



May 1, 2008

**Comparative Effectiveness: Can We Get Better Health Value for the Dollars We Spend?  
Panelist Responses to Unasked Questions**

On April 4, 2008, the Alliance for Health Reform and Robert Wood Johnson Foundation held a Capitol Hill briefing entitled “**Comparative Effectiveness: Can We Get Better Health Value for the Dollars We Spend?**” Due to time constraints, the panelists were not able to respond to several of the questions posed by the audience during the Q&A session.

The Alliance compiled those questions and invited the speakers to address as many as they wished. Below are responses from **Karen Ignagni** of America’s Health Insurance Plans, **Wilhelmine Miller** of the George Washington University and the Institute of Medicine’s Committee on Reviewing Evidence to Identify Highly Effective Clinical Services, and **David Nexon** of the Advanced Medical Technology Association.

***I. Costs Associated with a Federal CE Program***

- 1) **How large an effort should we envision? \$15 million/year? \$150 million? A billion or two?**
  - **KAREN IGNAGNI:** The estimates for what is “needed” vary widely depending on the agreed upon scope for the CE entity. Given the scale of the nation’s health care budget (2 trillion), the nation should consider dedicating at least one tenth of one percent for evaluation of the comparative effectiveness and value of medical tests and treatments. Several hundred million dollars annually would be the *minimum* required to be able to meet the goal of supporting a robust program of evidence review while also commissioning important prospective clinical trials to gather new data. This goal could be reached over three to five years, with preliminary funding dedicated to establishing the CE entity and its board to initiate research. An initial five-year budget of \$700 million would be necessary to meet public expectations.
- 2) **The IOM has issued a draft report on comparative effectiveness containing a table showing how much duplication there is in CE research. How will this be avoided in the future?**

- **IGNAGNI:** Duplication in CE research exists today, as we have no central focus for what studies need to be done or a standard methodology for how they should be done. The current approach results in multiple research interests, competition for available federal funds and no ability to establish national priorities. A designated and independent entity, whose sole purpose will be CE studies as AHIP suggests, would create a research plan and consistent methodology to assure that funding is directed to address gaps in research without inefficient duplication.
- **WILHELMINE MILLER:** The IOM committee believes that health plans and other organizations would welcome the opportunity to reduce their information costs if they could be assured of high-quality, standards-based systematic reviews.

## **II. Effect of CE on Medicare Cost and Coverage Decisions**

### **1) Does Medicare have any authority currently to deny payment based on comparative effectiveness – for example, the less effective of the heartburn drugs described by Dr. Clancy?**

- **DAVID NEXON:** With regard to prescription drugs, private plans offering part D benefits have considerable leeway in which competing drugs they will include in their formulary. For other services, Medicare can determine that a treatment is not reasonable and necessary. It would not typically make that kind of a blanket, national non-coverage determination for a service that is safe and effective. And I don't think it should.
- **IGNAGNI:** CMS is empowered to deny coverage (and therefore, payment) for interventions it judges not to be “reasonable and necessary”. Coverage decisions apply to drugs (excluding Part D drugs), devices and procedures. Today, CMS may use results from comparative effectiveness and other research to deny coverage if an intervention or technology is shown to be less effective than existing alternatives. However, CMS has no current authority to deny coverage for less cost-effective therapies.

### **2) Are there reports about the impact – or expected impact – of comparative effectiveness research on Medicare's cost? For example, will healthier people become Medicare eligible?**

- **MILLER:** I am not aware of quantified estimates of the positive health impacts of the higher quality, more appropriate care that could result from the take up of clinical recommendations stemming from comparative effectiveness research. However, it is reasonable to expect that more effective health care, preventive

services and chronic disease management in particular, would result in healthier cohorts reaching Medicare eligibility.

- **NEXON:** I'm not aware of any such studies. In theory, better care for people before they turn 65 should result in healthier Medicare beneficiaries and lower costs. Studies at Duke have shown that the decline in disability among the elderly we have seen in recent years will be reducing Medicare costs \$73 billion annually by next year.
- **IGNAGNI:** The Congressional Budget Office released a report entitled "Research on the Comparative Effectiveness of Medical Treatments" that discusses the potential impact of comparative effectiveness on the public and private sectors. The report indicates that in the long term, after initial investments are made, comparative effectiveness research will likely significantly reduce health care spending. The reduction in spending would apply across public and private programs, including Medicare. The report can be found at: <http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf>

