registries (e.g. transplant, patients with devices)	Medium

Major Strategic Issues

- Balance of investments (e.g. research vs. data infrastructure; and type of investment within categories)
- Scheme for Priority Setting
- Coordination of OS funding with other investments in HHS and across Federal govt
- Other?

Agenda for Listening Session

1.	Background on Council and CER	2:00 – 2:15
2.	Panel 1 (10 panelists for 3 min comment each, Council questions)	2:15 – 3:00
3.	Panel 2 (10 panelists for 3 min comment each, Council questions)	3:00 – 3:45
4.	Panel 3 (10 panelists for 3 min comment each, Council questions)	3:45 – 4:30
5.	Time for Open Public Comment	4:30 – 4:55
6.	Closing	4:55 – 5:00

Format for Listening Session

- Room 800 Humphrey, overflow in Great Hall, 14th 2-5pm
- Webcast and call-in capability
- 3 panels of 10 pre-selected panelists and then open public comment (volunteer at registration desk on day of session – 7 open slots max)
- Press table set-up
- Council will ask questions of panelists (not vice-versa)
- Strict 3 minute time limit for each public comment and then 15 minutes or less of Council questions
- Will assign primary 5 Council member group to be responsible for asking questions of each panel but any Council member can ask a question

Reminder Activities and Timeline

Activity	Description	Date
Opening Meeting	Introductions, Scope, Objectives Discussion	March 27th
1st Working meeting	Presentation and Discussion of NIH, AHRQ, and VA CER work-to-date and future plans	April 10th
1st Listening Session (DC)	3 hours to hear external stakeholders	April 14th
2nd Working meeting	Outline of Report to Congress Beginning presentation and discussion of proposals for OS funds from CER workgroup	May 1st
2nd Listening Session (DC)	2-3 hours to hear external stakeholders	May 6th
3rd Listening Session (midwest - chicago?)	3 hours (core of interested Council members could travel) Could coordinate with Regional/State Office (e.g. Governor)	May 14th
3rd Working Meeting	Discussion of Inventory of current CER within strategic framework Presentation of proposals for OS funds	May 29th
4th Working Meeting	Final presentation of proposals Begin Discussion of Proposals and funding priority recommendations	June 12th
5th Working Meeting	Review and Suggest Edits for Draft Report to Congress on Current CER and recommendations for OS funding of CER	June 26th
Finalize Report	Final Draft of Report to Congress	Week of June 30th
Monthly Meetings	Updates on Progress and further Recommendations on CER funding and priorities. Review overall funding within strategic framework to ensure addressing gaps	July 10th and ongoing

Federal Coordinating Council for Comparative Effectiveness Research

June 12, 2009

Agenda

1. Brief Debrief on Listening Session
2. Discussion of Report to Congress
3. Finalize Definition, Prioritization Criteria, and Framework
4. Review Next Steps and Timeline
5. Other?

Listening Session Debrief

- Thoughts on feedback from panelists on definition, criteria and framework
- Any new major themes or issues?

Definition

Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in “real world” settings. The purpose of this research is to improve health by developing and disseminating information to patients, providers, and decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances. To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations and sub-groups. Defined interventions compared may include medications, procedures, medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies. This research necessitates the development, expansion, and use of a variety of data sources and methods to assess comparative effectiveness and actively disseminate the results.

