Comparative Clinical Effectiveness:

Leveraging Innovation to Improve Health Care Quality for All Americans

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By

Gail R. Wilensky, Ph.D.
Senior Fellow, Project HOPE

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Project HOPE
7500 Old Georgetown Road ♦ Suite 600
Bethesda, Maryland 20814-6133
(301) 347-3902 ♦ (301) 654-7269

Mr. Chairman and Members of the Finance Committee: Thank you for inviting me here to testify on how information on comparative clinical effectiveness can facilitate getting the right care delivered to the patient at the right time. My name is Gail Wilensky. I am currently a senior fellow at Project HOPE, an international health foundation that works to make health care available to people around the globe. I have previously directed the Medicare and Medicaid programs as the Administrator of the Health Care Financing Administration and also chaired the Medicare Payment Advisory Commission. The views I am presenting here reflect my training as an economist, my experience at HCFA and MedPac and also my membership on a committee established by AcademyHealth (the professional society for health services research) that considered the placement, structure and financing of comparative effectiveness research. My testimony reflects my personal views and not necessarily the views of Project HOPE, AcademyHealth or any other organization.

I am here today primarily to discuss ways to develop information on comparative clinical effectiveness (CCE) through the creation of a new Center for Comparative Clinical Effectiveness, but I also want to note how the use of information on geographic variation can help set priorities for research in such a center and how the development of health IT will greatly facilitate the generation of information needed for a center on CCE. All three of these issues are synergistic with the theme of this hearing: Leveraging Innovation to Improve Health Care Quality for All Americans.

My testimony is based on an article I wrote that was published on-line in *Health Affairs* in November of 2006 which laid out the fundamentals regarding the placement, financing and functions of such a center, modified by the evolution in my thinking that has occurred since the publication. This evolution reflects the result of the many conversations I have had about CCE over the last 20 months with potential stakeholders and funders, elected officials and policy analysts and supporters or opponents of CCE.

Rationale:

In a period when the country is still struggling about how and how much to reform American health care, there seems to be a developing consensus on the need for better information, particularly on comparative clinical effectiveness—that is, information on how various medical conditions respond to treatment using different therapeutic interventions. Driving this interest is the recognition that the current rate of spending growth in health care (a long-term average annual growth rate that is 2.5 percentage points faster than the growth rate in the economy) is simply not sustainable and that even with this spending growth, there are clear and persistent indications of problems with patient safety and with quality. As current and previous CBO testimony has also made clear, geographic variation in the rate of spending for treating individuals with similar medical conditions indicates the opportunities that are available to reduce spending without harming clinical outcomes. The existence of wide variations in Medicare spending per beneficiary has been known for many years but more recent studies by Elliot Fisher and his colleagues at Dartmouth has indicated that the high spending areas

are neither associated with better health outcomes nor are they more consistent with patient preferences.

To be sure, better information will not by itself be enough to moderate spending (or not by very much) and maybe not even be enough to change physician behavior. Realigning financial incentives between patients, clinicians and institutions, encouraging healthy lifestyles by consumers, rewarding clinicians and institutions that provide efficiently-produced, high quality care, setting reimbursement rates to reflect comparative clinical effectiveness information along with cost data and many other changes will also be needed to significantly affect spending growth rates. On the other hand, without better information on what works when, for whom, and provided under what circumstances, it is hard to imagine how the U.S. will be able to develop strategies that will allow the country to learn to spend "smarter" and without this, it is hard to imagine how we will lower the longer-term "excess" spending growth rate.

Role of the Center:

The interest in comparative clinical effectiveness information is neither new nor is it limited to the U.S. Other countries, however, have tended to focus their analyses primarily on pharmaceuticals and devices and their assessments tend to be important or required elements in the coverage or reimbursement decisions of their national health systems. This is beginning to change as more and more countries realize that too much money is spent on medical procedures to keep their focus only on pharmaceuticals and devices.

I am advocating for a Center for Comparative Clinical Effectiveness that would have a different focus and purpose than those used in other countries: one that serves an **information function** rather than a decision-making function. Further, I believe that comparative effectiveness information should primarily be used to inform better clinical decision-making and to be available for use in the design of more appropriate reimbursement rather than being used to set new requirements for coverage. Current FDA coverage rules focusing on safety and efficacy seem quite sufficient to me.