**3) For Ms. Ignagni and Mr. Nexon:  
Comparative effectiveness research affects Medicare as much as the rest of the health care arena. What would your advice be for Senators (drafting a comparative effectiveness bill) about how to address jurisdictional issues between the Finance and Health Committees?**

- **IGNAGNI:** We recommend any comparative effectiveness entity exist as an independent public-private partnership that is funded in part, through the Medicare trust fund. Financing through the Medicare trust funds is important in two ways: 1) Public programs such as Medicare, Medicaid and SCHIP would benefit significantly from the information generated from a comparative effectiveness entity, and 2) The trust funds are not subject to the annual appropriations process and, therefore, are insulated from year-to-year funding changes. Such a funding arrangement would be within the jurisdiction of the Senate Finance Committee. Over time, we envision private funding from all participants in the system, including employer plans, health plans and state and local government plans.
- **NEXON:** I wouldn't presume to advise the Committees on jurisdictional issues.

**4) For Mr. Nexon:  
Are AHRQ reports sufficiently nuanced to preclude over-inclusive coverage decisions?**

- **NEXON:** I think the AHRQ comparative effectiveness reports have generally been appropriately qualified and the findings have been well articulated to physicians and patients. This does not, of course, assure that they will be used properly by all audiences.

### **III. Effect of CE on Providers, Manufacturers, and Private Insurers**

#### **1) Would device and pharmaceutical manufacturers, hospitals, doctors and other service providers accept limitations on what would be paid for? In other words, is tying payment to comparative effectiveness research politically feasible?**

- **IGNAGNI:** CE research conducted under rigorous standards has the potential to demonstrate which therapies will yield the greatest value to patients and public and private purchasers. This information will be helpful to clinicians as they make decisions about the relative effectiveness of certain services and technologies. It is also reasonable that payers could consider using this information in a transparent way to make reimbursement decisions to reward those services and technologies that have the greatest impact on improving patient outcomes. The alternative is, for the nation to bring together the best researchers to assess effectiveness but be prohibited from looking at the value question, which is uppermost on the minds of consumers and large and small business purchasers.
- **NEXON:** Whether it is politically feasible or not, it would not be good policy. As I noted in my speech, comparative effectiveness research is a good guide to what works best on average, but not what is necessarily best for an individual patient. If payment isn't adequate for a therapy, it can pose a barrier to access, even if it is the best treatment for a particular patient.

#### **2) How will we ensure that comparative effectiveness research is disseminated and followed by the clinical community?**

- **MILLER:** The IOM Committee agreed that identifying effective health services is just one step toward ensuring an effective health care system. There is little value to identifying effective services or developing evidence-based practice guidelines, if the knowledge gained does not lead to higher quality health care delivery and improved patient outcomes. However, this issue was outside the scope of the study. Setting standards for best practices (e.g., through clinical guidelines) differs fundamentally from successfully implementing them through quality improvement projects, which take place at a local level. More research is clearly needed in this area.
- **NEXON:** There needs to be an aggressive effort to work with the medical specialty societies to disseminate results. As we move more toward electronic medical records and health IT, the results of CE research may be incorporated in decision prompts. Pay for performance can also stimulate doctors to utilize comparative effectiveness research in clinical decision-making.
- **IGNAGNI:** The new CE entity should have as one of its goals the creation of a dissemination plan that will reach clinicians and a feedback mechanism to monitor usefulness of the information. In addition, the clinical community needs to be deeply engaged through all facets: topic selection, research methods,

evidence analysis, and formulation of findings. Greater adherence to CE research findings would come from the enhanced authority of CE information that would emerge from an independent CE entity working closely with professional societies and other organizations.

**3) What, if any, ramifications would exist for a physician who decided to prescribe a treatment against the conclusions of a CE board? Will we/should we get to a point where such a decision would be grounds for alleging malpractice? Should compliance be protection against such a charge?**

