Implementing Comparative Effectiveness Research: The Value Proposition for Patients, Physicians, and the Health Care System

July 25, 2007
Washington, DC

Conference Report

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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.healthindustryforum.org](http://www.healthindustryforum.org)
Key Themes from Implementing Comparative Effectiveness Research

Overview

The momentum behind an expanded national capacity to generate comparative effectiveness research continues to build. Most stakeholders concur that better evidence about quality and value will drive better clinical decision making and could potentially slow the rate of growth in health care spending.

How evidence is developed, whether it is trusted, and how it is used by patients, providers, and payers will ultimately determine the success of comparative effectiveness initiatives. Using the data to influence physicians, improve patient decision making, and encourage innovation are broadly supported by stakeholders. Substantial improvements in health communication methods will be required to fully realize the potential benefits.

Less clear is whether expanded comparative effectiveness research will result in value-based coverage or reimbursement policies. Several comparative effectiveness case studies were discussed, highlighting the fact that even when a costly treatment is no more effective than less expensive alternatives, payers face very strong social, economic, and political pressures against narrowing coverage or reducing reimbursement.

This debate shows that new comparative effectiveness research will be funneled into a complex health care system. While the new research will be valuable, realizing the full benefit of this information will require substantial changes in health system financing, organization, and incentives.

Context

Implementing Comparative Effectiveness Research, held in Washington D.C. on July 25, 2007, brought together leading experts and practitioners to continue the conversation on how to make expanded comparative effectiveness research a reality.

This was The Health Industry Forum’s third conference focused on comparative effectiveness research. The first asked whether capacity to conduct this research should be expanded in the US and examined models from other countries. The second focused on defining the activities of a comparative effectiveness center and discussing how it would be structured, governed, and funded. This third Forum focused on engaging likely users of comparative effectiveness research; patients, physicians, and payers; identifying key concerns and challenges about expanded use of comparative effectiveness information; and examining specific examples of how this research could apply to existing technologies.

Key Themes

- Momentum for comparative effectiveness is building.

Participants at this Forum reinforced a central theme from past Forum conferences: it is no longer a question of whether there should be an expanded comparative effectiveness research capacity in the U.S. only a question of how it should be organized and developed. Despite concerns about potential uses of these data, all stakeholder groups generally support expanding the current comparative effectiveness research infrastructure and funding.

- Stakeholders must trust the research.

The word “trust” permeated this Forum. It was accompanied by words such as “independence,” “rigor,” and “transparency.” There needs to be trust in the research findings, the research process, and the entity responsible for prioritizing, funding, and disseminating the research.

- Despite imperfect data, comparative effectiveness research can fill many critical knowledge gaps.

Even with much better evidence there will always be substantial uncertainty surrounding certain therapies and patients. This supports the assertion that physicians need flexibility to exercise clinical judgment. At the same time, where evidence is available, there must be greater accountability for ensuring that practice patterns are consistent with the evidence.

- Modifying coverage and reimbursement policies in response to new comparative effectiveness will frequently present challenges.

A case study of Intensity-Modulated Radiation Therapy (IMRT) illustrated that a treatment may be covered widely and reimbursed at a much higher rate even though technology appraisals reveal limited evidence of clinical superiority.

Despite a lack of compelling evidence for some expensive therapies, payers often feel constrained by contractual, legal, market, or political forces that make it difficult to limit coverage or reduce reimbursement rates. Even with strong evidence payers face significant challenges revisiting coverage or reimbursement policies once technologies are widely used. This could limit the ability of expanded comparative effectiveness data to drive improvements in quality or affordability. As Dr. Altman commented, “If we can’t take away [treatments that are less effective in terms of quality and/or value], then a big part of the potential value of comparative effectiveness research won’t be realized.”

- Communicating the results of comparative effectiveness research effectively is another key challenge.

Several participants commented that the usefulness of comparative effectiveness research hinges on finding much more effective methods for distilling and disseminating findings so that they are actionable for patients and physicians.
Establishing a National Comparative Effectiveness Research Capacity

Speaker: Sean Tunis, M.D., MSc, Founder, Center for Medical Technology Policy

Summary

- Comparative effectiveness research assesses the benefits, risks, and sometimes the cost effectiveness of treatment alternatives through a variety of research methods.
- Efforts to expand the national capacity for comparative effectiveness research is being driven by the desire to control health care spending growth rates along with the desire to fill key knowledge gaps to inform medical decision making.
- While past federal efforts to create capacity for comparative effectiveness research have not met with long-term success, some believe that the current environment is more favorable for establishing a sustainable capacity to support this type of research.

Context

Dr. Tunis set the stage for the Forum by describing the basic terms and concepts of comparative effectiveness research. He provided a framework for discussing an expanded US capacity for comparative effectiveness research.

Key Points

- Comparative effectiveness research compares the benefits and risks of treatment alternatives.

