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SHAPING CONVERGENT STRATEGIES IN COMPARATIVE EFFECTIVENESS RESEARCH

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Table of Contents

Session Title	Speaker(s)	Page
About the Sponsors		4
Key Themes		5
Comparative Effectiveness Research: Initial Success, Future Challenges	Gail R. Wilensky, PhD <i>Senior Fellow, Project HOPE/Health Affairs</i>	6
The Patient-Centered Outcomes Research Institute (PCORI)	Carolyn M. Clancy, MD <i>Director, Agency for Healthcare Research and Quality (AHRQ)</i> Michael S. Lauer, MD, FACC, FAHA <i>Director, Division of Cardiovascular Sciences (DCVS), National Heart, Lung, and Blood Institute (NHLBI), National Institutes of Health</i> Sarah A. Kuehl <i>Senior Budget Analyst, Democratic Staff, U.S. Senate Budget Committee</i>	8
Defining Credible Evidence to Inform Decision-Making and High-Value Health Care Services	Sean R. Tunis, MD, MSc <i>Founder and Director, Center for Medical Technology Policy</i> Nancy A. Dreyer, MPH, PhD, FISPE <i>Chief of Scientific Affairs and Senior Vice President, Outcome Sciences, Inc.</i> Barbara J. McNeil, MD, PhD <i>Head, Department of Health Care Policy and Ridley Watts Professor of Health Care Policy, Harvard Medical School; Practicing Radiologist, Brigham and Women's Hospital</i> Kathleen A. Buto <i>Vice President, Health Policy, Johnson & Johnson</i>	10
Luncheon Keynote	Janet Woodcock, MD <i>Director, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration</i>	13
CER: Informing Public and Private Payer Decision-Making	Helen Darling <i>President, National Business Group on Health</i> Charles M. Yarborough, MD, MPH <i>Lead Medical Director and Director, Medical Strategies, Health & Wellness, Lockheed Martin Corporation</i> Douglas R. Hadley, MD, MBA <i>Medical Officer, Director, Coverage Policy Unit, CIGNA HealthCare</i> Brian Sweet, BS Pharm, MBA <i>Chief Pharmacy Officer, WellPoint, Inc.</i>	15

Session Title	Speaker(s)	Page
Utilizing CER at the Point of Care to Improve Patient Outcomes	<p data-bbox="704 415 943 443">Scott S. Young, MD <i>Senior Medical Director and Co-Executive Director, Care Management Institute, Kaiser Permanente</i></p> <p data-bbox="704 527 1349 611">Walter "Buzz" Stewart, PhD, MPH <i>Associate Chief Research Office; Director, Center for Health Research, Geisinger Center for Health Research</i></p> <p data-bbox="704 642 1349 758">Steven D. Pearson, MD, MSc, FRCP <i>President, Institute for Clinical and Economic Review (ICER), and Visiting Scientist, Department of Bioethics, National Institutes of Health</i></p> <p data-bbox="704 789 1105 842">Myrl Weinberg, CAE <i>President, National Health Council</i></p>	17

About the Sponsors

The National Pharmaceutical Council (NPC) was established in 1953 and is supported by the nation's major research-based pharmaceutical companies. NPC's overarching mission is to sponsor and conduct scientific analyses of the appropriate use of biopharmaceutical innovation, with a strategic focus on evidence-based medicine for health care decision-making, to ensure patients have access to high-quality health care.

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The Health Industry Forum is based at Brandeis University, chaired by Professor Stuart Altman, and directed by Robert Mechanic. The Forum brings together public policy experts and senior executives from leading healthcare organizations to address challenging health policy issues. The Forum conducts independent, objective policy analysis, and provides neutral venues where stakeholders work together to develop practical, actionable strategies to improve the quality and value of the US healthcare system.

Conference presentations and other background materials are available at www.healthforum.brandeis.edu

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Key Themes

Overview

Different forms of comparative effective research (CER) have existed for some time. But significant funding in the stimulus package and inclusion as part of health care reform legislation has made CER more prominent. Federal policy-makers are interested in the creation and dissemination of evidence about which treatments are most effective for which patients in which situations. The intent is that CER will enable clinicians, patients, and payers to make more informed decisions, resulting in improved health outcomes and lower costs.

While there is general support among multiple stakeholders for CER, there are significant implementation challenges. These challenges include developing methods for creating “credible evidence”, disseminating evidence after it is created, and having the evidence put into practice.

It will take time for the methods to be determined, for research to be conducted, and for the results to be disseminated and used. During this time, CER’s status will remain fragile. Needed are patience and compelling evidence—tools that make disseminating evidence easy and effective, along with some early wins.

Context

On June 24, 2010, the National Pharmaceutical Council, the Health Industry Forum, America’s Health Insurance Plans, and the Blue Cross Blue Shield Association brought together comparative effectiveness research thought leaders. The focus of the keynote speakers and the panelists was the way forward for CER in the United States, especially in light of CER’s inclusion in health care reform.

Key Takeaways

- **It is an exciting moment in the evolution of CER.**

Throughout this conference, speakers expressed their excitement about the future of comparative effectiveness research. Speakers included representatives of providers, employers, payers, patient advocates, the Food and Drug Administration (FDA), the National Institutes of Health (NIH), and Agency for Healthcare Research and Quality (AHRQ).

The excitement is based on the fact that CER was included in the stimulus plan, where it received \$1.1 billion in funding. CER was also part of health care reform, which called for the establishment of the Patient-Centered Outcomes Research Institute (PCORI), with long-term funding of \$600 million per year.

The creation of PCORI and this level of funding show that CER has many supporters. Over the next 5–10 years there

is a significant opportunity to demonstrate the value that can be derived from CER.

- **Most speakers support a separation between information creation and decision making.**

There was general support for the notion that PCORI should focus on generating evidence, with decisions regarding coverage and reimbursement left to other parties.

However, while decision-making authority is not part of PCORI’s mandate, several speakers indicated that a key benefit of CER is to identify treatments that aren’t effective so as to maximize health for the individual patient.