Minimum Threshold Criteria

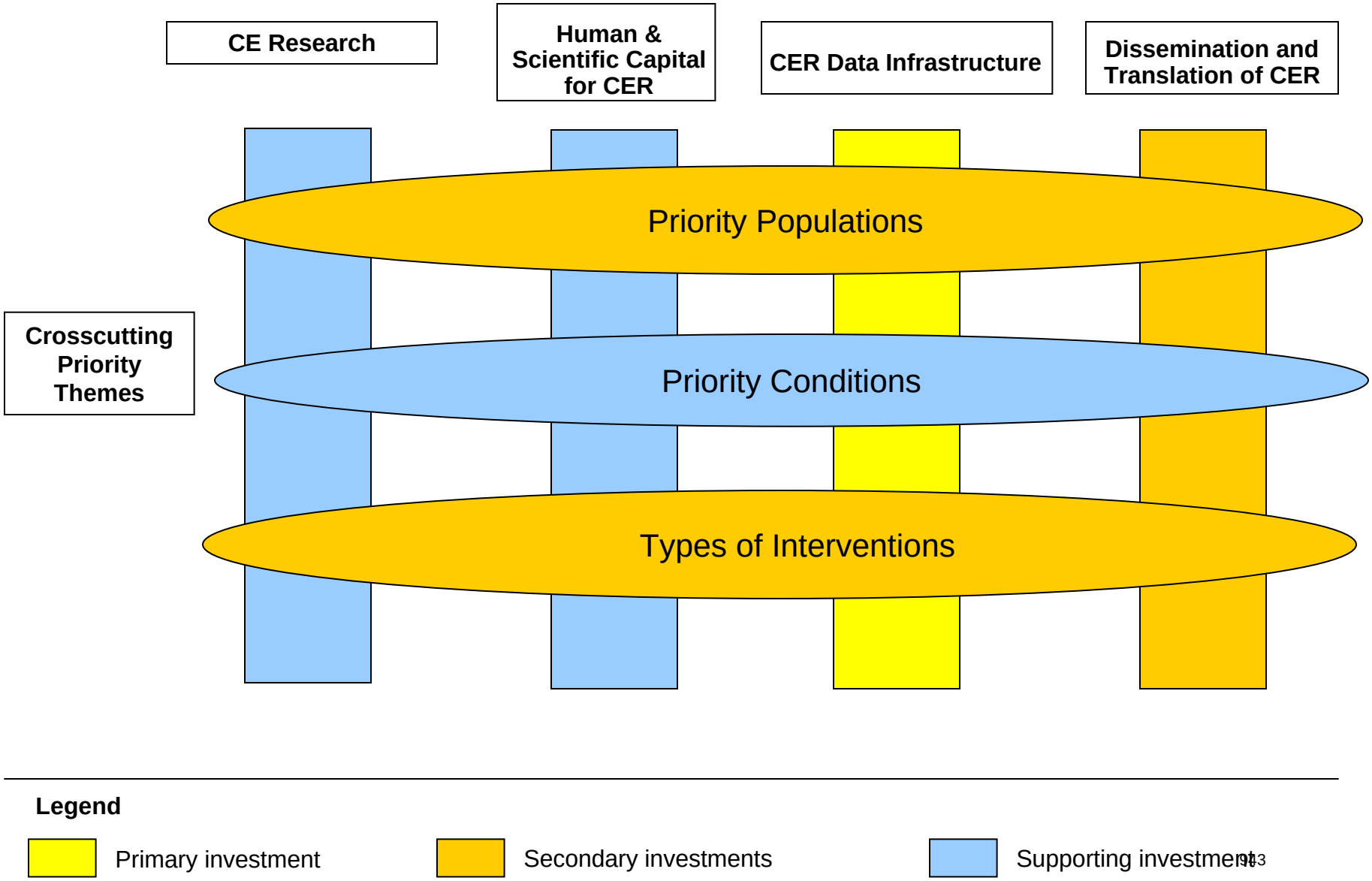
1. Included within statutory limits of Recovery Act and FCC definition of CER
2. Potential to inform decision-making by patients, clinicians, and other stakeholders.
3. Feasibility of research topic (including time necessary for research)

Prioritization Criteria

The criteria for scientifically meritorious research and investments are:

- Potential Impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm)
- Potential to evaluate comparative effectiveness in diverse populations and patient sub-groups and engage communities in research
- Uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- Addresses need or gap unlikely to be addressed through other funding mechanisms
- Potential for multiplicative effect (e.g. lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government)

Strategic Framework



Next Steps and Plan for Clearance

Mon June 15th	Report enters clearance at HHS
Wed June 17th	Comments due back
Thurs June 18th	Report enters clearance at OMB
Friday June 19th	Council meeting (discuss revisions)
Mon June 22nd	Comments due back
Tues June 23rd	Revision Sent to HHS and OMB
Thurs June 25th	Final Comments due back
Friday June 26th	Council meeting
Mon June 29th	Final Report Complete
Tues June 30th	Formatted, printed and sent out

Federal Coordinating Council for Comparative Effectiveness Research

June 19, 2009

Agenda

1. NIH update
2. Report to Congress and Clearance Process
3. Next Steps
4. Communications
5. Other?

Report

- Most major changes were to inventory
- Multiple other changes from divisions incorporated
- Overall positive feedback
- Other issues?