Comparative effectiveness research generally focuses on evaluating the performance of different drugs, devices, procedures, or diagnostics for treating specific conditions. Comparative effectiveness research may also examine alternative quality improvement or disease management strategies.

The methods used in comparative effectiveness include systematic review of existing research, retrospective studies using claims and or electronic medical record (EMR) data, prospective experimental studies, and modeling. Ideally, an appropriate methodology will be selected based upon the specific question that needs to be answered.

“We will need to use all of these methods; we need to use the proper tool for the proper job or question.”
— Sean Tunis, M.D.

- The primary motivation for expanding the availability of comparative effectiveness research is to slow the growth in health spending and to improve knowledge gaps that can lead to more effective treatment.

A leading driver fueling interest in comparative effectiveness research is the unsustainable growth in health care spending which has increased 2.5 percent faster than the overall domestic economy for the past 40 years. If the US continues on this path, spending on Medicare and Medicaid alone will represent 20 percent of the country’s gross domestic product (GDP) by in 2050 versus about 5 percent today.

New medical technologies are a major driver of health care spending with studies indicating that their impact ranges from 20 to 50 percent of total health care spending growth. A survey by Victor Fuchs found that 81 percent of economists identify technology as the primary cost driver in health care. Recent studies show significant geographic variation in the cost of health care that are not related to differences in quality -- representing potential opportunities to reduce spending without harming health outcomes.

The US has no systematic process for assessing the effectiveness for medical technologies compared with alternative therapies. Most new technologies come to market without comparative effectiveness data and in many areas of medicine, practice patterns are not supported by evidence. For example, $20 billion is spent each year in the US on wound care, with wide-spread use of Negative Pressure Wound Therapy (NPWT). But there is insufficient data indicating whether NPWT actually works better than standard wound care. The only studies that explore this are poorly designed and have fewer than 25 patients. There are many opportunities where better information about the comparative risks, benefits and costs of alternative treatments could facilitate to improvements in the efficiency and effectiveness of health care.

“There is increasing recognition of widespread gaps in knowledge to support informed decision-making.”
— Sean Tunis, M.D.

Other reasons provided in support of comparative effectiveness research are to help patients and clinicians become more informed decisions makers; to improve value for the money spent; to improve quality and safety; and to sustain innovation. The motivations for expanding comparative effectiveness research are important, as they will drive priority setting, study methods, and political dynamics.

- There is hope that the current, favorable environment will successfully expand the nation’s comparative effectiveness research capacity.

Interest in comparative effectiveness research isn't new. Dr. Tunis quoted a September 1994 proposal from the Office of Technology Assessment (OTA) calling for large, simple clinical trials to yield comparative information on health technologies. Yet a number of federal efforts to support comparative effectiveness research initiated during the 1980s, including the OTA, have not survived. Today, most of the existing capacity and funding are located in AHRQ, NIH, CMS (through its policy of coverage with evidence development). Other sources of comparative effectiveness research include the life sciences industry, the Blue Cross Blue Shield Association, and private organizations like ECRI and Hayes. Despite a multitude of players, there remains a

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shortage of comparative effectiveness research capacity and funding.

Given past history, what would be necessary to ensure that a new effort would be sustainable if significant funding were made available for comparative effectiveness research? Dr. Tunis believes that the current environment is fundamentally different than during the 1990s: including greater concern about spending growth and practice variation, increased support from providers and the life sciences industry, more accessible claims and EMR data—and greater consensus that comparative effectiveness information would be beneficial.

Dr. Tunis suggested that with proper funding, the timing is right for establishing a new national capacity for comparative effectiveness research. A number of factors will be critical to the success of such an effort. The proposed structure, which includes an independent board, should be insulated from politics. All stakeholders must be invited to participate, and there will need to be full transparency. Comparative effectiveness research should now also include work on comparative value (which has not always been the case), and there must be more emphasis on pragmatic trials and registries.
Comparative Effectiveness Research: The Value Proposition for Stakeholders

Panelists: Naomi Aronson, Ph.D., Executive Director, Blue Cross and Blue Shield Association, Technology Evaluation Center
Armin Ernst, M.D., Chief, Interventional Pulmonology, Beth Israel Deaconess Medical Center
Robert Galvin, M.D., Director, Health Care, General Electric
Francis Visco, President, National Breast Cancer Coalition

Moderator: Stuart Altman, Ph.D., Dean, The Heller School, Brandeis University

Summary

▪ Employers see value in comparative effectiveness research but want the data produced to be actionable, trustworthy, and include cost effectiveness information.

▪ Health plans support comparative effectiveness research and want an independent entity with stable long-term funding to oversee it.

▪ Physicians are enthusiastic about comparative effectiveness research that will help them serve patients more effectively. They want to be central participants in the development of this research.