- **While the concept of CER is generally supported, implementing CER won’t be easy.**

Among the key implementation issues discussed were:

- *PCORI’s governance.* CER’s implementation will be affected by how PCORI is governed. PCORI’s board will include representatives from multiple stakeholders, including physicians and patients.
- *Research methods.* PCORI is tasked with establishing a methodology committee that will determine the process and standards for CER and what constitutes “credible evidence.” Several participants described the need for PCORI to use types of research beyond randomized control trials (RCTs), such as high-quality observational data.
- *Disseminating the evidence.* Creating credible and compelling evidence is seen as only half the challenge. Equally important is being able to broadly disseminate the evidence that is created to both clinicians and patients. Historically, disseminating research results isn’t given the same consideration as the conduct of research. But for CER to be effective, much consideration must be given to how results are disseminated.
- *Putting the evidence into practice.* Ultimately, CER is only valuable if the evidence that is created is put to use. This includes translating evidence into useful information in a form that patients can easily understand. It means creating decision-support tools that clinicians will use at the point of care. And, it eventually includes using evidence regarding the effectiveness and value of various treatment options to help payers make coverage and reimbursement decisions. The way in which evidence is put into practice will influence how industry creates and brings to market new drugs, devices, and treatments.

Comparative Effectiveness Research: Initial Success, Future Challenges

Keynote Speaker: **Gail R. Wilensky, PhD**, Senior Fellow, Project HOPE/Health Affairs

Overview

Funding for comparative effectiveness research (CER) in the stimulus bill and in health care reform provided a critical exciting step forward for CER. The CER initiative, however, remains fragile, and significant challenges remain. Initial funding is limited, and controversy surrounds the concept, including what will constitute “credible evidence,” whether practicing physicians will use this information, and how CER will be used to moderate spending. There also are concerns by patient advocacy groups that CER may limit treatment options and lead to health care decisions that are driven by cost.

The initial set of studies funded by the government will not only yield important and useful information, but the projects funded by the stimulus bill will also provide important learning opportunities about the priorities and processes of the CER initiative. Early wins will hopefully demonstrate the value of CER.

Context

Dr. Wilensky discussed CER in light of the passage of health care reform and provided an overview of future challenges.

Key Takeaways

- **In the U.S., CER is viewed as an information-gathering activity, not a decision-making tool.**

CER is approached differently in the U.S. than in other countries, where CER has been practiced in some form for 50 years. In other countries, CER focuses mainly on pharmaceuticals or devices. In the U.S., CER is viewed as a way to assess alternative methods, including drugs, devices, procedures, and health delivery approaches. The biggest difference, however, is that in the United States, CER will be used primarily to generate information on how treatments impact patients, rather than utilized for making coverage decisions.

- **The inclusion of CER in health care legislation is exciting, but concern about its future remains.**

CER has gotten much attention since its inclusion in the stimulus bill and in health reform, but its future remains fragile due to controversy surrounding the concept. There are limitations in the legislative language and uncertainty about the role of CER and how the research that is produced will be used, particularly with limited funding. Proponents must be thoughtful regarding how CER is rolled out, overcoming enormous implementation challenges for both the federal government and practitioners. In addition, provider groups, patient

advocacy groups, and certain members of Congress continue to have concerns about CER, and may create resistance to the future CER initiatives.

“I have a high level of concern about the very fragile nature of CER.”

— Gail R. Wilensky

- **Much learning can be derived from the initial CER studies.**

The American Recovery and Reinvestment Act (ARRA) allocated \$1.1 B for CER. This funding provided a great start for CER, and offers opportunities to learn. Empirical results from the initial ARRA-funded studies should be available in the next few months. Early results of the studies should provide some “winners” in terms of information that patients and physicians think is important.

But lessons from the processes and priority setting will also be important. A tremendous amount of public input was received in setting priorities. Learning from this outreach process will be valuable, such as the importance of transparency in reassuring groups still concerned about CER. The Institute of Medicine (IOM) and the Federal Coordinating Council established under ARRA provide examples on how central organizations can help create definitions, coordinate activities, and help set priorities. Also important are decisions about acceptable methodologies for credible evidence. When IOM listed the top 100 CER priority topics, it was a first step in rebutting the notion that only randomized control trials (RCTs) provide useful information. Half of the IOM’s 100 priority topics used data gathering other than RCTs.

“Early results of the studies that were funded by the stimulus bill will be very important.”

— Gail R. Wilensky

- **PCORI faces many challenges, especially on how to define “credible evidence.”**

Health care reform legislation included the creation of a Patient-Centered Outcomes Research Institute—PCORI. PCORI faces many challenges as it moves forward, including:

- *Setting up the institute.* A Governing Board needs to be appointed by September. Hiring the right executive director and senior staff and developing a priority-setting strategy will be extremely important.
- *Developing rules of “credible evidence.”* PCORI’s most critical step will be determining the rules for what constitutes “credible evidence.” This decision will ultimately determine how valuable PCORI will be. A

methodology committee will be created that must prepare a report in 18 months, which will determine the important “first word” on credible evidence. Some important questions that need to be answered.

- What does it mean to look at how care is provided in a usual care setting?
 - How will observational data and other non-experimental design data be used?
 - What constitutes systemic reviews? For example, the IOM study revealed that many systemic reviews do not follow “rules of the road” in evidence gathering and many may need to be redone or reconsidered.
- **Ultimately, the biggest challenge will be determining how CER information will be used.**

The primary stated purpose for CER is to improve health outcomes. But an important secondary purpose (and of significant interest to Dr. Wilensky and other economists) will be to serve as a building block to “spend smarter.” Using CER to moderate spending, however, will be a major challenge, and the use of cost information within the CER process remains controversial.

While proponents suggest that comparative effectiveness data should be included in the approval process of the Food and Drug Administration (FDA), in Dr. Wilensky’s view, the FDA should continue to make coverage decisions based solely on safety and efficacy. The role for CER is to help make reimbursement decisions, which should be outside the purview of the FDA.

However, CMS has many limitations and legislatively cannot use cost in coverage decisions. This is an area where private payers can show leadership by using CER findings for value-based purchasing and reimbursement decisions. Pay-for-performance is an example of an area where private payers have taken the lead.

The health care reform law makes it clear that PCORI cannot mandate coverage or reimbursement. However, Dr. Wilensky advocates that PCORI should produce data about the relative value of different treatment options, which payers could use in making reimbursement decisions.

“I like marrying CER with value-based reimbursement and value-based insurance ideas, but am very concerned about how this rolls out.”
— Gail R. Wilensky

- **Improving health outcomes will require physician buy-in and involvement from patient/advocacy groups.**

The success of CER will be based on disseminating the findings from research and having these findings adopted in practice. However, getting physicians to adopt

evidence-based medicine has been very difficult. For CER results to be implemented, it will be important to have physician buy-in, especially when challenging conventional wisdom.

“To say that we have experienced difficulties in changing physician behavior to adopt evidence-based medicine is an understatement.”
— Gail R. Wilensky

It will also be important to involve patient and advocacy groups, who are concerned that CER is focused on costs and averages. Many patient and advocacy groups are concerned that CER will take away treatment options that may represent a cure. PCORI, policymakers, and CE researchers must help convince them that CER is sensitive to patient subgroups and variants, which can be incorporated into CER guidelines and outcome information. Although CER can be an important tool in learning how to spend smarter, this is the most controversial part of making use of CER because it goes to the heart of what many people worry about.

