



Setting Priorities for Comparative Effectiveness Research: Congressional Briefing

*November 29, 2007
Washington, DC*

Conference Report

Background

Health Industry Forum

The mission of the Health Industry Forum is to develop practical, actionable, market-oriented strategies to improve the quality and value of the US health care system. To support this mission, the Forum sponsors independent, objective policy analysis and provides a range of neutral venues where health care leaders and other stakeholders work together as a group to develop strategies and solutions. Additional information may be found at www.healthindustryforum.org.

Previous Meetings on Comparative Effectiveness Research

This congressional briefing marks the fourth meeting that the Health Industry Forum has convened on comparative effectiveness research (CER) in the past 19 months.

Robert Mechanic, the executive director of the Health Industry Forum, gave a brief overview of the previous three multi-stakeholder conferences on CER:

- *Conference 1 (4/06)*. This meeting focused on efforts within the UK in regard to comparative effectiveness research. The key question posed was whether the US should be doing something similar in developing the capacity for and funding CER. The conclusion of participants at this meeting was a resounding "yes."
- *Conference 2 (11/06)*. This meeting concentrated on the many actions and decisions required in establishing CER capacity. Questions that were discussed included: Where should an entity to oversee CER be located? How should it be funded? Who should fund it? Exactly what should it do? Should it do systematic reviews or randomized clinical trials? Should it just focus on clinical effectiveness or should cost also be considered? Many of the key issues surrounding CER were identified and a series of options were presented.
- *Conference 3 (7/07)*. This meeting focused on how users (physicians, health plans, patients, etc.) will use CER to

make decisions. All stakeholders agreed that adequate information for decision making is lacking and that better information—especially information from head-to-head comparisons of different treatment alternatives—will help make better decisions. There was less consensus about how CER will be used for coverage and payment decisions.

Congressional Briefing Overview

On November 29, 2007, The Health Industry Forum convened a Congressional Briefing on comparative effectiveness research in an effort to educate congressional staff and other federal policymakers. This briefing brought together diverse health care stakeholders, including physicians, health plan representatives, consumer experts, policymakers, and representatives from the federal government. Presenters and panelists provided background on CER, its potential benefits, and the relevant issues in establishing national capacity for CER.

The briefing consisted of an introduction by Robert Mechanic on the major problems facing the health care system that are driving consideration of comparative effectiveness research. Dr. Murray Ross then provided an overview of the key issues associated with an expanded national capacity for comparative effectiveness research. He also gave an overview of the key elements contained in proposed legislation.

Two panels followed. First, a private sector panel shared their perspectives on the value of comparative effectiveness research. Then a panel composed of representatives from different areas within the federal government offered their views. Panelists also offered their thoughts on the initial priorities for CER.

This Conference Report provides a high-level summary of the presentations and the panel discussions. Additionally, The Kaiser Family Foundation has provided a complete webcast and transcripts of the Briefing through Kaisernet, which can be accessed from The Forum's website.

Establishing a National Comparative Effectiveness Research Capacity

Presenters: **Stuart Altman**, PhD, Dean and Sol C. Chaikin Professor of National Health Policy, Brandeis University
Robert Mechanic, MBA, Senior Director and Fellow, Health Industry Forum, Brandeis University
Murray Ross, PhD, Director, Health Policy Analysis and Research, Kaiser Permanente Institute for Health Policy

Overview

Comparative effectiveness research (CER) has gained support in the health care community. By creating a national capacity to gather comparative clinical and cost information on new and existing treatments, policymakers will have a better understanding of appropriate medical use. The belief is that such information can improve the value and effectiveness of health services and curtail excess health care spending.

There is agreement on many aspects of a national CER entity, such as the need for comparative information and that CER should be condition-based. But before a national CER capacity can be developed, industry leaders and policymakers need to address a number of complex legislative, logistical, and financial issues such as determining the appropriate scope, governance, and funding for a new CER entity. Two key recommendations emerged: 1) a CER entity should only *advise* health care decisions, not dictate prices or coverage decisions; and 2) early CER efforts should focus on low-risk, high-impact research areas to generate early wins.

Context

Robert Mechanic described how excess spending is negatively impacting the US health care system and economy. He argued for the establishment of CER to help rein in excess health care costs. Dr. Ross detailed the benefits of CER, identified areas of agreement, and addressed unresolved issues. He also offered thoughts on setting priorities.