▪ Patient advocates support evidence-based medicine and want a meaningful role in setting priorities for comparative effectiveness research. They expressed some concerns about the potential use of these data to justify cost-driven decisions.

▪ Participants distinguished between situations when the evidence is strong versus when it is uncertain. Requiring submission of data and participation in clinical trials as a condition of coverage when evidence is uncertain is one strategy for closing knowledge gaps.

Context

Following Dr. Tunis’s presentation, four panelists representing different stakeholder groups shared their views on comparative effectiveness research.

Key Points (from Panelists)

▪ Employers support comparative effectiveness research, but demand that it include cost effectiveness information.

Dr. Galvin emphasized that employers are strong proponents of comparative effectiveness research, but stressed that the data generated must be credible, actionable, and include cost effectiveness information. Employers will focus on:

— The usability of the information. Employers don’t just want more information; they want more actionable information. Employers will use comparative effectiveness data for employee education and to make decisions about benefit design.

— Their role in the process. Employers can use these new data to facilitate employee education. They can also support contracted health plans in using comparative effectiveness data for coverage and payment decisions.

— The type of data produced and how it is generated. Employers believe that generating cost effectiveness data as part of a comparative effectiveness research is imperative.

“From an employers point of view comparative effectiveness without cost effectiveness doesn’t make a lot of sense.”
— Robert Galvin, M.D.

It is essential that this information come from a trusted source. Employers and employees are concerned about conflicts of interest of those conducting or reviewing studies. Some believe that disclosure is the solution. Dr. Galvin suggested that in addition to strong disclosure requirements, review panels be composed of individuals with differing conflicts so that some balance is achieved.

▪ Health plans support comparative effectiveness research and care deeply how the research is produced.

Dr. Aronson indicated that health plans believe that an entity focused on comparative effectiveness research can help address the health systems’ systemic quality and affordability issues. This entity must be independent and have an expert board comprising representatives from a variety of stakeholders. The entity should be funded by all payers—public and private—who must provide stable funding to protect the organization’s political independence.

“The funding mechanism is essential to sustain this institution and ensure political independence.”
— Naomi Aronson, Ph.D.

This entity should support a broad research portfolio, including different types of research in a range of different areas. It is important to recognize the importance of conducting primary research when necessary because some questions can only be addressed through head-to-head trials. Health plans want to leverage existing capacity to avoid duplication.

▪ Physicians want to do what is best for their patients, which the current system doesn’t assure.

Dr. Ernst a practicing Pulmonologist stated that physicians are focused on treating their patients effectively and that most physicians believe that new technologies and treatments are extremely important for improving the quality of care. But the current system of FDA approval, based on minimal safety
The first step when comparing medical technologies is to define “better”—which could be less pain to the patient, faster recovery, better outcomes, or less money—and then to provide a consistent and sound blueprint for testing to assess whether new technologies are in fact better.

Physicians need to be at the table in creating this blueprint for testing, as they need to be comfortable with the evaluation methodology.

Another physician commented that physicians are not trained to think about cost effectiveness. She expressed concern that the discussion of comparative effectiveness is very “top-down” when what is needed are ways to equip physicians to practice medicine differently.

### Patient advocates support evidence-based decision making but are concerned about their ability to influence research priorities.

Ms. Visco described how the National Breast Cancer Coalition (NBCC) supports the use of evidence-based medicine and evidence-based decision making. NBCC has many programs focused on educating patients about the clinical evidence and how to incorporate it into their decision making process.

Ms. Visco expressed concerns about how research priorities would be set, whether consumers would be sufficiently involved in the process, and the quality of the evidence used for decision making. She stressed the need for effective dissemination of information to the medical community, which has frequently resisted incorporating new evidence into practice. At the core of these concerns are questions of trust about how comparative effectiveness data will be gathered and used.

These comments were expanded on by another advocate who said that patients are wary of how evidence will be used in policy making and coverage decisions. She is concerned about the apparent lack of guideposts or “rules of the game.” While some proponents of comparative effectiveness research define “better” as improved health, she is concerned that many stakeholders are primarily concerned with cost savings and market share.

### Participants’ Comments

- **Strong versus weak evidence.** Forum participants discussed how strength of evidence has implications for policy development:
  
  — **Strong evidence:** There are numerous examples of wide practice variation despite the existence of broadly accepted evidence-based guidelines. When there is clear consensus about the evidence, the policy goal should be to promote care consistent with the evidence.

  — **Weak evidence:** Several participants stated that even with improved comparative effectiveness research there will be numerous situations where the evidence is unclear. There was substantial discussion about policy decisions when the data are unclear. For example, should patients be required to pay more for treatments not clearly supported by the evidence? Ms. Visco said that there needs to be a bright line determining whether something is covered or not. Her belief is that treatments without supporting evidence shouldn’t be covered given the crisis in health care affordability and the related challenges of expanding basic coverage for the nation’s uninsured. Another participant suggested the need to collect data to reduce uncertainty. For promising treatments payers should consider coverage with a requirement that patients be enrolled in a trial or clinical data registry.