Other Important Points

- **Important legislation attempts.** CER-focused legislative attempts prior to ARRA set the stage for the inclusion of CER in health care reform legislation. Prior CER-focused legislative attempts included H.R. 2184 (Allen/Emerson), H.R. 3162 (CHAMP Bill), and S. 3408 (Baucus/Conrad).
- **Advocates that understand.** The National Breast Cancer Coalition is an example of a staunch supporter of the concept of CER and of adopting evidence-based medicine. This group learned the hard way that rushing to adopt new technologies in the absence of evidence can prove potentially harmful to patients.

The Patient-Centered Outcomes Research Institute (PCORI)

Moderator: **Gail R. Wilensky, PhD**, Senior Fellow, Project HOPE/Health Affairs

Panelists: **Carolyn M. Clancy, MD**, Director, Agency for Healthcare Research and Quality (AHRQ)
Michael S. Lauer, MD, FACC, FAHA, Director, Division of Cardiovascular Sciences (DCVS), National Heart, Lung, and Blood Institute (NHLBI), National Institutes of Health
Sarah A. Kuehl, Senior Budget Analyst, Democratic Staff, U.S. Senate Budget Committee

Overview

The panelists strongly support comparative effectiveness research (CER) and the creation of the Patient-Centered Outcomes Research Institute (PCORI). PCORI's structure as a public/private corporation with a diverse board bodes well for its future success. It will be important for PCORI to learn from the experiences of organizations that have conducted CER, like the NIH and AHRQ. Also important will be not just conducting research, but ensuring that research is disseminated broadly to clinicians and patients, and is put to use in the real world.

Context

The panelists discussed the legislative intent behind the establishment of PCORI, discussed lessons learned from other research efforts, and laid out challenges that PCORI must address.

Key Takeaways

- **The legislative intent behind CER was to empower patients and providers by helping them understand which treatments are most effective, for whom, and in what situations.**

Comparative effectiveness research's inclusion in the American Recovery and Reinvestment Act (ARRA) and the Patient Protection and Affordable Care Act (PPACA) makes this an exciting time for CER. Ms. Kuehl reflected on the process that led to CER's inclusion in PPACA and explained the legislative intent.

Much of the language in PPACA regarding CER—and the establishment of PCORI—came from the Baucus/Conrad bill that was introduced in 2008 and that did not pass. The concepts and language in that bill regarding CE research were created from a blank sheet of paper with a simple, overarching goal: empower patients and providers by helping them understand which treatments were most effective.

Those involved in drafting the bill started by coalescing on a set of principles that included:

- Creation of an entity to conduct and oversee CER that was deemed credible and trustworthy by patients and providers.
- The ability to produce objective research.
- Complete transparency.

- Insulation from politics.
- A stable funding source.

Those crafting the Baucus/Conrad bill considered many types of board models, looking at entities such as the SEC, the Federal Reserve, and others. They settled on a public/private corporation governed by a board with representation from multiple stakeholders, providing the basis for how PCORI is structured.

It was important to have multiple voices heard, particularly those of patients and clinicians. PCORI's board has 21 members, including seven that represent providers and at least three who represent patients and consumers. The directors of the NIH and AHRQ are also on the board. This structure helps to provide the independence that is necessary for continued survival in the turbulent political arena. The board will set the research priorities, allocate funds to various studies, establish advisory committees, and adopt methodological standards.

"It is important that all stakeholders' voices are heard [on the board]. We wanted to build consensus and to incorporate the views of patients and clinicians."

— Sarah A. Kuehl

The legislation required compromise. Some CER advocates were disappointed that cost effectiveness is not part of PCORI's core mission. But opponents of health care reform feared that CER would be used to ration care. Those fears had to be taken seriously, and safeguards were established to bolster transparency and ensure that CER was not linked to coverage decisions. With time and experience, the level of fear and hostility may diminish and these issues can be revisited.

- **AHRQ supports CER and has an important role to play.**

For some time, AHRQ (the Agency for Healthcare Research and Quality) has been involved in effectiveness research, often termed "outcomes" research. Established as part of the Medicare Modernization Act of 2003, AHRQ's Effective Health Care Program has received a total of \$129 million for CER. Through its efforts, the Program has published more than 45 products, including clinical information guides for clinicians, consumers, and policymakers.

As part of ARRA's \$1.1 billion for CER, AHRQ received \$300 million. And, the President's proposed budget for FY 2011 includes \$286 million for AHRQ to conduct patient-centered research, an increase of \$261 million over 2010.

Based on AHRQ's research experience, Dr. Clancy made the following observations that are relevant to PCORI:

- *It is important to get the research question(s) right.* Proper framing of research questions is critical. Questions should be posted for public comment to ensure the final results are meaningful to real-world situations.
- *It is important to balance benefits and harms.* Patients and providers genuinely disagree about their preference for risk or side effects of treatment. Variation that results from informed decision making offers future opportunities to evaluate the outcomes of different decisions.
- *Research needs to be looked at in conjunction with thoughtful dissemination.* Generating great research is not enough. Information must be provided to clinicians and patients in ways that it is put to use.

"CER can't just be interesting to researchers; it must have impact for patients' health and outcomes."

— Carolyn M. Clancy

- *Transparency is vital to developing trust.* Trust is crucial for PCORI and a successful CER program: trust in the process, trust in CER outcomes, and trust in PCORI's recommendation. The key to building trust in the system is to create a transparency in how key decisions are made.

AHRQ is extremely excited about PCORI. There are opportunities to identify synergies and to leverage the research infrastructure that AHRQ has already created. AHRQ looks forward to leveraging the link between CER and post-marketing surveillance efforts that are already underway. Also, PCORI can learn from a governance evaluation that AHRQ is conducting. AHRQ plans to play a key role throughout the process, from helping to train researchers to build research capacity to operationalizing the findings that emanate from CER.

▪ **The NIH is excited about CER and PCORI, but sees several challenges it must address related to CER.**

The NIH, which has long been committed to CER, is extremely excited about PCORI. PCORI has the potential to raise research to a higher level. Its creation presents the opportunity to refine research methods.