Plan for Clearance

Mon June 15th	Report enters clearance at HHS
Wed June 17th	Comments due back
Thurs June 18th	Report enters clearance at OMB
Friday June 19th	Council meeting
Mon June 22nd	OMB Comments due back
Tues June 23rd	Revision Sent to HHS and OMB
Thurs June 25th	Final Comments due back
Friday June 26th	Council meeting
Mon June 29th	Final Report Complete
Tues June 30th	Formatted, printed and sent out

NEXT STEPS

- Map proposals to strategic framework
- Identify proposals that are complementary and/or that overlap and combine as appropriate
- Identify gaps in set of proposals vis-à-vis high-level strategy articulated in report to congress
- Work with divisions/authors and work group to add or modify proposals as necessary in preparation for funding decisions
- Begin building more detailed implementation and budget plans for proposals (including staffing/skill sets necessary to execute)

Communication Plan

- Press
- Legislative
- To provider and medical community

Federal Coordinating Council for Comparative Effectiveness Research

July 17, 2009

Agenda

1. Debrief on FCC Report Release
2. Debrief on IOM report and cross-walk process
3. Discussion of process for OS funding and combined CER operational plan
4. Other?

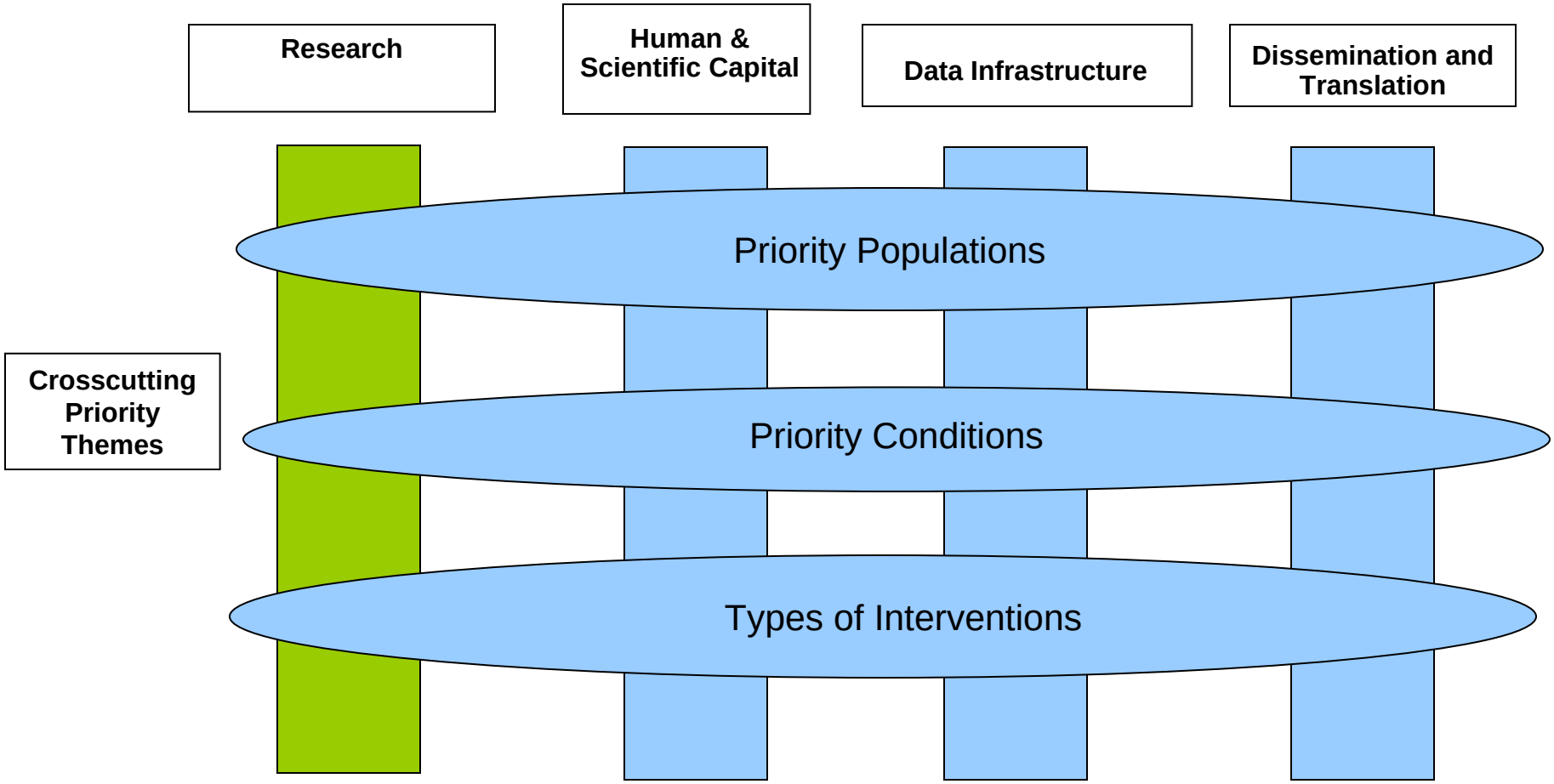
Debrief FCC report

- Hill briefings on June 29th
- Press call on June 29th
- Reaction thus far positive
- Thoughts?