Dr. Tunis argued that there will never be perfect information, but that comparative effectiveness research can help to systematically fill knowledge gaps. Despite uncertainty, he contended, we can take a much smarter approach to improving the effectiveness of health care delivery and there is a great deal of low-hanging fruit.

- **Data collection infrastructure.** One participant said that health care delivery systems already have much of the data necessary to support comparative effectiveness research, but that these data are not collected or aggregated in an organized way. This individual argued that the first task of any entity created to oversee comparative effectiveness should be to improve clinical data collection systems to facilitate collecting the right evidence.
Translating Comparative Effectiveness Research into Actionable Information

- **Speakers:** Steven Pearson, M.D., MSc, Institute for Clinical and Economic Review (ICER) and America’s Health Insurance Plans
  - Steve Phurrough, M.D., MPA, Centers for Medicare & Medicaid Services (CMS)

**Summary**

- Comparative effectiveness research has the potential to benefit patients, physicians, and insurers by supporting more evidence-based treatment decisions and payment policy. Numerous cultural, administrative, and political barriers must be overcome to realize this potential.
- One technology evaluation group, The Institute for Clinical and Economic Review (ICER), has developed an appraisal process that rates both clinical effectiveness and comparative value, which are combined into an Integrated Evidence Rating.
- The Centers for Medicare and Medicaid Services is interested in comparative effectiveness research, but existing federal law and regulations limit CMS’s ability to directly support such research.
- While some participants suggested that insurers should not pay for expensive treatments without supporting clinical evidence, payers are frequently pressured to do so and are reluctant to be perceived as “taking things away” from patients.

**Context**

This session provided practical examples of how comparative effectiveness research can be used. Dr. Pearson described the Integrated Evidence Rating System and provided an example of it in use. Dr. Phurrough described some of the challenges faced by CMS in supporting comparative effectiveness research and discussed a recent example.

**Key Points**

- **Comparative effectiveness research has many applications.**
  
  Dr. Pearson described a range of uses for comparative effectiveness research for patients, clinicians, and insurers. In addition to its use for informing coverage decisions, insurers can utilize comparative effectiveness research for patient or physician decision support, reimbursement policy including pay-for-performance systems, and as part of value-based insurance benefit design. Appraisals must be rigorous, independent, and presented in a format that can be incorporated into medical policy and easily understood by patients and physicians.

  "The key to comparative effectiveness is translating research into action.”
  —Steven Pearson, M.D.

- **ICER has developed an appraisal process and an integrated rating system.**
  
  The health technology appraisal process should build trust, support dialog, and support innovation. ICER has developed a process based on the principles of rigor, collaboration, and transparency. The process includes:
  
  — A **scoping committee:** This committee, which comprises patients, clinicians, researchers, health plans, and life science company representatives, focuses on defining the key questions to be addressed in the assessment.
  
  — **Systematic review and economic analysis:** ICER conducts systematic review of existing literature to rate comparative clinical effectiveness and an economic analysis to determine comparative value:
    
    - **Comparative clinical effectiveness.** A technology’s clinical effectiveness is rated in comparison to alternative treatments as: Superior, Incremental, Comparable, Potential/Unproven, or Inadequate.
    
    - **Comparative value.** A decision analytic model assigns a rating of: High, Reasonable/Comparable, or Low. This rating is based largely on the cost per additional quality-adjusted life year (QALY). While there are no specific thresholds, in general, technologies that are cost saving or have a cost per additional QALY of $50,000 or less are considered High Value. Treatments with cost per additional QALY of $50,000 to $150,000 are Reasonable/Comparable Value. Treatments with cost per additional QALY of $150,000 or more are generally Low Value.
  
  The comparative effectiveness rating and comparative value rating are combined for an Integrated Evidence Rating (IER). This rating provides a language and structure for comparing different technologies (i.e., like a bond rating.) The following table lays out the ratings.

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Health Industry Forum.
IMRT in comparison to 3D-CRT: do so. With this as background, ICER conducted an appraisal of percent of physicians not using IMRT said they were planning to radiation oncologists grew from 32 to 73 percent and in 2004, 90 percent of physicians not using IMRT said they were planning to do so. With this as background, ICER conducted an appraisal of IMRT in comparison to 3D-CRT:

Comparative clinical effectiveness: A systematic review of the existing resource found no evidence of a survival benefit for IMRT versus 3D-CRT. Limited evidence suggested a decreased risk of proctitis (inflammation of the bowel) with IMRT of 2-4 percent compared with 14-16 percent for 3D-CRT.

Based on these findings, ICER’s draft appraisal suggested a comparative clinical effectiveness rating of “Incremental.” However, the ERG felt the data quality was insufficient to support this rating and changed the rating to Potential/Unproven.

