The Policies And Politics Of Creating A Comparative Clinical Effectiveness Research Center

The issue of comparative effectiveness research has become a political hot button as health reform and economic stimulus collide.

by Gail R. Wilensky

ABSTRACT: As part of the early efforts of the Obama administration to begin health care reform, $1.1 billion for comparative effectiveness research was included in the stimulus bill. Although this amount can be considered as an initial down payment, difficult issues such as where to place an ongoing effort, the role of such research in informing clinical decision making or reducing health care spending, and the governance to ensure full involvement by stakeholders have not yet been resolved. Legislation proposed over the past two years offers some insights into the options available going forward. [*Health Affairs* 28, no. 4 (2009): w719–w729 (published online 25 June 2009; 10.1377/hlthaff.28.4.w719)]

GIVEN THE PROMINENCE OF HEALTH CARE in the 2008 presidential campaign, health care reform is naturally an important part of President Barack Obama's domestic policy agenda, even if not as important as it appeared to be before the economy's dramatic slowdown. Still, it is hard to imagine tackling the economic challenges while ignoring the pressures from increased health spending and the 15 percent of Americans who are uninsured.

It is too early to tell whether legislation providing coverage to all Americans will pass early in this administration. Despite strong interest by the administration and the Democratic Congress, it is not clear how a program that could cost as much as $1.5 trillion over ten years will be funded. Because of the passage of two large unfunded bills in the past eight months, this funding concern has complicated the prospects for health care reform: in addition to Republican concerns, some Democrats are becoming uneasy about any additional new increases in the deficit.[1]

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Early in the administration, it became clear that some aspects of health care reform would not be delayed. Action on reauthorization of the State Children's Health Insurance Program (SCHIP) began early in 2009, and the stimulus package includes some health reform components. The inclusion of expanded funding for Medicaid (across-the-board increases plus special increases for high-unemployment states) and subsidies for Consolidated Omnibus Budget Reconciliation Act (COBRA) continuation coverage for low- and middle-income unemployed people for up to eighteen months after ending their employment was not surprising. Monies for health information technology (IT) and comparative effectiveness research were less obvious but important components of reforming the health care delivery system.

Despite many areas of disagreement, it became clear during the election that there is substantial agreement between the two parties regarding where reform is needed in the health care delivery system—better treatment of chronic disease; care coordination; health IT; and better, more transparent information on prices and quality—although not necessarily regarding the steps that should be taken to achieve these reforms. Prior to the election, there had also seemed to be growing consensus that more and better information on comparative clinical effectiveness was needed to improve care quality and potentially slow health spending as well.[2] However, the passage of the stimulus bill, with its funding for comparative effectiveness research, has galvanized a diverse group of opponents, including patient advocacy groups, industry and clinical organizations, and many Republicans, who are charging that information about comparative effectiveness could lead to rationing and government intrusion into the doctor/patient relationship.[3] In addition, there are opportunities for disagreement on the structure, funding, and, most importantly, the purpose of the entity or entities charged with developing such information.

The Rationale

Observers of U.S. health care—economists in particular—frequently remark on the so-called excess spending gap in health care—growth that has averaged around 2.5 percentage points faster than the rest of the economy, in real terms (adjusted for inflation). If this sustained growth continues, it will greatly stress the federal budget and adversely affect the private sector—inhibiting wage growth and making it increasingly difficult for employers to continue providing health insurance to their employees.

Although no one believes that lowering spending growth—let alone absolute levels of spending—will be easy, there is encouraging evidence that lower spending need not adversely affect health care. Research by John Wennberg, Elliott Fisher, and others indicates that areas of the country where there are high rates of health spending have no better health outcomes or responses to patients' preferences than areas with lower rates of health spending.[4] This suggests that strate-

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gies to reduce spending to the median or lower levels of observed spending could save large sums of money without harming patients. However, providers' incomes would be affected, and the potential for pushback from clinicians and institutional providers should not be underestimated. Compelling evidence that lower levels of health care use can provide as good or better outcomes is likely to be necessary, if not sufficient, for sustaining this change.

Current interest in better information on comparative clinical effectiveness reflects how little is known about what works best, for whom, and under what circumstances. Historically, countries that have used such analysis to support coverage or reimbursement decisions have mostly focused on the comparative effectiveness of new drugs and devices. However, assessment is also important for existing drugs and devices and at least as relevant for medical procedures—maybe more so, because spending is so much greater for procedures than it is for drugs and devices.