Debrief IOM report

- Reactions from IOM briefing?

IOM Recommendations for CER



Legend

Specific CER funding priorities outlined

General Considerations only

IOM General Recommendations

- Prioritization of CER topics should be sustained and continuous and informed by topic briefs, such as current state of research
- Obtain public input and maintain transparent processes
- Regular reporting of activities and recommendations is necessary
- HHS Secretary should establish a mechanism—such as a coordinating advisory body – to strategize, organize, monitor, and evaluate the implementation and impact of the CER program.
- CER program should involve consumers, patients, and caregivers
- Devote sufficient resources to methods
- Develop and promote robust data and information systems
- Develop and support the CER workforce
- Promote rapid adoption of CER findings and conduct research to identify the most effective strategies for dissemination

IOM cross-walk

- We are in process of cross-walking IOM 100 research topics to completed, ongoing, and planned CER (most of these fall under purview of AHRQ and NIH)

OS funding

- Based on Council recommendations, IOM report, public input, and work done within HHS by ARRA CER workgroup, portfolio of funding options presented for Secretary's consideration
- These directly link to strategic framework and Council recommendations

Combined CER plan

- Secretary is to submit combined operational plan for $1.1 billion of CER funding by July 30th to Congress
- Draft of this plan is under review within the Department and then will be shared with OMB
- Once finalized, it will also be shared with Council

Council Next Steps

- ARRA stated that, “The Council shall submit to the President and Congress an annual report regarding its activities and recommendations concerning the infrastructure needs, organizational expenditures and opportunities for better coordination of comparative effectiveness research by relevant Federal departments and agencies.”
- This will require intermittent updates and meetings. The frequency of updates remains to be determined.
- Potential next meeting, likely in August, would be presentation of ARRA CER funding

Federal Coordinating Council for Comparative Effectiveness Research

First Public Listening Session

April 14th, 2009

Agenda for Listening Session

1.	Background on Council and CER	2:00 – 2:15
2.	Panel 1	2:15 – 3:00
3.	Panel 2	3:00 – 3:45
4.	Panel 3	3:45 – 4:30
5.	Open Public Comment	4:30 – 4:55
6.	Closing	4:55 – 5:00

Objectives of Listening Session

- Opportunity for Council to hear from diverse set of stakeholders
- Obtain public input on priorities and how Council should approach its mission
- Solicit input on concerns about comparative effectiveness research and the Council's role
- Listen to ideas on how this research can empower patients and providers and improve care for all Americans

Panels and Open Comment

- Each of the first 3 panels will include 10 panelists who will give public comment for up to 3 minutes
- After entire panel gives comments, Council members will have opportunity to ask the panelists questions
- Period of open public comment at end with as many speakers as time allows

Composition of Council

1.	Carolyn Clancy, MD	AHRQ
2.	Peter Delaney, PhD, LCSW-C	SAMHSA
3.	Ezekiel Emanuel, MD, PhD	OMB
4.	Jesse Goodman, MD, MPH	FDA
5.	Garth Graham, MD, MPH	Office of Minority Health
6.	Anne Haddix, PhD	CDC
7.	Deborah Hopson, PhD, RN	HRSA
8.	David Hunt, MD	ONC
9.	Michael Kilpatrick, MD	Dept of Defense
10.	Joel Kupersmith, MD	Dept of VA
11.	Michael Marge, Ed.D.	Office of Disability
12.	Elizabeth Nabel, MD	NIH
13.	James Scanlon, PhD	ASPE
14.	Neera Tanden, JD	Office of Secretary
15.	Tom Valuck, MD, MHSA, JD	CMS

Executive Director, Patrick Conway, MD, MSc

ARRA Statute Description of Comparative Effectiveness Research

- Statute states that CER funding "shall be used to accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that:
  - (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions;
  - (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data."

Reporting Requirement for Council

- Not later than June 30, 2009, the Council shall submit to the President and the Congress a report containing information describing current Federal activities on comparative effectiveness research and recommendations for comparative research funds allocated to the Secretary

Other CER reports in Statute

1. IOM report on national priorities for CER research by June 30, 2009.
2. The Secretary, jointly with AHRQ and NIH, shall provide Congress an operating plan for all CER funds not later than July 30, 2009.