In considering PCORI's creation, there are five major challenges/opportunities that NIH must address:

1. *Determining how to interact with stakeholders in setting research priorities.*
2. *Shaping and supporting the next generation of CER studies.*
3. *Helping researchers effectively use non-experimental observational methods.*
4. *Extending the principles of CER beyond patient-oriented clinical science to systems-oriented implementation science.*
5. *Leveraging NIH's multi-disciplinary expertise in high-throughput technologies so that CER complements rather than conflicts with the promise of personalized medicine.*

Other Important Points

- **Cross-government collaboration.** Many federal agencies are involved in some form of CER research. The panelists believe it is extremely important that collaboration take place across the government, possibly through the creation of some type of forum as well as through harmonizing the rules for CER.
- **Timely dissemination.** It is important to provide patients and clinicians with relevant information at the appropriate time when information is needed. AHRQ is working on figuring out how to deliver information to the right party at the most appropriate point in the care process.
- **Educating the public.** A participant commented that more PR efforts are needed to educate the public about what CER is and why it is beneficial. CER needs to be translated for the public. Ms. Kuehl commented that PCORI will hold open forums for the public and envisions a robust role for its advisory committee.
- **Minimizing the burden on physicians.** A participant remarked favorably about the potential synergies between CER and market surveillance, but expressed concern that this might be a burden on physicians.
- **Early wins.** It is important that the early CER studies produce wins that demonstrate the value of CER to both clinicians and the public.
- **Recommended reading.** Dr. Lauer recommended reading two articles on CER: an article by Dr. Clancy and NIH director Dr. Francis Collins titled "[Patient-Centered Outcomes Research Institute: The Intersection of Science and Health Care](#)," published in *Science Translational Medicine*, June 2010, and an article he wrote with Dr. Collins titled "[The Path to Personalized Medicine](#)" in *JAMA*, June 2010.

Defining Credible Evidence to Inform Decision-Making and High-Value Health Care Services

Moderator: **Les Paul, MD, MS**, Vice President, Clinical and Scientific Affairs, National Pharmaceutical Council

Panelists: **Sean R. Tunis, MD, MSc**, Founder and Director, Center for Medical Technology Policy
Nancy A. Dreyer, MPH, PhD, FISPE, Chief of Scientific Affairs and Senior Vice President, Outcome Sciences, Inc.

Barbara J. McNeil, MD, PhD, Head, Department of Health Care Policy and Ridley Watts Professor of Health Care Policy, Harvard Medical School; Practicing Radiologist, Brigham and Women's Hospital
Kathleen A. Buto, Vice President, Health Policy, Johnson & Johnson

Overview

One of the most difficult initial challenges for a robust comparative effectiveness research (CER) enterprise will be defining “credible evidence” and then ensuring that the studies comply to these criteria. Panelists offered thoughts on how PCORI’s Methods Committee—which is charged with defining the processes and standards for good evidence—should proceed with this task. They stressed the importance of aligning and harmonizing CER with other types of research and emphasized the need to communicate these findings in a way that enhances decision-making. Only through a transparent, reliable process will patients, physicians, and caregivers be able to understand and act on the data.

validity, generalizability, feasibility, and timeliness for the research.

Yet the process also must be flexible and transparent. Recommendations also must appropriately consider how to evaluate important patient sub-populations, and allow for incorporation of new information, data, or advances in technology. Most important, it must set the precedent for an ongoing process for developing and updating such standards that provides for input from relevant experts, stakeholders, and decision-makers and for sufficient opportunities for public comment.

Dr. Paul offered key considerations for achieving clarity in CER. They are:

Context

Dr. Paul framed the discussion by sharing the responsibilities of the PCORI Methods Committee and asking the panelists to address two questions:

1. How can we ensure a more predictable and transparent environment where credible evidence is generated and interpreted with high scientific standards to inform health care decision making?
2. How do we ensure that CER findings are disseminated in a timely fashion with adequate reference to the findings’ strengths, weaknesses, and other limitations as well as provide information that is understandable by consumers?

For CER Study Design

- What is the research question?
- Who is the decision audience for the findings?
- Were the methods used well matched to the research question?

For Interpretation of CER Findings

- For whom are the results applicable?
- Are there aspects of the study design that might influence the results?
- What is the strength of the comparative findings?
- What is the stability of the findings—are they likely to change in the short term with new research?

Key Takeaways

- **PCORI’s Methods Committee must develop the processes and standards for CER.**

The legislation establishing PCORI called for the creation of an Institute Methods Committee that would, within 18 months, develop and periodically update methodological standards that are scientifically based. The group’s task will be daunting—to develop a translation table designed to provide guidance and act as a reference for PCORI’s Board to determine research methods that are most likely to address each specific research question. To do this, the group must create methodological standards that build on existing work and address specific criteria for internal

- **Currently in health care, the rules of comparison have not been defined. This is a key part of what PCORI must do.**

Assessing the standings for the FIFA World Cup is easy because there are well-defined rules of comparison. But in health care, rules for comparing different treatments have not been defined.

“We know how to compare soccer teams. We have no idea how to compare most therapeutic interventions.”

— Sean R. Tunis

Even in current CMS regulations, the requirements for evidence standards are not well defined:

- *Determining CMS coverage.* CMS requires “adequate evidence to conclude that an item or service

improves net health outcomes.” (What is “adequate” evidence?)

- *Obtaining a unique HCPCS code to allow billing for procedures and devices.* This requires “significant therapeutic distinction.” (What is “significant?”)
- *Receiving a new tech add-on payment.* This requires showing “substantial clinical improvement.” (What is “substantial”?)

It is the role of PCORI’s Methods Committee to determine a process to establish and maintain detailed methodological standards for CER.

The Center for Medical Technology Policy, which Dr. Tunis directs, has worked on developing methodology standards for CER—and they know how difficult it is. They have developed “effectiveness guidance documents” in different therapeutic areas. These documents provide guidance to researchers on how to produce useful evidence for comparing interventions, similar to FDA guidance documents. Dr. Tunis believes it is important that trials for comparative effectiveness be aligned with trials for regulatory purposes. CER studies will need to balance validity that the research is accurate with relevance and feasibility in the real world.

▪ **High-quality observational studies can play a role in CER.**

Dr. Dreyer focused on the question, “What counts as credible evidence?” Typically, people think of randomized, controlled, double-blind studies as the “best” type of research, trumping everything else. Dr. Dreyer suggested this is not the right perspective.

Instead of only focusing on RCTs, researchers should look at the totality of evidence and recognize what is well done and what provides valuable understanding about treatments for various patient groups. When using this lens, there is an important role for observational data. In many instances, high-quality, observational data can be “good enough” to help make decisions about which interventions are best, for whom, and when; sometimes observational data is better, largely because of its direct relevance to the population of interest.