The purpose of the Center on CCE is to fund new research as well as funding systematic reviews of existing research, and to disseminate and otherwise make available what is known about the likely clinical results of using different treatment options for different subgroups of the population. The focus therefore needs to be on medical conditions rather than on specific interventions or therapeutics and needs to include medical procedures rather than only be limited to pharmaceuticals and devices. The work of the center is based on the premise that technologies are rarely **always** effective or **never** effective (assuming that some kind of approval process is required such as the FDA) and that the role of the information made available by the center is to help inform decision-makers about the probability that a favorable outcome will occur. Thus, comparative clinical effectiveness not only provides information that is comparative across various interventions but also recognizes that the outcomes may differ substantially for various subgroups of the population. Because of the nature of the discovery process and

incremental changes that occur over time, it is important to recognize that investment in CCE needs to be thought of as a dynamic process and not once-done, finished forever.

I had previously assumed that systematic reviews of what is already known about the likely outcomes of various therapeutic interventions would be a relatively straight-forward first step in determining what new research should be undertaken. An important report released by the Institute of Medicine in January of 2008 entitled "Knowing What Works in Health Care: A Roadmap For The Nation" indicates that much more work also needs to be done regarding the scientific synthesis and interpretation of existing evidence, particularly in terms of establishing rules of evidence and appropriate methodologies for use in the syntheses. Comparatively speaking, however, investments in learning how to make better use of existing data are likely to be far less than the investments that will be needed to collect and analyze new data.

There have been occasions where researchers have spoken as though only data reflecting the results of double-blinded randomized control trials should be regarded as an appropriate basis for decision-making. My views are the comparative effectiveness analyses need to include data from many sources although it will be important to make clear the robustness of the data collection strategies and methodologies used in the analyses and presumably the conclusions made from the data should also reflect the robustness of the data. All data have limitations and errors. Specifying these limitations and biases and correcting for them where possible is appropriate and should be expected of information made available through a CCE center. It will also be important to find

ways to reduce the costs and time required for the collection of prospective data. Such efforts as those by Sean Tunis in his "real world" randomized trials or Bryan Luce in developing his PACE initiative (Pragmatic Approaches to Comparative Effectiveness) which makes use of Bayesian statistical approaches to establish shorter end points in certain types of clinical trials are examples of efforts in this vein.

Placement of the Center:

The question of where the center should be placed has prompted considerable discussion. I believe the placement of the center need to be determined by the defining characteristics of the center's information. To be more specific, the data made available by the center must be regarded as **objective, credible, and transparent**—protected from both the political process as well as the interests of affected parties. The information should also be timely, span the full range of data available and be understandable to the various parties who want to make use of the data but the most important characteristics are those associated with "trust". Without that, the center won't be able to serve its fundamental reason for existing.

Some have argued the merits of keeping the Center directly within government, with many choosing to house it in the Agency for Health, Research and Quality, AHRQ, the place where the Medicare Modernization Act directed a limited amount of comparative clinical effectiveness analysis to occur. Others have argued the merits of keeping it outside of a direct involvement with government. While any placement will have its advantages and disadvantages, on balance the two that are most appealing to me are the

creation of a new free-standing entity, perhaps modeled after the Federal Trade Commission or the Federal Reserve Board or the use of a Federally Funded Research and Development Center, **FFRDC**, which is **attached to AHRQ**. FFRDC's are entities that are primarily funded by government (minimum of 70%) and are sponsored by an executive-branch agency, which monitors its use of funds. There are several that have been around for many years. The Lawrence Livermore Labs is one of the larger, better known FFRDC's. This model best reflects the dictum of "close…but not too close to government" and also assures a close linkage with AHRQ, the lead agency for health services research which needs importantly to continue in that role. Starting the center is AHRQ while it remains small has the advantage of not needing to create a new bureaucracy but if that is the choice made, its appropriateness or desirability should be assessed after two or three years. I am concerned that if a CCE center were to reside permanently in AHRQ, it would overwhelm all other health services research. I am also concerned about past vulnerability shown by AHRQ's predecessor agency AHCPR to political pressure although I recognize that this could be a problem anywhere.

I also think the Center would be most effective if it had both **intramural** (in-house research) and **extramural** (contract research) functions as do both AHRQ and the NIH. The in-house researchers provide an important element of expertise and hands-on experience but my assumption is that much of the work would be contracted out to universities, free-standing research groups, etc.