Listening Sessions

- Complete transcript of today's session will be made available on HHS website
- FCC will be taking written public comments at least through end of May
- Plan for two more listening sessions within the next 6 weeks
- Encourage people to give public comment at listening session and/or submit written comments

Federal Coordinating Council
for Comparative Effectiveness Research
Working Meeting
June 5, 2009
1:30-3:30 p.m.

Counsel Members Present

Elizabeth Nabel (NIH)
Executive Director)
Tom Valuck (CMS)
Director)
Neera Tanden (Office of the Secretary)
Carolyn Clancy (AHRQ)
Ezekiel Emanuel (OMB)
Garth Graham (Office of Minority Health)
Michael Kilpatrick (DOD)
Joel Kupersmith (VA)
Michael Marge (Office on Disability)
James Scanlon (Planning & Evaluation)

Council Staff Present

Patrick Conway (Council

Cecelia Casale (Council Deputy

Non-Council Members Present

Kevin Hennessy (SAMHSA)
Margaret Cary (VA)
Brian Alexander (VA)
Gina Clemons (VA)
Kathy Kendrick (AHRQ)
Mike Millman (HRSA)
Margaret Cary (VA)
Jean Slutzsky (AHRQ)
Cliff Goodman (Lewin)

Next Listening Session

Patrick Conway, Council Executive Director, chaired the meeting. He noted that the third listening session would be June 10 from 12-3 p.m. He said that panelists had been asked to respond to the draft definition, criteria, and framework for comparative effectiveness research. As a result, he suggested that the next draft of the report would like contain some slight revisions, based on the public input, to the definition, criteria, and framework.

Discussion of Report to Congress

Conway asked Council members to please send him any specific edits (substantive and/or editorial) by close of business on June 8. Conway added that the inventory (both research and data infrastructure) was due to the Council on June 10.

Conway then briefed the Council on his meeting with ASPA earlier in the week. He noted that ASPA has requested that the Executive Summary be reworked to gear the summary to a lay audience, and to talk more directly about the value of CER to inform

patients and clinicians. He added that ASPA had expressed some concern about messaging, particularly related to cost, overuse, underuse, and misuse.

Conway next asked the Council to provide feedback on the first draft of the report. Are there major areas that are lacking, or other items that we need to discuss? he asked.

The Council discussed what had been written, and what they felt needed to be strengthened. Council members agreed that greater focus was needed in the report on the unique opportunity afforded by the $400 million in OS funds—in the context of to the broader $1.1 billion for CER.

A Council member expressed the need for a clear statement up front that addresses why CER matters. He added that there was a lot of language about populations, but not necessarily tying the discussion to the fact that CER is going to make everyone's life better. Another member agreed, suggesting that the report needed to start by talking about why each stakeholder group needs CER. He added that it was important to talk about why this work is being done and what it will add for both individuals and the whole system. A third member stressed that the Report to Congress is meant to be a framework from which decisions will be made (and guidance for the Secretary)—and not a spend plan.

One member noted that he hoped that the CER inventory would include the work being done by the Veterans Administration.

There was some discussion about conditions, and whether a list of specific conditions should or should not be included in the report. Some of the discussion revolved around whether all the conditions listed in the draft met the Council's priority criteria; other discussion focused on the difficulty of prioritizing conditions for inclusion. One member pointed out that the IOM report will include priority conditions, which perhaps argues against the Council doing the same. In the end, the Council agreed not to list specific conditions but to focus instead on looking at gap areas.

There was also discussion about including the meeting minutes as an appendix to the report. The Council agreed to provide a summary of the minutes to provide greater transparency about the Council's process and deliberations.

Recommendations for Priorities

Next, Conway laid out four basic questions regarding recommendations for priorities, and he walked the Council through each:

1. What should be the balance in spending priorities among the major activities vs. cross-cutting themes?
2. What should be the distribution of spend priorities across the four major activities?
3. What themes should be priorities? What should be the distribution of spend priorities across those themes?
4. Does the overall distribution make sense vis-à-vis the prioritization criteria?

1. Balance in spending priorities among the major activities vs. cross-cutting themes.

The Council looked at three broad options for spending priorities. One Council member expressed an immediate preference for Option 1 (60-80 percent in activities, and 20-40 percent in themes). He noted that NIH's work was more theme-based, and he suggested that the Council needed to look at the missing pieces in infrastructure and translation. Another member agreed. A third member suggested that, rather than thinking of the four major categories as discrete buckets, perhaps the Council should think of them as a pathway for analyzing gaps. As an example, he said that the answer for getting research in one gap area (such as minority men with developmental disabilities) may be to have better CER data infrastructure.