As important as better information is, by itself it might not change physicians' (or patients') behavior and probably won't greatly moderate spending. It will also be important to change incentives for clinicians and patients, better align incentives between clinicians and institutional providers, and use information on comparative effectiveness with cost data in setting reimbursement rates. The Commonwealth Fund Commission on a High Performance Health System provides more detail about strategies that can improve value and produce major savings.[5]

Why Government Involvement?

Most groups taking public positions on comparative effectiveness research have explicitly envisioned a government role in its structure, placement, or financing. Nonetheless, the issue of whether the government should be explicitly involved is occasionally raised, as it was in my presentation to Republican staff late in 2008.[6]

For many, government involvement would improve the credibility, objectivity, and balance of the information studied and the methods used. This type of activity produces benefit that extends beyond the funding; economists call these broader effects "externalities." Thus, such activities tend to be underfunded if left to the private sector. Information on comparative effectiveness meets part of economists' classic definition of a "public good": once the information is available, its use by one person doesn't diminish availability for others, and it's hard—although technically possible—to exclude users other than whoever paid for it.[7] If only those who had paid could access certain Web sites containing comparative effectiveness information, individuals and organizations that do not participate in funding the

center could be kept from from accessing the data produced; however, excluding potential users might not be desirable public policy. Private payers could fund as much research as they thought worthwhile, but each would consider their own needs and uses, leading to underproduction of data that might be more broadly useful, as well as to uncoordinated individual initiatives. This could cause undesirable duplication of effort and fragmentation of results. Government involvement in assuring the accuracy and reliability of information is supported by many groups; however, there is less agreement about how much public involvement is desirable in the placement or financing of a center for comparative effectiveness research.

Role Of A Center For Comparative Effectiveness Research

Among several important decisions concerning the nature of a comparative effectiveness research center, the most fundamental is to define its role and functions as well as its scope of work.[8] Two of the most recent pieces of legislation concerning comparative effectiveness research prior to the passage of the stimulus bill—the Comparative Effectiveness Research Act (S 3408, known as Baucus-Conrad for its two sponsors) introduced in the Senate in 2008 and the Children's Health and Medical Protection (CHAMP) Act (HR 3162) passed by the House in 2007—as well as my previous paper laying out options arrive at similar positions about the role, scope, and functions of a comparative effectiveness research center.[9] Each source posits that the primary purpose of such a center would be to provide objective, credible information on the likely clinical outcomes of different strategies to treat the same medical condition. The information produced and disseminated would be intended to better inform clinical decision making and to help design sensible reimbursement strategies. The center thus would serve only an information function. The scope would be all medical technology, broadly defined, including medical procedures and existing as well as new technologies.

■ **Information gathering, not decision making.** This means that the center itself would not make decisions. Payers, both public and private, would use the information in setting policies, but they might use it differently, coming to different decisions regarding reimbursement or coverage on the basis of the same information. It makes more sense to me to use the information for reimbursement policy rather than for coverage decisions, because current Food and Drug Administration (FDA) determinations of safety and efficacy seem both sufficient and appropriate for making coverage decisions; however, payers could and should decide for themselves how best to use such information. Requiring firms or providers to report on the comparative effectiveness of newly covered drugs and devices as a condition of coverage, as the Centers for Medicare and Medicaid Services (CMS) has occasionally done, may be another reasonable way to force the generation of new data to better inform future reimbursement decisions.

■ **Funding research; disseminating data.** The center would fund new re-

search, including clinical trials; collect new data as appropriate and synthesize existing research; and disseminate new as well as existing knowledge about the likely outcomes of different treatments for different population subgroups. The focus should be on treatment of medical conditions rather than specific interventions and therapeutics—although some analysis is likely to compare therapeutics or devices as part of comparing treatment options. For the information to help moderate spending, it must cover medical procedures as well as pharmaceuticals and devices.

■ **Emphasis on comparative data.** The center's output should include comparative data, not only across various interventions, but also across various subgroups—defined now probably by age, sex, or ethnicity, but perhaps in the future by genotype or metabolic type. Analysis of the treatment options for a particular medical condition should not be a one-time endeavor. Given the nature of discovery and the incremental changes associated with many innovations, investments in comparative clinical effectiveness should be ongoing and dynamic.