Key Learnings

- **Unsustainable growth in health care spending is jeopardizing both the employer-sponsored and federally-funded health insurance systems.**

Over the last 40 years, health care spending has grown at an annual rate of 2.5% above GDP growth. Despite attempts to control this spending (regulation, competition, managed care), the problem persists. In fact, growth in spending has accelerated in the past five years. The negative consequences of this sustained and accelerating growth are being felt by government and the private sector, as well as consumers. Growth in health care premiums is far outpacing growth in the wage base, which funds Medicare. As premium costs have risen, the percent of workers covered by employer-based insurance has decreased, from 65% in 2001 to 59% in 2007. Many large companies, such as GM, have severely cut health benefits to retirees to reduce health care costs.

Uncontrolled spending is equally troubling for the federal government. If health care spending continues to grow at current rates (2.5% more than GDP), in 40 years Medicare/Medicaid spending will represent 20% of the nation's economy, versus 4.5%-5% today. Most economists and policymakers agree that before this occurs, government will intervene to prevent a wide-scale system calamity.

"According to the trends, we are headed toward a meltdown."

— Robert Mechanic, MBA

- **The nation's high spending is not reflected in outcomes. A desire for greater value is driving interest in CER.**

Despite the fact that the US spends more than any other nation on health care (both as a percentage of GDP and in absolute terms), the quality of care does not reflect this investment. Research shows that care patterns exhibit substantial unwarranted variations. Underuse, misuse, and overuse of health care resources are problems that have been well-documented but not effectively addressed. Data from the now defunct Office of Technology Assessment (OTA) suggests that only 20% of care practiced in the US is supported by valid clinical research. The conclusion: the status quo in health care is neither sustainable nor desirable.

"We have a lot of reasons to think that we're not getting all we could for the amount of money we spend in health care."

— Murray Ross, PhD

The reason CER is on the policy agenda is that assessing the value the nation derives from its health care expenditures remains difficult without CER. The country needs the capacity to compare treatments to weigh the relative benefits, risks, and costs—a national capacity that doesn't currently exist.

- **It is believed that national CER capacity would improve the value of health services and excess spending.**

Mr. Mechanic framed the future policy debate as whether public funds should be used to pay for services of limited or no value. Approximately \$100 billion is spent annually on medical research in the US. Yet virtually no funds are dedicated to assessing the clinical and economic value of alternative treatments. Since the OTA's elimination in 1995, the US lacks any dedicated organization to evaluate whether the \$2 trillion it spends on health care is grounded in evidence.

The recommended solution for filling this void is establishing a capacity for comparative effectiveness at the national level. A CER entity would prioritize research among health conditions,

develop evidentiary standards, evaluate research findings, and ultimately disseminate research results and recommendations. CER would provide benefits to multiple parties:

- Patients and doctors.* CER would provide objective information to enable more appropriate treatment decisions.
- Public and private payers.* CER would offer information to make value-based—as opposed to “all or nothing”—coverage and payment decisions and to more closely tailor reimbursement to the actual value of services.
- Drug and device manufacturers.* CER would create a more transparent, predictable, and consistent pricing environment.

“Comparative effectiveness information [will] help the people who deliver care provide appropriate services; not just more services.”

— Robert Mechanic, MBA

- **A CER entity would operate solely in an advisory capacity; it would not dictate pricing or coverage decisions.**

Both Mr. Mechanic and Dr. Ross emphasized that building CER capacity does not mean taking decision-making authority away from patients, providers, or payers. A CER entity should *not* have a mandated influence over spending and care decisions (as does the UK’s National Institute for Health and Clinical Excellence). Both speakers also stressed the importance of developing a comparative effectiveness research capability that focuses on the value (i.e., cost effectiveness) of technologies and not simply their clinical effectiveness.

- **There is substantial agreement on many aspects of CER.**

Unlike many areas within health care, there is much agreement pertaining to CER. Areas of agreement include:

- Better information is needed.* Clinicians, patients, and payers all need better information about what works and what doesn’t.

“Ignorance is not bliss.”

— Murray Ross, PhD

- Condition-based.* Most stakeholders agree that CER should be condition-based and should have broad scope in evaluating treatment alternatives. For example, a new federal CER entity could seek to review comparable therapies for prostate cancer, including radiation, surgery, and medically-based treatments. Further, evaluations should assess drugs, devices, biologics, and procedures.

- Inclusiveness.* There is agreement that the perspectives of all stakeholders should be taken into account in setting CER priorities and as part of the evaluation process.

- Trust is critical.* Trust is essential for everyone to believe the information that is produced. Trust is needed for both scientific and political credibility.

- Stable funding.* All parties agree that funding must be stable and broad-based to sustain short-term political winds. Stable

funding represents a change in the rules of the game; not a one-off undertaking.

- Driving value.* Comparative effectiveness is not about saying no and stifling information. It is about providing information to the marketplace so all parties can make independent, informed decisions. In this way, CER encourages productive innovation and drives value.