While the Council generally supported a 60:40 ratio of activities to themes, there was still a lingering question about the need to identify (a) research gaps; (b) implementation gaps; and (c) both. One person cautioned that there is likely no single standard for identifying gaps as the aim is to have a CER enterprise that is responsive to the needs of multiple stakeholders.

2. Distribution of spend priorities across the four major activities.

The Council quickly honed in on a distribution that puts the majority of the funding into the areas of infrastructure and translation. There was a consensus about allocating about 60 percent of the funds to infrastructure and 20 percent to translation (with the remainder split between research and human and scientific capital). One member noted that this funding stream offered a rare opportunity to make substantial investments in infrastructure. Others talked about the fact the some investments in human and scientific capital are needed now and about the need for some allocations for primary research (e.g.. centers and consortia).

The Council also noted that it will be important, in the Report to Congress, to talk about why the Council believes that infrastructure is a key issue and that large investments in this category reflect a changing approach to CER.

3. What themes should be priorities?
And what should be the distribution of spend priorities across those themes?

Looking at the draft list of priority populations, one Council member suggested that veterans should be included because they have unique conditions (e.g., post-traumatic stress disorder) and multiple chronic conditions. Another suggested adding another group—people with a co-occurrence of mental health disorders with physical

co-morbidities. A third member pointed out that the rationale for including most of these groups is that they have not traditionally been enrolled in clinical trials.

Regarding interventions, there was some discussion about the inclusion of delivery systems. While members agreed that it was important to include, they noted that this is a different approach than the traditional CER. One person noted that, given the president's interest in savings and the cost-effectiveness of system design, perhaps language needed to be included in the Report to Congress that talks about this being a new and important area of research. One person suggested talking about delivery systems in terms of efficacy and promising practices, and how promising delivery systems might be scaled and spread.

There was also some discussion about procedures, and the fact that there is very little research into most procedures, including very few efficacy-based clinical trials. Wrapping up the discussion of interventions, one Council member noted that many of the interventions seemed to be population health activities and that research in this area could be used to advise public health departments.

4. Does the overall distribution make sense vis-à-vis the prioritization criteria?

Finally, Conway opened up the discussion of spending priorities across the themes (i.e., should investments in themes be focused primarily on interventions or on populations—or somewhere in between?)

The Council wrestled with the issue, with some members falling on the side of investing more heavily in populations while others suggested that the focus of OS funds should be on interventions. One Council member pointed out that there is already a lot of interest and work being done on populations; another suggested that both are important priorities.

Next Steps and Timeline

Wrapping up the meeting, Conway reminded the Council of next steps and the timeline for getting the report cleared by HHS and OMB.

Federal Coordinating Council
for Comparative Effectiveness Research
Working Meeting
June 12, 2009
1:30-3:30 p.m.

Counsel Members Present

Elizabeth Nabel (NIH)
Executive Director)
Tom Valuck (CMS)
Director)
Neera Tanden (Office of the Secretary)
Carolyn Clancy (AHRQ)
James Scanlon (Planning and Evaluation
Garth Graham (Office of Minority Health)
Michael Kilpatrick (DOD)
Joel Kupersmith (VA)
Michael Marge (Office on Disability)
Pete Delany (SAMHSA)
Mike Millman (HRSA)

Council Staff Present

Patrick Conway (Council

Cecelia Casale (Council Deputy

Non-Council Members Present

Kathy Kendrick (AHRQ)
Jean Slutzsky (ARHQ)
Howard Holland (AHRQ)
Brian Alexander (VA)
Susan Queen (FDA)
John Poelman (ASPE)
Rosaly Correa-de-Araiyo (OD)
Cliff Goodman (Lewin)
Liz Handley (FDA)
Kate Goodrich
Anand Pareuh (ASH)
Lauren Hunt (ASPE)

Debrief on Listening Session

Patrick Conway, Council Executive Director, chaired the meeting. He noted that participants at the third listening session echoed many of the same themes heard earlier.

Council members discussed some of what they had heard of note. These included the importance of expanding the methodological toolkit; peer review (including making a distinction between the entity doing the research and the review process); the concept of a global network for CER; the idea of a national patient library; and the potential role of patient registries, including those run by the specialty societies. Council members noted that they'd also heard calls for new emphasis in the areas of psychiatry, trauma, and emergency care.