Comparative value: The economic analysis estimated the cost per case of proctitis avoided at $313,000, and the cost per QALY at $706,000. Based on these data, IMRT’s comparative value rating was “Low Value.”

The table below shows IMRT’s final Integrated Evidence Rating, and hypothetical examples of how its ratings might compare to other alternatives.

### CMS Case Study

Dr. Phurrough presented a case study of an effort by CMS to help support timely comparative effectiveness research. Age-related macular degeneration is a leading cause of blindness in the elderly. The FDA recently approved Lucentis, a new Genentech drug priced at approximately $2,000 per month, to treat this ailment. Because of the high costs, some ophthalmologists began experimenting with off-label use of Avastin, another Genetech drug with a similar mechanism approved for treating colorectal cancer. Avastin is substantially less expensive with a monthly cost of $50 to $100 per month. Ophthalmologists have reported positive results; both Medicare and individual patients could save substantially if Avastin were found to be equally effective to Lucentis.

The National Eye Institute (NEI) decided to sponsor a head-to-head trial of Avastin and Lucentis. However, Genentech would not donate Lucentis for the trial and NEI did not have sufficient funds to purchase the drugs; therefore it asked CMS to sponsor the drug cost. Historically, CMS policy did not cover the experimental therapy being studied as part of a clinical trial even if the therapy was covered as a standard therapy. However, that policy was just changed specifically to allow CMS to help support the NEI trial.

### CMS and private payers face challenges in supporting clinical trials.

Government and private payers face a range of challenges in supporting clinical trials. Equity issues arise unless payers are willing or able to waive co-payments; the co-pay for Avastin is about $20 monthly versus $400 for Lucentis. Such a co-pay disparity makes it hard to randomize participants, although the Office of the Inspector General has allowed CMS to waive the co-pay for this trial.

The issue of blinding is also complicated if drugs are funded by CMS or insurers. Physician offices must submit claims and receive payment for administered drugs, complicating the blinding process. Also, patients covered by insurance receive “explanation of benefits” which identify treatments received. While these aren’t insurmountable problems, they do complicate the process.

Finally, CMS is presently constrained by its inability to consider cost-effectiveness in its coverage decisions. Although CMS could cover Avastin if shown to be effective for macular degeneration on the basis of clinical trials, it could not decide to eliminate coverage for Lucentis solely on the basis of cost.

### Participants’ Comments

- **(Mis)pricing treatments.** The fact that IMRT is reimbursed at $42,000 while 3D-CRT is reimbursed at $10,000 sparked many comments including: “It’s just mispriced.” In response, Dr. Pearson said that the only way that individuals could conclude that it was mispriced was by looking at data showing the limited...
incremental clinical benefit and low value. Dr. Pearson said that some payers are using ICER appraisals to support make reimbursement changes.

- **Unwillingness to take anything away.** Despite the IMRT appraisal, payers at the Forum expressed reluctance to use the data to reverse course—due to pushback from the radiologists. Francis Visco expressed frustration saying, "I can't support a system that doesn't take things away, [a system in which] something just becomes part of the infrastructure. . . . Why can't we stop doing things that are not supported [by evidence]?" Dr. Altman commented, "If we can't take [ineffective treatments] away, a big part of the value of comparative effectiveness research isn't realized."

- **Adding the patient dimension.** One participant suggested that in addition to rating treatments on dimensions of clinical effectiveness and comparative value, there needs to be a patient dimension added.

- **Rigor and transparency.** The need for trust in the appraisal process and transparency in the data was a key theme echoed repeatedly.

- **Issues with patient enrollment.** A trust-related issue that could hinder comparative effectiveness research is the general reluctance of patients to participate in clinical trials.
Translating Comparative Effectiveness Research into Actionable Information

Panelists: Troyen Brennan, MD, MPH, Senior Vice President, Chief Medical Officer, Aetna, Inc.
Joseph Drozda, Jr., MD, FACC, Consultant, Special Projects, Centene Corporation
Peter Juhn, MD, MPH, Vice President, Evidence and Regulatory Policy, Johnson & Johnson
Gail Shearer, Director, Health Policy Analysis, Consumers Union

Quick Summary

- Realizing the full value of comparative effectiveness research will require significant changes in the broader health care system to better support patients, physicians and payers.
- Substantial improvement in techniques for communicating research results to consumers is essential for them to fully benefit from comparative effectiveness research.
- Physicians are enthusiastic about the clinical value of comparative effectiveness research, but are wary about how it may be used for coverage, reimbursement and guideline development.
- Insurers see value in comparative effectiveness research but will face challenges in using the data to help drive changes in coverage and reimbursement.
- Manufacturers express support for comparative effectiveness research contingent on clear “rules of the road” so that they understand the coverage/reimbursement landscape when making development decisions.

Context

Panelists representing consumers, physicians, insurers, and product manufacturers shared their perspective on future uses of comparative effectiveness research and potential future challenges.