- **IGNAGNI:** The standards for malpractice are different than the standards for comparative effectiveness research. Consideration should be given, however, to amending state malpractice laws to take this into account; for example, a physician's reliance on CE standards could serve as a presumption in a medical negligence lawsuit that a physician has taken into account objective scientific data when considering treatment options. This is an important part of transitioning to a system which relies on objective evidence based practice.
- **MILLER:** I personally believe that evidence-based, best practice guidelines developed in accordance with the structure and process recommended in the IOM Committee report constitutes a better standard than the community practice standard that prevails in malpractice cases today. The basic tenet of evidence-based medicine is that decisions about the care of individual patients should be based on "the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients" (Sackett et al., 1996). This means that individual clinical expertise should be integrated with the best information from scientifically based, systematic research and should be applied in light of the patient's unique values and circumstances (Straus et al., 2005). It does NOT mean that the individual physician should not use his/her best judgment. See the special issue of the *Journal of Health Politics, Policy and Law*, Vol. 26, no. 2 (2001), "Evidence: Its Meanings in Health Care and in Law," for discussions of the relationships among medical malpractice law, evidence-based medicine, and clinical practice guidelines.
- **NEXON:** CE research would presumably be considered, along with all other sources of medical evidence, and the totality of the circumstances in a given case of alleged malpractice. A case-by-case analysis would be required.

**4) How will the private insurance market respond to CE research when it comes to coverage decisions? Would insurers refuse to cover treatments that were found to be less expensive, or would they require enrollees to pay more for these less effective treatments, or some other response?**

- **IGNAGNI:** Insurers currently conduct assessments that use multiple studies in making coverage determinations. CE research, conducted with a standard methodology, would help enhance the ability of insurers to use best evidence to

educate clinicians and patients, support coverage decisions, and guide reimbursement methods that reflect the relative value of services and technologies. Just as health plans are beginning to reward clinicians for quality outcomes, more information from an independent, objective source could help facilitate this transition.

- **NEXON:** I think either of the first two results is possible and already occurring in some instances. For the reasons I discussed in my talk, I think this is unfortunate and potentially could result in substandard care for individual patients.

#### *IV. How CE Should Operate*

**1) Most efforts measure the comparative effectiveness of drugs and medical devices, although this is not where the majority of health care dollars are spent. Why isn't comparative effectiveness more focused on the larger expensive areas such as procedures and delivery of care?**

- **IGNAGNI:** CE research should be framed broadly to include evaluations of tests, drugs, devices, and procedures. Research prioritization for CE will not just look at where the majority of health care dollars are spent, but also consider where there is significant variation in clinical practice, and where CE research could most effectively address critical gaps in evidence on safety, effectiveness, and value.
- **NEXON:** I think the pending legislation does examine procedures. But I think the thrust of the question is exactly right: real savings are most likely to be found in altering the processes of care, e.g., through more effective prevention and better management of chronic disease, than by one-on-one comparisons of individual treatments.

**2) It seems like many comparative effectiveness reports illustrate evidence gaps – highlighting the need for additional data. Shouldn't any new comparative effectiveness entity be responsible for generating data instead of doing the same type of meta-analysis studies that everyone is already doing?**

- **NEXON:** That's a tough question. Original research would be valuable, especially in areas where drug companies have little incentive to do studies, such as off-patent products. On the other hand, gold standard clinical trials are so expensive that they would eat up the resources of the new entity fairly rapidly.
- **MILLER:** "Knowing What Works in Health Care," the IOM Committee report, documents the need for additional -- and more rigorously conducted and consistent--evidence reviews based on existing research and evidence reports, and also for a more trustworthy process for developing clinical recommendations and guidelines based on evidence reviews. This is not to say that evidence generation

is not also needed in order to formulate useful clinical recommendations for many clinical interventions and health conditions.

- **IGNAGNI:** Yes, a new federal CE entity should help combine efforts to assess and make the best use of existing evidence while commissioning important studies to gather additional data. CE research must use standard methodologies to ensure that comparisons can be made. Using reviews of existing data to highlight the most important evidence gaps will help target and prioritize future work. This would be the most effective way to harness CE research and drive improvements in the health care system.

**3) With limited resources, would new research be original, or synthesis of existing work?**

- **NEXON:** See response to previous question.
- **MILLER:** If resources for a clinical effectiveness program are very modest, evidence reviews and synthesis would be more feasible than would undertaking new clinical research. Both types of research are needed, however. Funds for original research cannot be spent wisely without an assessment of current knowledge and evidence to frame the research question and avoid duplication of effort. At the same time most syntheses are likely to conclude that evidence is insufficient to reach definitive conclusions. Thus, original research is required on high priority topics.
- **IGNAGNI:** Both. We recognize there is a good foundation of comparative effectiveness research that already exists or is underway in the private sector and through agencies such as AHRQ and the NIH. We expect that as the CE board prioritizes its research agenda, there will be an assessment of existing research and new work to be initiated in order to bring meaningful results to consumers.