One Council member noted the importance of closing the loop between researchers (including professional associations doing such work) and the practice base. He added that the same platform should ideally support both research and dissemination in order to lead to changes in care processes and quality improvement. A second Council member suggested that the challenge in all of this work will be to operationalize it—and make sure that patients and providers have useable information and a place where they can get answers. Another member said that the subtext throughout the discussion was that the CER enterprise needs to change the way it does things, whether in methodology, in dissemination, in its approach to individuals vs. groups, or in its approach to peer review.

Regarding methodologies, the Council had a brief discussion about the role and value of randomized clinical trials as well as the need to think of novel ways to approach the comparison between two standard therapies.

Discussion of Report to Congress

Turning to the Report to Congress, Conway asked Council members for any major edits, discussion items, or new points that need to be included in the report.

There was considerable discussion about the CER research and infrastructure inventories, which were just incorporated into the report. Although Council members felt that the report did a good job of defining the inventory as an iterative process, they suggested that it needed to make clear that the numbers were rough and approximate—and that no uniform benchmark exists for delineating what is and isn't a CER project. They also expressed some confusion about how the data in the tables should be interpreted. Several members suggested that the tables either should be in an appendix to the report or left out entirely.

There was also some discussion about whether or not veterans should be considered a priority population. Much of the discussion focused around the statutory definition of priority populations.

Next, the Council looked at the narrative on recommendations for OS funds, and discussed whether the language could be clarified by having two key categorizations (primary and secondary) rather than three categorizations. The Council decided to leave the language as is, and to see what kind of feedback they received during the review process.

CER Definition, Prioritization Criteria, and Framework

The Council reviewed its draft definition, prioritization criteria, and strategic framework based on the feedback it had received via public comments and by participants

during the third listening session. The Council began with discussion of a revised definition.

Conway said that the revised definition reflects the fact that many of those who commented and suggested that the purpose of CER should be to inform health, and that CER needed to include both development and dissemination. One person suggested it should improve health *outcomes*, not health per se.

There was some discussion about the use of the term "real world." While some members were uncomfortable with the vernacular phrase, the Council ultimately agreed that it was a term that everyone would understand. One member pointed out that some of those providing feedback had suggested that the definition was too academic—and that this should help counter that.

The Council agreed to the following revised definition of comparative effectiveness research:

> Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in "real world" settings. The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.
>
> - To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations and sub-groups.
> - Defined interventions compared may include medications, procedures, medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies.
> - This research necessitates the development, expansion, and use of a variety of data sources and methods to assess comparative effectiveness and actively disseminate the results.

The Council next reviewed the revised prioritization criteria for comparative effectiveness research. There was considerable discussion about the minimum threshold criteria, and particularly around how "expressed needs" might be operationalized. Several members talked about the idea that CER must be responsive to the needs of the health care enterprise, including responsive to both individual patients and public health needs.

There was also discussion about unmet needs, and the need for CER to have an effector arm.

The Council also briefly discussed the prioritization criteria, agreeing that #4 should talk about organizations rather than funding mechanisms.

The Council agreed to the following definition for the prioritization criteria:

Minimum Threshold Criteria (i.e. must meet these to be considered):

1. Included within statutory limits of Recovery Act and FCC definition of CER.
2. Potential to inform decision-making by patients, clinicians, and other stakeholders.
3. Responsiveness to expressed needs of patients, clinicians, and other stakeholders.
4. Feasibility of research topic (including time necessary for research).

Prioritization Criteria

The criteria for scientifically meritorious research and investments are:

1. Potential impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm).
2. Potential to evaluate comparative effectiveness in diverse populations and patient sub-groups and engage communities in research.
3. Uncertainty within the clinical and public health communities regarding management decisions and variability in practice.
4. Addresses need or gap unlikely to be addressed through other organizations.
5. Potential for multiplicative effect (e.g., lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government).

Finally, regarding the strategic framework, the Council agreed to change the definition of the category Translation and Adoption to Dissemination and Translation.

Review of Next Steps and Timeline for Report to Congress

Wrapping up the meeting, Conway reminded the Council of next steps and the timeline for getting the report cleared at HHS and OMB. He said that he was hoping that the Council would have an opportunity to preview the IOM report, and here from its authors, prior to the scheduled IOM briefing for members of Congress and their staffs.

Finally, Conway noted that ASPA is planning to use the Executive Summary of the Report to Congress as its primary handout on the report. Congress will receive the full document, which will also be available on the HHS Web site.

Comparative Effectiveness (CE)

Kalipso Chalkidou
Gerard Anderson

What Will Be Different?

- How do other countries operate their comparative effectiveness programs?
- What are the differences between the US CE program and CE programs in other countries?
- What are the important lessons for the US?