Key Points

- Consumer heterogeneity will necessitate a wide range of strategies for communicating comparative effectiveness information.

Proponents of comparative effectiveness research like to say that it will have great value to consumers. Through its extensive interactions, Consumers Union has identified a number of lessons that apply to communicating comparative effectiveness information.

- **Consumers assume their physician already uses evidence.**
  Most patients believe that their physicians already incorporate the best medical evidence into their practices. A challenge will be educating consumers about comparative effectiveness research without scaring or overwhelming them. For example, some consumers may interpret terms like “evidence-based medicine” as meaning rationing.

- **Consumers have very different needs.** Consumers assimilate information differently. Some like short summaries while others prefer long detailed reports. Consumers access information differently -- via the Internet, written materials, or oral reports, and through a variety of languages.

- **Trusted intermediaries are important.** While it is possible to reach some consumers through direct media, the most effective source for conveying information is through trusted intermediaries, especially physicians and pharmacists.

  "The most effective sources for communicating with patients about comparative effectiveness are doctors and pharmacists."
  — Gail Shearer

- **Research needs to be unbiased and trusted.** This includes the output as well as the process used to create the research.

- **Physicians view comparative effectiveness research positively but have concerns about impacts on coverage, benefit design, and guidelines.**

  From a physician’s perspective, Dr. Drozda described comparative effectiveness research as two gift boxes:

  - **Box 1: A synthesis of clinical research.** Physicians will be excited about this box, as it contains valuable information that will help doctors in their practices.

  - **Box 2: Possible ways in which comparative effective research might be used.** This box includes coverage decisions, prior authorization, benefit design, guidelines, and assorted other medical policies that will utilize comparative effectiveness research. These concern physicians.

In proceeding with comparative effectiveness research, Dr. Drozda says that physicians would like to see:

- **Partnership.** Physicians want to be enlisted as part of the comparative effectiveness process (probably best done through professional and specialty organizations). Physicians want to be involved in creating registries, data standards, measures, guidelines, etc.

- **Effective communication of results.** Much thought and effort needs to be devoted to informing doctors about the results of comparative effectiveness research so that doctors can use the information productively. The key is communicating information in ways that induce appropriate behavior change.

- **Expanded comparative effectiveness research may catalyze new types of insurance benefit design.**

Dr. Brennan, who prior to working for a larger insurer worked as an executive at a large provider organization, shared an anecdote from his personal experience about the challenges of...
enacting new policies based on comparative effectiveness research.

Using an example discussed in a previous session, Dr. Brennan described first hearing a patient who had received IMRT tell others that getting IMRT could reduce his chances of side effects. Then, consultants engaged by Dr. Brennan’s organization recommended expanded use of the more highly reimbursed IMRT treatment in an effort to increase revenues to his provider organization. Following a meeting with the head of radiation oncology, use of IMRT more than doubled. Although he was not familiar with the clinical evidence, the decision seemed rational both from a patient and medical center perspective.

As an insurance executive, Dr. Brennan became aware that there are no clinical data to support IMRT. Because of this, Aetna decided not to cover IMRT, and was subsequently “blasted” in the Milwaukee press. In response, Aetna hosted a conference of leading radiation oncologists hoping to come to a clear consensus, but the doctors could not reach agreement. Aetna’s conclusion: IMRT is now so deeply ingrained in radiation oncology that it wasn’t worth the fight to deny coverage despite the lack of clinical evidence.

“The reason the U.S. has the most expensive health care is because we have accumulated technology.”
— Troyen Brennan, M.D.

Dr. Brennan said that one possible use of comparative effectiveness research could be to create different benefit designs, where co-pays and deductibles would differ based on the strength of the evidence. Potentially more detailed data could demonstrate which treatments are reasonably indicated for different patient subgroups. Patients for whom a treatment is not indicated would have to pay more for that treatment. However, he acknowledged that such a benefit structure would be complex and controversial.

Manufacturers want to know the “rules of the road.”

Dr. Juhn reiterated that it is no longer a question of whether there is support for comparative effectiveness research—but how it will unfold. However, he cautioned about having realistic expectations for the role of new comparative effectiveness research and its impact on health care decisions.

From the perspective of a manufacturer that invests significantly in research and development, it is important that there be clear “rules of the road” for using comparative effectiveness information and that these rules will not change halfway through a product’s development cycle.

In planning for an expansion in comparative effectiveness research, Dr. Juhn raised issues that need to be dealt with—among them, thinking through how appraisals can accelerate innovation by fostering streamlined approval and potentially by rewarding superior products with premium prices.

He also commented that the health care system needs to deal with what he termed “the coverage problem.” In his view coverage can be dealt with by either limiting the choices to patients or by allowing a broad array of choices to be made available, then creating incentives for the best choice. He favors the latter option.