Governance:

The governance of such a center is almost as important as its placement. Again, the key concepts are credibility, objectivity and transparency. This means a governing body that is reflective of all the major stakeholders (including both industry and patient advocates), with staggered year appointments by the executive branch (and maybe subject to Senate confirmation) so that no one administration has too much control. Specialized scientific advisory boards would presumably be created for advice on particular comparative effectiveness studies, particularly those involving new research. Full public disclosure of any conflicts or interest will be critical along with full public access to the meeting transactions.

Funding:

Like any new entity, a Center for Comparative Effectiveness would require several years to reach a "steady-state" which I have assumed would be several billions of dollars. My guess is that the center could reach a critical mass of activity with a few hundred million dollars on its way to reaching this steady state.

Because information is clearly a "public good" as the economist uses the term, it should be available to all users without attempting to introduce exclusionary measures. My preferred funding would be by direct appropriation, as is the funding for the NIH. That, however, may not be a realistic strategy. Another option is to combine funding sources that include monies from direct appropriations, a contribution from the Medicare trust fund and a small assessment on **all** privately covered lives. Putting an assessment only on those lives not covered by the ERISA pre-emption would be a serious mistake since

the self insured will be able to benefit in much the same way as the other privately insured. Although all will benefit from the availability of such information, thus the rationale for a direct appropriation, payers will be especially advantaged by having this information available.

The Role of Costs:

The most controversial issue to date has been whether or not to include cost-effectiveness or cost-benefit analysis directly in a center for Comparative Clinical Effectiveness. While I firmly believe the data made available by the Center should be used by payers in their cost-effectiveness and cost benefit analyses and that funding to CMS should be made explicitly for this purpose, along with the ability of the agency to use such elements in their reimbursement decisions, I believe it is best to keep these functions housed separately. Payers would be wise to have their C/E and C/B analyses subject to the same criteria of credibility and transparency that are so critical to the acceptance of comparative clinical effectiveness information. This will be critical to their acceptance and credibility. It is also my expectation that different payers would use the information differently in designing their reimbursement policies. While I don't minimize the controversies that are likely to continue regarding coverage and reimbursement decisions, at least the bases for making these decisions will start from a common data base.

My rationale for the separation is two fold. One reason is technical. Measurement issues and policy decisions involved with C/E and C/B analyses are more controversial and subject to dispute: where in the life cycle is the technology and how much does that affect

costs, whose costs are being measured—Medicare, small purchasers, large purchasers, etc, what functions are or are not absorbed by the purchaser, i.e. is the purchaser wholesale or retail, etc. Because of these technical issues but also because of the more controversial nature of the implications of cost analyses, including the perceived threat regarding coverage or reimbursement that could result from these analyses, I believe combining the inclusion of cost analyses will increase the political vulnerability of a center for comparative clinical effectiveness. Since CCE information is the **most elemental building block** to learning how to spend smarter, it needs to be protected.

Priority Setting:

Even with the anticipated addition of substantial funding for CCE, setting appropriate priorities will become one of the most important functions of the governance structure. General guidance seems to me to be clear and obvious. Priority for new systematic reviews and also for the collection of new data from prospective trials should reflect those medical conditions for which the payers costs are substantial and where the geographic variations are also significant. In general, these are likely to be medical conditions for which there is substantial uncertainty about the proper diagnosis, given the symptoms and/or substantial uncertainty about the proper treatment, given the diagnosis.

To summarize: the center for Comparative Clinical Effectiveness would be an information center, not a decision-making center, providing credible information for clinicians, patients and payers to use to make better decisions. Priority-setting for new

systemic reviews as well as the collection of new prospective data should be based in large part on the treatment of medical conditions that are both high cost and that exhibit substantial variation. Such information would have many important purposes including the development of a reimbursement system in which co-payments could be tiered to what makes the most sense clinically and economically, with the treatments that are associated with the highest likelihood of success having the lowest copayments, all informed by credible, objective transparent data. Patients and clinicians that want more or want to choose different therapies should be able to do so but should need to pay more for their choices. Medicare does not currently have such authority in setting reimbursement rates and granting the agency this authority would be one of the many changes that would need to occur in learning to spend smarter under Medicare. As a small start to allowing Medicare to generate more evidence so that it can improve its national coverage decision-making, local carriers who grant coverage should be required to do so with some type of evidence development.