Several sets of guidelines exist to assess what are high-quality observational studies. These include GRACE, ENcEPP, GPP, and STROBE. Dr. Dreyer focused on the GRACE initiative (www.graceprinciples.org), which stands for **Good ReseArch for Comparative Effectiveness**.

- *Goal.* To develop principles of good practices for observational studies of comparative effectiveness to enhance quality and facilitate use for decision-making by physicians, patients, and payers.
- *Concept.* GRACE is built as an iterative model for consensus. The principles are posted online for public comment and are currently on their fourth iteration.

— *Principles.* GRACE’s key principles are:

- **A study plan** that is clinically relevant, practical, addresses what people want to know, and focuses on specific diseases, conditions, and treatments. The study plan includes measures of effectiveness, safety, and tolerability.
- **Transparent analysis and reporting.** This includes transparency in data collection, comparison to patients with similar likelihood of treatment and benefit, and consideration of alternative explanations.
- **Validity of the results and interpretation.** A hierarchy of evidence helps readers understand where confounding is most likely to be an influence in observational studies, and how to balance the strength of the evidence with the potential impact of bias.

GRACE is still in its infancy, and will continue to evolve. It provides guidance for creating and using high-quality observational data in comparative effectiveness research. A public library of case studies is being assembled to provide examples where observational data has supported decision making.

▪ **What makes data compelling, regardless of their source, are their strength, reliability, and generalizability.**

Dr. McNeil pointed out several examples where good data have been used to drive changes in clinical practices, including beta blockers after AMI, taxanes used in the treatment of breast cancer (by NICE), orlistat for obesity (by NICE), and drugs for Alzheimer’s. In the first example, widespread use of beta blockers took nearly a decade, while the other examples saw adoption in just a few years.

However, there are also many examples where good data are not used.

- *Patients.* At times, patients don’t follow good data because they have personal preferences toward another course of action. Patients have different preferences for aggressive treatment or may be fearful of potential side effects. In other instances, patients ignore data due to cost. More data on comparative effectiveness may not help drive good practices.
- *Physicians.* Doctors ignore data if they are intuitive thinkers and act independently, or if the data don’t fit their personal experience. The conclusion is that the production and use of new data must coincide with education of MDs toward rational thinking. Change here will be slow.
- *Hospitals.* There are a variety of reasons why hospitals don’t use data, including: no goals for improvement; MD leadership or administrative support; lack of a

good feedback/measurement mechanism; and different short-term lack of strong preferences.

The reality is that the acceptance of comparative effectiveness research will be muted unless CER produces unimpeachable evidence, and unless there is a focus on dissemination and education. This includes educating medical students.

“The data from CER must be unimpeachable, with attention to all possible misgivings of users regarding data.”

— Barbara J. McNeil

▪ **Defining good evidence matters to industry.**

Ms. Buto noted that industry hates uncertainty, especially surrounding major investment decisions for future product development. Defining clear standards of evidence provides industry with greater certainty. Clear rules around evidence will clarify how decision makers will assess the clinical value of new treatments, which will help industry make decisions on investing in clinical studies and get new treatments to market faster.

Industry supports a public-private PCORI entity that will provide leadership in comparative effectiveness research, including convening researchers, ensuring transparency, building trust among stakeholders, setting standards for methodological rigor, providing clarity about the roles of different types of research designs, developing translational tools for evidence, and generating consumer understanding and insight in the use of evidence.

From Ms. Buto’s perspective, moving forward with CER requires addressing two important issues:

— *Harmonization of standards.* Regulatory agencies and payers are requiring comparative effectiveness research, but these entities currently have different research standards. On the whole, harmonization of standards is likely to be preferred by industry. It reduces the cost by helping to minimize requirements for similar studies by different entities, leads to more predictable adoption and diffusion, improves post-marketing assessment, and provides an opportunity to develop a “hybrid” design that would meet both regulator and payer needs. The downside is that harmonization could increase the number of studies needed for registration, if it were interpreted to add payer-required studies to study requirements for regulatory approval, it could therefore slow adoption and diffusion, and it could create confusion. Despite these negatives, the benefits of harmonization of study standards may make it preferable.

— *Communication of findings.* For CER to have value, the results must be clearly communicated. Doing so will be a challenge. Ms. Buto is concerned that results will be used selectively to justify barriers to patient access to treatments. She is also concerned that CER will focus on short-term results rather than long-term or societal benefits.

One way to assure appropriate use of CER findings is to ensure that communications are tailored to the appropriate audiences, that they are useful and actionable in real-world settings, and that they are timely, balanced, and objective.

Luncheon Keynote

Janet Woodcock, MD, Director, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration

Overview

The inclusion of Comparative Effectiveness Research in recently passed healthcare legislation is a defining moment. Drug development will certainly change in the new era of CER; how it will change is unclear. Issues of methodology will arise with regard to evidentiary standards and reliability of the evidence, creating great challenges for drug developers and regulators.

Historically, drug research focused on determining if a drug was safe and effective. Regulators focused on whether a particular product's benefits outweighed its risks. But this information does not provide practitioners or patients with the real-world, long-term outcome information they need to make day-to-day decisions. CER is an opportunity to build more useful data on the comparative effectiveness of different therapies. Implementation of CER will be difficult as drug developers and regulators work to balance the costs of drug development with the need for evidence.

Context

Dr. Woodcock discussed the history of drug development and the impact CER will have on drug development, clinical trials, and drug regulation, especially as it pertains to the Food and Drug Administration.

Key Takeaways

- **In the upcoming era of comparative effectiveness information, drug development will change, but how it will change is unknown.**

The inclusion of CER in the health care legislation is a watershed moment, similar in some ways to the amendments to the 1962 Food Drug and Cosmetics Act, which required that drugs had to demonstrate efficacy prior to being marketed.

Drug development practices and strategies may need to be modified in the new era of CER, as issues relating to methodology, evidentiary standards, and the reliability of evidence are raised. Many questions need to be answered: How will the evidence be generated? Who will be responsible for generating it? When in the drug life cycle will evidence be generated?

"There are big methodological issues that will be faced with regard to evidentiary standards and reliability of the evidence."

— Janet Woodcock

- **Current FDA requirements do not generate all the useful, real-world information that is needed to prescribe drugs.**

There is a disconnect between current FDA requirements and the real-world information needs of clinicians and patients. To date, the essential questions on which the FDA has been focused are, "Is the drug effective?" and, "Do the benefits outweigh the risks?" These are the questions that must be answered before a drug is approved.

But the answers to these questions don't give patients, doctors, and other health care providers all the information they need to make individual patient-care decisions regarding different treatment options. Current FDA submissions are based on research that is of limited duration and is conducted in controlled settings. It doesn't provide complete data about safety in all situations and subgroups, results compared to other potential interventions, or long-term outcomes. Consequently, these studies don't answer all the questions that clinicians and patients have about medication use.