- **Three broad legislative issues must be resolved before the US can effectively move forward with CER.**

Several pieces of legislation have been introduced that involve CER in some way. Three key issues must be addressed:

1. *Scope.* It must be determined whether research on comparative cost effectiveness will be part of CER. Dr. Ross noted that the CER proposals he has seen mention cost only in a cursory fashion, but not as a core part of the proposal. In his view cost is an essential part of CER.

“It boils down to something very simple, which is: are we serious about addressing unsustainable spending trends or are we not...I don’t know how you can even have this discussion without cost being part of the equation.”

— Murray Ross, PhD

2. *Governance.* Much of the CER discussion has focused on where a CER entity should be housed. Options include a new entity, within an existing government entity, or outside of government. Whatever the model, the government’s fiduciary accountability must be balanced with the risks of excessive political interference and undue government influence over the process.
3. *Funding.* Deciding on the appropriate funding level for CER is difficult. For perspective, the nation will spend \$25-30 trillion on health care over the next decade. What level of funding is appropriate to provide data on whether the dollars spent on health care are well spent? Questions also exist as to whether the funds should be mandatory or discretionary spending, as well as what the private sector’s role in funding should be.

Additional challenges to implementing CER include accounting for differences across technologies, developing the appropriate analytic framework to define “better” care, and determining how results of CER will be conveyed and to whom. Each of these challenges is significant and must be addressed. But Dr. Ross doesn’t see any of these issues as insurmountable.

- **In the early stages, CER should focus on low-risk, high-impact research areas.**

Recognizing that this work will be difficult, Dr. Ross proposed a number of initial priorities for a CER entity to address to secure early “wins”:

- Conditions with alternative treatments available so comparisons may be made.
- Conditions with significant clinical or financial implications.

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- Research areas with a reasonable expectation that results will influence provider and/or patient behavior.
 - Research areas with a reasonable expectation of getting usable results.

- Research areas for which the outcome is not obvious or treatment is self-evidently necessary.

Private Sector and Federal Agency Panel Discussions

Private Sector Panel:

Jay Schukman, MD, MSc, Anthem Blue Cross & Blue Shield of VA
Armin Ernst, MD, Beth Israel Deaconess
Jean-Paul Gagnon, PhD, Sanofi-Aventis
Gail Shearer, Consumers Union

Moderator: **Stuart Altman**, PhD, Brandeis University

Federal Agency Panel:

Carolyn Clancy, MD, Agency for Healthcare Research and Quality
Michael S. Lauer, MD, FACC, FAHA, National Heart, Lung, & Blood Institute
Eugene Rich, House Committee on Ways and Means

Moderator: **Michael McGinnis**, MD, Institute of Medicine

Overview

There is broad stakeholder support for creating national capacity for comparative effectiveness research (CER). Stakeholders agree that adequate information on what works in health care is lacking and that CER can help fill this void. The expectation is that CER will result in more informed decision making throughout health care. Stakeholders agree on the need to form a center to oversee CER; this center must be funded by government (at least in part) and must be financially stable, inclusive, transparent, and trusted.

The most significant area of disagreement pertains to whether CER should focus solely on comparative clinical effectiveness, or if value should be part of CER's scope. Participants from the federal government see CER as initially focused just on clinical effectiveness, while others are of the view that value is an essential aspect of CER, especially if one of CER's goals is to address costs.

Context

Following Dr. Ross's remarks, panelists expressed their perspectives on CER and suggested initial CER priorities. The panels were composed of: representatives from a health plan and a pharmaceutical company, a physician, and a consumer communication expert; and individuals from different areas of the federal government.

Key Learnings

▪ There is broad support for CER, with agreement on many aspects of what CER should look like in the US.

Consistent with Dr. Ross's remarks, there is much agreement surrounding the elements required for CER to be successful. Panelists agreed that:

- *There is a need for CER.* The current health care system does not produce the outcomes or deliver the value that is desired. In addition, current information gathering tends to focus on the information needed for FDA approval or reimbursement decisions, with little or no collection of evidence on what works. Stakeholders are in agreement that evidence about what works is necessary.

"Most of the time most MDs don't know what the best course of action is for [patients]."

— Armin Ernst, MD

- *There is strong support for CER.* All stakeholders support building national capacity for CER. In general, stakeholders envision an entity (a "CER center") that will among its tasks determine research priorities, coordinate and leverage research and expertise, develop recommendations, and disseminate results.

- *CER includes clinical effectiveness.* While participants don't agree on the full scope of CER, they agree that at a minimum it must focus on comparative clinical effectiveness. Evaluations should be condition-focused and should assess the full range of alternative treatments for a condition.

- *CER should receive federal funding.* The panelists see CER as a public good and believe the federal government should support and fund creation of CER capacity. (The panelists didn't address the amount of funding required or funding from the private sector.)