Dr. Juhn’s specific recommendations for a comparative effectiveness process included: broad stakeholder participation; transparent processes; a clear demonstration of payer independence; and adequate resources to conduct good studies.

Participants’ Comments

- **Benefits options.** In addition to the possibility of evidence-based benefit designs, Dr. Altman wondered whether different types of insurance products might evolve based on what they would cover. For example, one product might pay for all treatments deemed to offer any clinical benefit, while another lower-priced product might pay only for treatments supported by evidence as being clinically and/or economically effective.

- **Changing CMS policy.** Dr. Phurrough said that while comparative effectiveness research is important, substantial results require a long-term commitment by payers to help make it work. To do so, the public through its representatives in Congress has to change the rules under which CMS operates.

- **You can’t please everyone.** A representative from the U.K.’s National Institute for Health and Clinical Excellence was asked to share her perspective on the day’s proceedings. She observed that despite the very different health care systems the challenges in the U.K. and the U.S. are similar. The U.K. perspective is that, “You can’t please everyone always.” The key is public awareness that society can’t afford everything at all times. Paying for any technology no matter what its value for those with health coverage has consequences such as creating a huge debt for future generations and creating spending levels that contribute to a large uninsured population.
Comparative Effectiveness and the Federal Budget

Speaker: Philip Ellis, Ph.D. MPP, Senior Analyst in the Health and Human Resources Division, Congressional Budget Office

Quick Summary

- Rising health care costs are the country’s greatest fiscal challenge. Health care spending continues to grow much faster than the overall economy.
- The Congressional Budget Office (CBO) is increasingly focused on analyzing options for mitigating future growth in health care spending.
- Expanded use of comparative effectiveness research is one option to address rising health spending. Using this information to make more informed clinical and policy decisions could decrease variation and increase efficiency. The potential magnitude of future savings is uncertain and will take time to realize. CBO believes savings are likely in the long term.

Context

Dr. Ellis described the fiscal challenges that rising health care spending represent, explained how the CBO is responding. He then provided the CBO’s perspective on comparative effectiveness.

Key Points

- Rising health costs are the country’s main fiscal challenge. Over the past 40 years, US health care spending has grown 2.5 percent faster than the overall US economy. If this continues for the next 40 years, in 2050 combined spending for Medicare and Medicaid would represent 20 percent of US GDP, which is the same percentage that the whole federal budget represents today.

  “Rising health care costs are the country’s central fiscal challenge going forward.”
  — Philip Ellis, Ph.D.

- Because of the impact of health care spending on the federal budget, health care analysis is a CBO priority. Mr. Ellis quipped that the CBO is increasingly becoming the “Congressional Health Office.” Actions the CBO is taking to focus on health care include increasing its staffing, shifting staff to work on health care analysis, and establishing a panel of health advisors.

  While the CBO’s role is to respond to questions from Congress, Professor Altman noted that on issues like comparative effectiveness, the CBO is increasingly proactive. Dr. Ellis said that the CBO is focused on providing more analysis to members of Congress about available options.

- CBO sees potential benefits in comparative effectiveness research.
  CBO is working on a report requested by the chairman of the Senate Finance Committee, but comparative effectiveness was previously on CBO’s radar. Dr. Ellis provided data indicating that a key driver of high health spending is the high rates of geographic variation. Many experts suggest that adopting more conservative practice patterns could reduce health spending by 30 percent. Even if half right, savings would be enormous.

  The challenge, however, is capturing these potential savings. The CBO believes that in the long term, comparative effectiveness would be likely to generate some savings, though it is very difficult to estimate the magnitude or the timing. As Dr. Ellis said, “Expanding the evidence base seems more likely to limit future spending increases than to fuel them.”

  “The CBO believes that comparative effectiveness would probably generate savings in the health care system.”
  — Philip Ellis, Ph.D.

However, in the short term, any potential savings are limited. Time is needed to conduct research, generate new findings, reach consensus, disseminate the results, and incorporate findings into practice patterns and incentives. Changes to Medicare would be required to fully implement policies based on research findings and such changes would take time.

Other Important Points

- Questions about estimating future savings. Dr. Ellis raised questions about measuring the impact of expanded comparative effectiveness research. What research approach is most effective? What is the relative power of information versus incentives? (He believes information along with changes in incentives can yield the greatest results.) Does comparative effectiveness just compare clinical effectiveness or include the relative cost of treatments?