The FDA must balance the desire to have new drugs come to market with the reality that many questions can only be answered in a real-world setting. But if FDA requirements are set too high, there won't be any drugs, so compromises have to be made.

- **Comparative effectiveness research can have great value for multiple stakeholders, and the FDA will face pressure to incorporate CER into its purview.**

Comparative effectiveness information is vitally needed; in many drug categories, practitioners now have many alternatives (for example, there are 17 different non-steroidal anti-inflammatory drugs). CER can help determine which of these drugs produces the best long-term outcomes for different groups and sub-groups of patients. CER can also bring value to payers in determining if a drug has incremental value versus its competitors. This is not an FDA requirement, but payers have great interest in knowing about the added value of a new drug introduction.

Currently, CER is not an FDA requirement for drug development. Consequently, many current health technologies and practices have no evidence base, and any evidence that does exist was generated for drug approval. Prior to 2007, there was an almost exclusive focus on pre-market evidence generation. This has begun to change with the passage of the FDA Amendments Act of 2007, which explicitly called for and authorized the FDA to require more evidence about product safety when a new potential risk is identified.

Although there will be an increased role of government in healthcare going forward, federal CER will likely not play a large role in evidence generation of the comparative effectiveness of new products. The costs for the government are prohibitive. This will mean that drug developers are the likely funding source for comparative effectiveness research.

It is likely that there will be pressure on the FDA to broaden its regulatory requirements to include both pre- and post-market research. There is also likely to be pressure on regulators to make decisions based on kinds of data not previously used for decision making.

"Federal CER is not likely to play a huge role in evidence generation about the comparative effectiveness of drugs."

— Janet Woodcock

▪ **The new era of CER will create challenges for drug developers as regulators try to balance the cost of development with the need for evidence.**

Drug development is in crisis. Problems with productivity, drug pipelines, stringent regulations, and current business models raise questions about the sustainability of the enterprise. Nine out of ten drug candidates fail while under development, with one successful drug carrying the cost burden of all the failures. The cost of drug development has increased dramatically, reaching upwards of \$1 billion. As a result, drug developers will increasingly focus on high-value interventions such as unmet medical needs, rare diseases, and serious illnesses.

The methodology of how to do CER has not been fully worked out, and there will be a lot of wrangling. Although the next five to ten years will be a turbulent time, substantial knowledge will be generated. As research methods and processes development becomes more advanced, evidence will become more reliable, and regulators will become more confident in using this evidence. Over the short term, the reconsideration of existing drugs will occur, especially those that have been chronically administered and lack long-term outcome comparison data. Everyone involved in this process needs to be patient. This new era can have significant benefits, but it will take time to unfold.

"There will be much turbulence as the whole system of drug development adjusts to an increased evidentiary base that will be developed under CER."

— Janet Woodcock

Other Important Points

- **Public demands.** The general public wants innovative new drugs to come to market as quickly as possible, and also wants these drugs to be rigorously tested to ensure they are completely safe. This is the balancing act that regulators and drug developers face.
- **Safety standards remain observational.** The current system of drug development does not approach safety in the same way as efficacy. Safety assessment remains largely observational. The coordinated, systematic use of CER can also aid in identifying safety signals.
- **Europeans are thinking ahead.** Europe is balancing early-market access to new drugs with the need for more data. In the U.K., technology assessors and regulators are trying to work together so that drug developers can have a seamless development program. The problem with this approach is that drug regulators and technology assessors don't share the same paradigm. Combining these responsibilities creates conflict in balancing the needs for safety, efficacy, and effectiveness.
- **New strategies for harnessing information.** The FDA is trying to drive more science into drug development. Modeling and simulation is an extremely promising area. A lot of energy is going towards data sharing to understand and model a treatment response.
- **Off-label use.** The level of evidence is substantially different when physicians prescribe drugs off-label, further complicating these issues. Although pharmaceutical companies cannot promote off-label use, there is body of evidence for alternative uses that can be presented in a scientific, non-promotional manner.

CER: Informing Public and Private Payer Decision-Making

Moderator: **Carmella Bocchino, RN, MBA**, Executive Vice President, America's Health Insurance Plans (AHIP)

Panelists: **Helen Darling**, President, National Business Group on Health

Charles M. Yarborough, MD, MPH, Lead Medical Director and Director, Medical Strategies, Health & Wellness, Lockheed Martin Corporation

Douglas R. Hadley, MD, MBA, Medical Officer, Director Coverage Policy Unit, CIGNA HealthCare

Brian Sweet, BS Pharm, MBA, Chief Pharmacy Officer, WellPoint, Inc.

Overview

Payers and employers strongly support comparative effectiveness research (CER). Evidence about how a treatment works in the real world compared to alternative therapies has the potential to improve the health of populations, improve the delivery of health care, and lower costs. Such evidence can be used by providers and patients to make more informed decisions based on each patient's medical and financial situation.

Context

Once the methods of conducting CER are recognized, how can we enhance the interpretation and real-world application of credible evidence to reduce uncertainty in care decisions? Would the development of an all-payer claims database provide data needed for CER? Public and private sector payers and employers shared their perspectives on CER.

Key Takeaways

Large employers support CER, and believe it has much potential to dramatically improve health care.

As the president of the National Business Group on Health (NBGH), Helen Darling represents 300 mostly large employers who provide health care coverage to over 50 million people. NBGH's members want:

- *Better health.* NBGH wants broad solutions that have a huge impact on millions of people.
- *Improved health care effectiveness, safety, and efficiency.* Such improvements can save and redirect hundreds of billions of dollars to improve health equity and coverage.
- *Cost control.* NBGH wants a better understanding of which treatments work for specific groups and in specific circumstances.

Comparative effectiveness research has the potential to help achieve these aims by using scientific evidence to determine what works in real-world practice settings. NBGH supports the creation of PCORI and agrees with the multi-stakeholder representation on the board, the focus on transparency, and the emphasis on operating independently. NBGH believes PCORI must be free of conflicts and must have a strong, experienced methodology committee. It must also have open

processes and numerous ways for stakeholders to express views and provide evidence.

In addition to PCORI, a health system that values and utilizes CER must:

- Ensure that new interventions are designed, tested, disseminated, and evaluated in a comparative way from the very beginning.
- Ensure that developers know that at the end of the development process they will have to demonstrate improved health outcomes compared with alternative interventions.
- Develop and maintain a "learning health care system," where new research is employed to continually improve health outcomes and equity, and develop future research questions.
- Train new investigators in comparative research methods, as well as educating board members and staff to assess evidence.