"The best science comes when people with very different backgrounds work together. Enhancing collaboration leads to the best quality work."

— Michael Lauer, MD

- *Trust, credibility, independence, and transparency are mandatory.* The effectiveness of CER will be based on creating information that is trusted by all parties. Establishing trust requires transparency at all stages of the process.

- *There must be inclusion.* The panelists agreed that the usefulness and credibility of the information created requires determining and answering the "right questions." (In Mr. Rich's view, these are questions that help patients and physicians make better decisions at the point of care.) Arriving at these questions must be done through a process that invites input and expertise from all key stakeholders. An inclusive process is needed to ensure that the information gathered is seen as credible and to assuage concerns that the process will be hijacked by one party (i.e. payers or pharmaceutical companies). Dr. Ernst conveyed that physicians want to be part of the CER process and that failing to include them would hurt the credibility of the results.

- *Stability is essential.* All panelists were of the view that the center created to administer CER must be insulated from political pressures, and adequate time provided for analyses.

—*CER should leverage existing capabilities.* There was not a clear viewpoint on where the new CER center should reside. But there was agreement that regardless of its location, it should collaborate and leverage the capabilities of existing research-focused entities such as NIH, the VA, and AHRQ.

—*All types of research fit under the CER umbrella.* The panelists didn't see CER as limited to one type of research. Most panelists envision CER as encompassing a variety of types of research, including literature reviews, observational studies, and randomized controlled trials (RCTs). Different types of research would be used in different situations. While RCTs are the gold standard, they aren't practical in all situations. Observational studies can be useful in collecting data on surveillance, safety, real-world confirmation of RCT results, and in generating hypotheses for future RCTs. (Dr. Clancy commented on the need for improved observational methods, noting that efforts are underway on this front.)

▪ **The biggest area of disagreement is whether CER's scope should encompass assessing value.**

The panelists had very different perspectives on whether CER should focus solely on clinical effectiveness or whether value should be part of the CER equation.

The panelists from the federal government were in agreement that current legislation restricts them from considering value. In addition, legislation being considered also focuses solely on clinical effectiveness and not value. They believe that incorporating any measures of value into CER would be extremely contentious in the near term. From a practical point of view, they suggested starting CER focused solely on clinical effectiveness.

Dr. Gagnon was also of the opinion that CER should focus on clinical effectiveness. He believes that when comparative clinical effectiveness data is produced, users can incorporate their own costs to consider the cost effectiveness of various treatments. This view was supported by an attendee from a pharmaceutical company who said that organizations already have the ability to measure their own cost, but they lack the ability to measure comparative clinical effectiveness. Therefore, he argued, comparative clinical effectiveness information is what is needed.

Dr. Schukman shared the view expressed by Dr. Ross in his opening remarks that value must be included as a part of CER. But he stressed that value is just one component of CER; it is not the entire story.

"We have to take cost and value into account."
— Jay Schukman, MD

Stuart Altman noted that one of the reasons that managed care was criticized in the 1990s was the perception that it was driven by cost and not value.

▪ **Panelists have different ideas on the initial CER priorities.**

Dr. Lauer thinks the first priority of the CER center should be setting priorities to avoid launching into projects that may not turn out to be useful. Mr. Rich envisions a CER commission composed of a broad array of stakeholders, charged with the responsibility for priority setting. And Dr. Gagnon proposed a regular (perhaps annual) process for priority setting.

Panelists suggested that CER priorities should be driven by factors such as the greatest information gaps and/or in areas where there is significant disease burden and cost, such as cardiovascular diseases and cancer. Some of the specific research areas mentioned where CER could have great impact include Alzheimer's disease, mental illness, back pain, obesity, autism, lung cancer staging, COPD/emphysema, digital imaging, and genetics. Also mentioned was the idea of research on care delivery and system integration.

▪ **An important aspect of CER is the dissemination of information.**

Several panelists mentioned the importance not just of conducting research, but of having the capability to disseminate information and educate users (both physicians and patients) about the findings of the information.

"It is not enough to know 'what'; we have to know 'how' to get information out and into practice."
— Carolyn Clancy, MD

Gail Shearer, whose organization, Consumers Union, specializes in providing consumers with relevant information to help them make more informed decisions, shared the following lessons from Consumers Union's experience in health care:

- Consumers have different information needs.* Consumers are not homogenous. Some prefer concise reports; others want long, detailed reports.
- Consumers prefer intermediaries.* Educating consumers on health care is best done through intermediaries; namely, physicians, pharmacists, health plans, and PBMs. However, more knowledge and experience for effectively communicating with consumers is required.
- Information must be unbiased and trusted.* There must be trust built into every step in the processes.
- Not everyone uses the Internet.* It is important to remember the many consumers still don't use the Internet.