- Don’t promise savings. One participant said that if lawmakers expect comparative effectiveness research to yield savings, Congress could pull the plug if savings don’t materialize quickly. He suggested positioning a comparative effectiveness initiative like the NIH, as providing great value but with no promise of savings.
Conference Participants- July 25, 2007

Stuart Altman, Ph.D.
Dean, The Heller School for Social Policy and Management
Brandeis University

Mara Baer, M.P.H.
Executive Washington Representative
Blue Cross Blue Shield Association

Frederick Boop, M.D.
Neurosurgery
Semmes-Murphey Clinic

Marc Boutin
Executive Vice President
National Health Council

Jennifer Bright, M.P.A.
Vice President, State Policy
Mental Health America

Marcelo Cardarelli, M.D., MPH
Director Pediatric Cardiac Surgery
University of Maryland Medical Center

Kalipso Chalkidou, M.D., Ph.D.
Associate Director, Research and Development
NICE

Garen Corbett, M.S.
deputy Director, Senior Research Associate
Health Industry Forum, Brandeis University

Dave Domann, M.S., R.Ph.
Senior Director, Healthcare Quality Management
Ortho-McNeil Janssen Scientific Affairs, LLC.

Joseph Drozda, Jr., M.D.
Consultant, Special Projects
CENTENE Corporation

Phil Ellis, Ph.D.
Analyst, Health Division
Congressional Budget Office

Lynn Etheredge
Consultant
Health Insurance Reform Project

Naomi Aronson, Ph.D.
Executive Director, Technology Evaluation Center
Blue Cross Blue Shield Association

Carmella Bocchino, R.N., M.B.A.
Exec. V.P., Clinical Affairs and Strategic Planning
America's Health Insurance Plans

Edward Bortnichak, Ph.D.
Vice President, Scientific & Medical Healthcare Relations
AstraZeneca Pharmaceuticals, Inc.

Troyen Brennan, M.D., M.P.H.
Senior Vice President and Chief Medical Officer
Aetna Inc.

Lynda Bryant-Comstock
Director, Medicare Quality & Patient Outcomes
GlaxoSmithKline

James Carey
Executive Director Health Policy
Novartis Corporation

Alexandra Clyde
Vice President, Health Policy and Payment
Medtronic, Inc.

Marcelo DaSilva, M.D.
Assistant Professor of Surgery
Penn State University College of Medicine

Diane Dorman
National Organization for Rare Disorders
Vice President for Public Policy

Jill Eden, M.B.A., MPH
Senior Program Officer
Institute of Medicine--The National Academies

Armin Ernst, M.D.
Chief, Interventional Pulmonology
Beth Israel Deaconess Medical Center

Alissa Fox
Executive Director for Policy
Blue Cross Blue Shield Association
Jean Paul Gagnon, Ph.D.
Director, U.S. Public Policy
Sanofi Aventis

Barry Gershon
Director, Federal Health Policy
Wyeth Pharmaceuticals

Mark Horn, M.D., MPH
Senior Director, Worldwide Public Affairs and Policy
Pfizer, Inc.

Peter Juhn, M.D., M.P.H.
Executive Director, Health Policy & Evidence-based Medicine
Johnson & Johnson

Sharon Levine, M.D.
Associate Executive Director of the Permanente Medical Group
Kaiser Permanente in Northern California

Blair Marshall, M.D., F.A.C.S.
Chief, Division of Thoracic Surgery
Georgetown University Hospital

Robert Mechanic, M.B.A.
Senior Fellow and Director, Health Industry Forum
Health Industry Forum

Parashar Patel
V.P., Reimbursement and Outcomes Planning
Boston Scientific

Doris Peter, Ph.D.
Consumers Union

Richard Popiel, M.D., MBA
Vice President and Chief Medical Officer
Horizon Blue Cross Blue Shield of New Jersey

Susan Reinhard
Senior Managing Director of the Public Policy Institute
AARP

Wayne Rosenkrans, Ph.D.
Director, Scientific and Medical Strategy
AstraZeneca Pharmaceuticals, Inc.

Kevin Schulman, M.D.
Professor of Medicine and Business Administration
Duke Clinical Research Institute

Robert Galvin, M.D.
Director, Health Care
General Electric Company

Curtis E. Hightower, M.D., DVM
Anesthesiology
MD Anderson Cancer Center

Joseph Jackson, Ph.D.
Group Director, Health Policy and External Relations
Bristol-Myers Squibb

Mark Krasnow
BullsEye Resources, Inc.

John Mahoney, M.D.
Corporate Medical Director
Pitney Bowes, Inc.

Steve McMillan
Director of Government Reimbursement
AstraZeneca Pharmaceuticals LP

LeighAnne Olsen, Ph.D.
Research Associate
Institute of Medicine

Steven Pearson, M.D., MSc
Dir., Institute for Clinical and Economic Review
America's Health Insurance Plans

Steve Phurrough, M.D., MPA
Director, Coverage and Analysis Group
CMS

Nancy Ray
Principal Policy Analyst
MedPAC

Alan Rosenberg, M.D.
VP of Medical Policy, Technology Assessment & Credentialing
Wellpoint, Inc.

Murray Ross, Ph.D.
Director, Health Policy Analysis and Research
Kaiser Permanente Institute for Health Policy

Gail Shearer
Director, Health Policy Analysis
Consumers Union