"By doing what is best—truly based on hard scientific evidence of what works for each patient and what treatments and actions actually make a meaningful positive difference in health outcomes—we will have the best health care for all Americans and we will neither overuse services nor drive up costs and waste."

— Helen Darling

CER is important for assessing the impact of alternative health approaches on populations.

At Lockheed Martin, Dr. Yarborough is focused on ways to improve the health for the entire population of 500,000 covered employees. He sees population health as an imperative, where investments in ways to improve the health of his company's population will translate into business value.

He described several NIH-funded CER projects, and concluded that we need CER studies to enhance decision making of providers and patients in order to improve an individual's health status. CER methods and results can be used in partnership with private health information, selectively supplementing the companies' data sets. CER methods are especially important for assessing the impact of alternative health care approaches on populations.

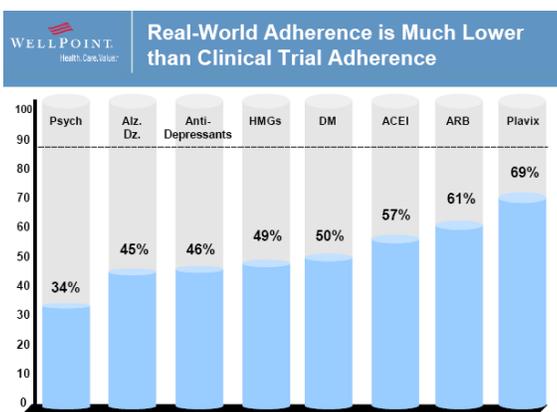
▪ **CIGNA believes CER should primarily be focused on patient outcomes, not on costs.**

Dr. Hadley described CIGNA’s perspective on CER. As an evidence-based company, Cigna strongly supports government programs to develop CER for important health care topics. CIGNA believes:

- *CER should focus on high-impact clinical areas.* The effectiveness of treatments for common chronic conditions is not established in many clinical settings, particularly cardiovascular diseases, cancer, obesity, and arthritic conditions.
- *Cost should not be considered as the initial goal of CER.* The focus should be on using science to compare clinical outcomes.
- *The only time cost should be considered is when two alternatives are clinically equivalent.* Only when two treatments have identical outcomes does it make sense to look at costs as part of CER.
- *CER should be patient focused.* The evidence looked at should be what matters to patients, such as mortality, morbidity, and quality of life. These measures are more important than intermediate laboratory or disease process measurements.

▪ **WellPoint strongly supports CER—and is already using CER.**

Brian Sweet at WellPoint shared his thoughts regarding drugs and CER. He explained that conducting a clinical trial and getting FDA approval is just the beginning of gathering evidence. Once a drug is approved, evidence can be gathered in a variety of ways. The evidence gathered after FDA approval is extremely important, as this is what is happening in the real world. WellPoint’s data shows that in the real world, adherence to various drugs is far lower than the 90% adherence rate common in RCTs.



It is the need to understand how drugs perform in the real world that makes comparative effectiveness research so important. CER provides understanding about sub-groups and about the outcomes that matter most to patients. It enables more informed decision making where risks,

benefits, and effectiveness can be considered. CER also provides data about what is best for a patient based on his/her health and financial situation. It enables true health care choices, and—when done well—it translates clinical evidence into action.

WellPoint was the first health plan to publish its own comparative effectiveness guidelines. In conducting its own CER, WellPoint rates studies as: useful, possibly useful, or not useful. Useful research is scientifically credible, relevant to WellPoint’s population, and includes all relevant treatment comparators. It must meet all specified criteria requirements and the results must be valid.

WellPoint has used its own CER to create an outcomes-based formulary. The goals of this formulary are to provide members with drugs and therapies that will help improve outcomes, quality of life, and productivity, and reduce the total cost of care.

The process used by the outcomes-based formulary is:

- *Critically review the clinical trial data.*
- *Evaluate the clinical value of a drug.* High-quality evidence is used to determine if a drug is favorable, comparable, or unfavorable in comparison to another drug.
- *Determine real-world outcomes and the total cost of care.* Analysis is conducted using integrated pharmacy, medical, and lab data. WellPoint has the largest claims database in the world, and uses it to determine which drugs are most likely to result in positive outcomes in the real world.
- *Advance health care quality and improve outcomes.* High-quality clinical trial data is combined with real-world outcomes data to provide members with drugs that result in optimal outcomes.

“A more expensive medication can be less expensive if the member’s health is improved, resulting in less use of health care resources.”
 — Brian Sweet

Just like the national CER initiatives, WellPoint expects that its CER results will improve decision making by payers and providers, improve population-based outcomes, and improve patient targeting for select therapies.

Utilizing CER at the Point of Care to Improve Patient Outcomes

Moderator: **Robert Mechanic, MBA**, Executive Director, Health Industry Forum, Heller School for Social Policy and Management, Brandeis University

Panelists: **Scott S. Young, MD**, Senior Medical Director and Co-Executive Director, Care Management Institute, Kaiser Permanente

Walter "Buzz" Stewart, PhD, MPH, Associate Chief Research Office; Director, Center for Health Research, Geisinger Center for Health Research

Steven D. Pearson, MD, MSc, FRCP, President, Institute for Clinical and Economic Review (ICER), and Visiting Scientist, Department of Bioethics, National Institutes of Health

Myrl Weinberg, CAE, President, National Health Council

Overview

While creating comparative effectiveness research is the necessary first step, for CER to have value the research must be disseminated to providers in ways that it can be readily adopted and integrated into clinical practice. Fortunately, many organizations—such as Kaiser Permanente, Geisinger Health Systems, and ICER—already have experience putting CER to use. Valuable lessons have been learned from these experiences that can inform how future CER is disseminated. Because changing physician behavior is very difficult, it is important to package CER information within clinical tools and care processes that make physician day-to-day lives easier.

Context

This panel focused on the physician and patient perspectives for using CER data for treatment decisions at the point of care. Panelists examined the opportunities and challenges of translating population-based findings into individualized treatment plans, and how to use health information technology and decision support tools to make CER results available in the physician office or at the hospital bedside.

Key Takeaways

▪ Kaiser Permanente is making data collection and the use of evidence at the point of care integral to how it delivers care.

Through its stable and diverse base of 8.7 million members, Kaiser has a wealth of information at its disposal for research and quality improvement. The cumulative patient encounters captured through its electronic medical record has populated rich, longitudinal clinical database to conduct effectiveness studies in a wide variety of clinical areas.