Counties We Studied

- Australia
- Brazil
- Canada
- **England and Wales**
- France
- **Germany**
- Russian Federation
- South Korea
- Sweden
- Turkey

Focus In Other Countries

- Drugs and devices
- Surgical interventions
- Diagnostic tests
- NOT
  - Public health interventions (UK only)
  - Alternative delivery systems

US Focus still to be determined
-Some delegation to IOM

CE entities were established as part of a comprehensive strategy and are integrated into the overall system

- US will provide money to AHRQ, NIH and Office of Secretary
- No new entities created aside from Federal Coordinating Council
- Not clear how it will be integrated into overall system aside from the fact that it cannot “ mandate coverage or reimbursement” or “clinical guidelines”

Examples of System Integration

- Australia part of drug coverage process
- UK part of larger reform initiative preceding increased spending on health care
- Germany part of initiative to revise health insurance system
- France part of effort to rationalize spending, improve quality, promote adoption of cost effectiveness

Australia - Pharmaceuticals Benefits Scheme (PBS)

- First established to provide drugs to WWI veterans returning to Australia
- Formulary established(1953)
  - Determination solely on clinical need
- Senior public servants initially ran program and made all decisions
- Membership expanded to include clinical and academic communities in 1970s
- Costs first included in 1993
- PBS is independent committee – clinicians, epidemiologists, economists and consumers
- Reports to Ministry of Health

NICE Responsibilities

- Part of UK's National Health Service
- Determines what does and does not work
- Best value for money
- Reduce unreasonable geographic variation

Separate entities conduct:

- Horizon scanning for emerging technologies
- NHS research on clinical and cost effectiveness of health technologies

Institute for Quality and Efficiency in Healthcare (IQWiG) Responsibilities

- Part of German social insurance system
- Cost and clinical effectiveness as conditions for evaluation coverage and reimbursement of coverage
- Identify quality standards

Reports to Joint Federal Committee (providers and sickness funds) that actually make the decisions

French High Health Authority

- Established 2005
- Independent public body
- Responsibilities
  - Provider accreditation
  - Guideline development
  - Definition of basic benefit package
  - Promotion of information technology

Independence from government is common

- Even when part of government no direct line reporting responsibilities
  - Australia evolved from part of bureaucracy to greater independence over time
  - Most countries the CE entity is a separate government agency, private entity, or public private partnership
- US government activity
  - Federal coordinating council all civil servants

Organizational Models

- Typically independent agency
- US will be part of DHHS

National Institute For Health and Clinical Excellence

- NICE is special health authority
  - 12 Directors
  - Independent appointments commission
  - Partners council (stakeholders)
  - Funding from NHS
  - Many groups participate in process

Germany

- IQWiG is advisory to Joint Federal Committee (providers and insurers)
  - 30 member board of trustees
  - Scientific board
  - Steering Committee

Some CE entities set standards while others only recommend

- Set Standard
  - NICE – mandatory for primary care and hospital trusts (technologies)
- Recommend Standard
  - IQWiG – recommended to Joint Federal Committee (JFC) – JFC must explain if disagree with IQWiG recommendations

US – Strictly advisory

Political Endorsement is Critical

- All the CE entities have made numerous controversial decisions
- Politicians come under tremendous pressure to overturn decisions
- In many countries this has happened only once because the politicians recognized that they would be brought into every decision and it would eviscerate the CE entity
- In US remember demise of OTA and near death of AHRQ

Size and Budget

- Generally small
  - NICE - 270 staff and $70 million
  - IQWiG – 90 staff and $30 million
- Most of cost is in other entities that conduct the research
- US much larger
  - $1.1 billion
  - Staff uncertain

Appeals Process

- Necessary if standards are binding
- NICE appeals on 3 grounds
  - Perverse – no reasonable person
  - Violation of NICE rules
  - Exceeds scope of responsibilities
  - 1/3 decisions appealed and ½ upheld

US -If not binding then no appeals process necessary

Methods

- Topic selection
- Assessment
  - Evidence synthesis
  - Prospective evidence generation
- Appraisal
- Costs

Topic Selection

- NICE:
  - Independent horizon scanning; web-based suggestions; field consultants; regular evidence review by IS teams
  - Multi stakeholder panels led by top UK clinician
  - Final ratification by SoS; gradually passed on to NICE to speed up process
- IQWIG: JFC as the main client, refers topics – IQWIG decides “patient information” topics
- PBS: (mostly) manufacturer driven – all new drugs

Collecting the evidence

- NICE: mostly outsourced: universities and professional groups
- IQWIG: in-house and outsourced
- HAS: mostly in-house
- PBS: outsourced