**4) To what extent should cost effectiveness be a part of comparative effectiveness? Is the end goal better value, or the promotion of evidence-based medicine, or both?**

- **IGNAGNI:** Both clinical effectiveness and cost effectiveness should be considered in this research. Cost-effectiveness research helps capture and compare all patient outcomes and the impact of tests and treatments on the health care system. This approach is necessary to help evaluate whether expensive new interventions might produce important benefits downstream that make them very cost-effective and appropriate. Similarly, cost-effectiveness is an important tool to help evaluate what patients and our health care system overall pay for new interventions that are equivalent to or only minimally better than existing options. Cost-effectiveness research should, therefore, be a core element of a federal CE initiative.

One example of how cost-effectiveness can be integrated into comparative effectiveness is the approach developed by the Institute for Clinical and Economic Review (ICER) at Harvard Medical School. ICER's rating system for comparative clinical effectiveness and comparative value demonstrate how rigorous research results can be translated into tools for patients, clinicians, and payers to seek improved outcomes and value. There are other tools that have a value assessment, including the BCBS/Kaiser Technology Evaluation Center (TEC).

- **NEXON:** As I indicated in my talk, I do not think cost-effectiveness should be part of comparative effectiveness. The methodology is arbitrary, and the conclusion—that a clinically superior treatment should not be provided because it is not “cost-effective” is not consistent with the values most of us hold. I don't think there are many Americans who would say that senior citizens on Medicare should get the cheapest treatment, not the best treatment. But that is the result that inevitably occurs if cost-effectiveness is used to make clinical decisions.
- **MILLER:** The charge to the IOM Committee explicitly excluded addressing the question of cost effectiveness. My personal judgment is that cost effectiveness analysis (CEA) is an important adjunct to studies of comparative effectiveness and that CEA can inform public and private decision makers about the resource implications of particular coverage and/or payment decisions so as to improve the value for money spent on health care. The goals of better value and the promotion of evidence-based medicine are mutually consistent and reinforcing.

## ***V. Miscellaneous***

### **1) Congress used to have an office that looked at technology and its effectiveness (Office of Technology Assessment), but it was disbanded in the mid 90s. Was this office well utilized? Are there any lessons we can take from how this office was set up/used?**

- **MILLER:** OTA issued well over 700 reports on the complete array of technologies over its short life span. Technologies were defined broadly to include programs with technological content, essentially defining OTA work as policy analysis with an emphasis on technology. The agency earned the respect of the U.S. and international scientific community; the National Academy of Sciences (IOM's parent organization) joined the unsuccessful fight to preserve OTA. Congress and OTA's oversight Board of Senators and Representatives also fought for OTA. In fact, the House of Representatives voted to preserve the agency and several (unsuccessful) attempts have been made since OTA's budget was eliminated to reestablish the agency. OTA only undertook studies requested by Congress; it was able to attract advisory groups from the top ranks of American science.

International groups consistently visited OTA to study the agency model and on return often sponsored organizations like OTA in their home countries. It seems clear that OTA was well utilized and its work is still referenced by those doing similar work. Although the National Academies work model is somewhat different in details, its studies are conducted in ways that reflect the OTA process in many aspects. In its early days, OTA defined technology assessment in health care. These studies are still relevant in many ways today. Furthermore, the OTA process (recorded in internal OTA documents) offer lessons for technologically oriented policy analysis today.

- **IGNAGNI:** The OTA evaluated many different technologies, not just in healthcare, and so had a very diffuse mandate. Its demise was due, in part, to a perception that the process wasn't transparent and did not have stakeholder involvement. This is why we have recommended an independent entity with a board including all key stakeholders and a funding stream that is not dependent upon an annual appropriations process.

**2) How well can we identify those who might really benefit from some new drug or treatment that doesn't work for most? Is such identification even possible? If we can do this effectively, is a formal CE initiative a necessity?**

- **IGNAGNI:** Formal CE is meant to help identify the characteristics of patients who will gain the biggest benefit from new drugs or treatments. CE research will help us learn about and apply "personalized" medicine innovations in the best way.
- **NEXON:** As we make progress on personalized medicine and molecular diagnostics, I think we will get closer to the day when we can make these judgments much more precisely and accurately. To the degree this is the case, the FDA approval process will fill some of the goals that comparative effectiveness research is designed to reach, but I think comparative effectiveness research will still be helpful to inform physicians and patients about many interventions for a long time to come.

\* \* \*