Kaiser Permanente is making CER actionable by using research to develop clinical pathways. The process involves the following steps:

- *Start with the evidence.* Randomized controlled trials create consensus-based evidence that forms the basis of clinical process changes.

- *Workflow processes.* The evidence leads to pathways that define how the steps in the care process fit together and who accomplishes them.
- *Informatics and clinical decision support.* Implementing the clinical pathways entails developing tools that make the right thing the easiest thing to do for both providers and patients.
- *Analytics and re-evaluation.* Kaiser analyzes whether the steps in a pathway are accomplished reliably and if the expected outcomes are being achieved.

Additionally, Kaiser Permanente has provided its clinicians an online library of research findings and best practices, which can be accessed at the point of care via desktop computers. Clinicians also have dynamic decision-support tools that enhance quality and patient safety. Patients are engaged via personal health records (PHRs), and receive tools and education to support self-care; more than three million members have PHRs and they send clinicians more than 650,000 emails monthly.

Kaiser has implemented several clinical pathways that have yielded great learning. These experiences have resulted in lessons related to integrated care systems and CER. These lessons include:

- Investigators can and should be fully integrated into the care environment. This is the best way to produce real-world evidence.
 - Data should be captured as care is delivered.
 - Evidence-based medicine must merge with everyday practice, recognizing that evidence is constantly evolving.
 - Priority populations should be identified, as well as specialized pathways and conditions.
 - Common pathways should be shared across networks of providers across all care settings.
 - Analytics need to be integrated with care delivery to provide focused timely evaluation and feedback.
- ### ▪ The key to translating research into practice is to make clinical decision support useful to clinicians.

Typically, once evidence is created, attempts are made to translate it into practice through guidelines, point-of-care reminders, and alerts. But this is only about 10% effective. When it is not effective, a common response is to create

even more guidelines and alerts. To Dr. Stewart, the reason this method is ineffective is because the guidelines, alerts, and methods are not useful to the clinician. Instead of externally imposing the guidelines and trying to force adherence, other approaches are needed. Specifically:

- *Learning should be practical.* The effective use of knowledge requires more than simply learning information. Learning requires numerous contextual, iterative, and cognitive processes. The way residents learn is through practical, hands-on experiences. Yet, continuing education is often not practical or hands-on. Learning needs to take place through practice.
- *Clinical decision support (CDS) needs to be useful to the physician.* Trying to get physicians to change their behavior is difficult. Instead, CDS needs to be designed to help physicians by making their job easier. A few examples illustrate the importance of utility:
 - Geisinger developed a patient aid and cardiovascular risk management tool that was used by physicians just 20% of the time. As opposed to blaming physicians, Dr. Stewart's conclusion was that Geisinger didn't make this system useful enough that physicians wanted to use it.
 - Geisinger also developed integrated visual display tools for rheumatology. Patients answer questions prior to seeing the physician, which saves time for the physician, displays trend data on outcomes, augments the provider's workflow, and automatically creates progress notes after the visit. Because physicians find this tool to be highly useful, they use it in more than 90% of visits. This shows that when the tool has utility for providers, they will use it.

"The main lesson is utility, utility, utility. It [clinical decision support tools that translate guidelines into practice] has to be useful to the physician."
 — Walter "Buzz" Stewart

▪ **CER is being used on a community basis as an input into medical policy decisions.**

Dr. Pearson described how a coalition in Massachusetts is using CER in an effort to reduce the confusion on how to approach medical conditions with multiple treatment options, such as prostate cancer. Comprised of leading providers, health plans, employers, and other entities such as the Chamber of Commerce, the goal of the Employers Action Coalition on Healthcare (EACH) is to establish a community consensus on the comparative effectiveness of management options for low-risk, localized prostate cancer. While significant data exist about different treatment options for prostate cancer, there is no

agreement on which options are clinically superior or provide more value to patients.

By working as a community, EACH hopes a mutually-agreeable consensus can provide critical mass and support, shifting community care patterns towards those options that produce higher value. Further, the group wanted to reduce the overall spending on prostate cancer treatment and wasn't shy about its desire to lower costs.

Dr. Pearson's organization, ICER, looked at five types of localized prostate treatments, with particular attention on three types of radiation therapy. ICER examined both clinical effectiveness and comparative value. ICER found two of the treatments to have comparable clinical value (brachytherapy and IMRT) and concluded that proton beam therapy, which is heavily marketed, lacked sufficient evidence to determine the clinical effectiveness. Brachytherapy (at \$10,000) was seen as high value, while IMRT (at \$30,000) and proton beam (at \$50,000) were judged as being low value.

The findings suggest ways in which the comparative effectiveness information can be used, including:

- *Creating information for patients.* Patient information is provided via a community website with representatives from multiple providers.
- *Fostering multi-disciplinary visits.* Instead of just having a patient meet with a surgeon, the group found substantial value when patients met with multiple clinicians to get different perspectives.
- *Changing pricing structures.* Payers can use CER to decide what to cover and how much to pay, which can influence patients' choices. EACH has not yet changed pricing but may do so in the future.

Lessons from ICER's experience with CER include:

- An objective authority is needed to make a clear judgment about the evidence, but complete consensus is unlikely.
 - Parsing patient populations using diagnostic and billing codes is complicated but not impossible.
 - Every stakeholder organization must be aligned at the top, and view small defeats as part of a larger win.
 - CER is vulnerable to large tempests and conflicting goals.
 - Aligning information with medical policy changes appeals to all stakeholders.
 - "Scaling" payment is preferable to non-coverage.
- **Patient advocates support CER. They want to be part of the CER conversation and want CER's focus to be on quality care, not cost savings.**

Ms. Weinberg stated that patients are skeptical about evidence-based health care. They don't want evidence to

be applied in a one-size-fits-all manner. Ms. Weinberg asserted that patients want a balance between science and personal treatment. Addressing this skepticism requires involving people with chronic diseases and disabilities in the CER conversation. Incorporating patients will lead to improved adherence to treatment, increased use of screening, increased patient satisfaction, better outcomes, and lower costs.

Skepticism will be overcome through research results that are credible, objective, and trustworthy. Trust will be built one step at a time. Research must be guided by principles of evidence-based medicine, and results must take individual life circumstances into account.

Other important considerations from the perspective of patient advocates are:

- Separate those who generate CER from those who make coverage decisions. This will provide consumers with greater confidence that decisions aren't made inappropriately. The National Health Council supports PCORI and the diverse board to include three patient representatives.
- Make quality care the first priority for CER.
- Avoid adverse consequences related to restrictions on access, quality, or safety.
- Urge widespread dissemination and use of CER results, utilizing patient organizations as a delivery mechanism.