Cost And Cost-Effectiveness Analyses

One of the most controversial issues concerning comparative effectiveness research is whether to include consideration of the costs of alternative technologies and even more formal cost-effectiveness analysis in the center's mission. S 3408 and HR 3162 explicitly use the term *comparative clinical effectiveness research* to indicate the sponsors' belief that the focus of interest should not include information on cost, although there is a statement in S 3408 that over time, comparative system effectiveness and cost-effectiveness should be explored.

At least two separate issues need to be considered regarding the use of cost and cost-effectiveness information in this effort. The first is whether the generation of such information should be included in a center for comparative effectiveness research, and the second is whether such information should be used in setting coverage or reimbursement rules. I believe that the answer should be no to the first and yes to the second.[10]

■ **Reasons to keep them separate.** There are several related reasons, most of them political, as to why it is important to keep comparative clinical effectiveness separate from cost-effectiveness, at least initially. Generating the kind of comparative effectiveness research that will be needed to address the large variations in how health care is provided in the United States will require sizable investments of both money and time in information. This should be regarded as a critical first step—necessary but not sufficient as a way to change behavior. Without good information on comparative clinical effectiveness information, there will be little comparative effectiveness research worth doing. Such information must be regarded as objective and credible and must be protected from the political process if it is to be used to drive changes in how medical conditions are treated. I believe that having cost-effectiveness information included as part of the comparative effectiveness analyses or as part of the work of an institute or center for comparative effectiveness research will

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taint the clinical effectiveness analyses that are produced or at least will make some of the results suspect.

Many interests will be challenged as the nation moves toward more evidence-based decision making. Some industry groups, patient advocates, and others are already claiming that comparative effectiveness research is just a way to ration care and bring the government into clinical decision making.[11] Including cost-effectiveness analyses in the portfolio of a comparative effectiveness research center will add fuel to this fire. Anything that increases such a center's political vulnerability, which I believe adding cost-effectiveness analyses would do, should be avoided.

In addition, information on cost-effectiveness is comparatively easier and less expensive to generate than the information on clinical effectiveness. It can also be generated separately. The CMS should be funded to do these analyses for comparative procedures that are important to the Medicare population. The CMS would also have to be given the statutory authority to make use of cost information in setting reimbursement rates or coverage—authority it does not now have. Private payers may choose to contract the cost-effectiveness work to not-for-profit groups already doing technology assessments such as the ECRI Institute or the Blue Cross Blue Shield Association Technology Evaluation Center, to add credibility to the analyses and provide some distance between the assessment and the payers.[12]

■ **Bringing value into the equation.** Not including cost and cost-effectiveness information in the activities of a center for comparative effectiveness research does not mean ignoring cost and cost-effectiveness analyses in setting reimbursement or even coverage decisions. Value-based insurance designs, for example, could be combined with information on comparative effectiveness CER so that lower copayments could be set for procedures and population subgroups that are most likely to achieve good clinical outcomes and higher copayments could be set for procedures that are unlikely to benefit that population subgroup or that provide small additional benefit at much greater cost.

Placement Of The Center

Many have argued for keeping the center directly within government; the most commonly cited home is the Agency for Healthcare Research and Quality (AHRQ), which is already responsible for conducting the limited comparative effectiveness research authorized by the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003. HR 1, the fiscal year 2009 American Recovery and Reinvestment Act (ARRA), focuses effectiveness research in AHRQ

but allocates substantial funds for the National Institutes of Health (NIH) and the broader Department of Health and Human Services (HHS). Others have urged not overwhelming the only agency focused on health services research with what may become a very large area when fully implemented. Still others have proposed keeping the research outside direct government control. The Baucus-Conrad bill, for example, places the research with a new entity that is not part of government.

Although any placement will have advantages and disadvantages, the option I find most appealing is to establish such a center as a federally funded research and development center (FFRDC), attached to AHRQ, or as an independent federal agency like the Federal Trade Commission or Federal Communications Commission. An FFRDC is an entity primarily (minimum 70 percent) funded by government, sponsored by an executive agency that monitors its use of funds. These models best reflect the approach of "close, but not too close" to government, promoting balance among credibility, objectivity, and independence; the center would be protected from both the political process and the interests of affected parties.

Legislative Proposals

Three legislative proposals to establish a center for comparative effectiveness research were introduced in 2007; a fourth proposal (S 3408) was introduced by Sen. Max Baucus (D-MT) and Sen. Kent Conrad (D-SD) in the late summer of 2008; and, most importantly, the ARRA contains provisions relating to such research. Sections 4 and 5 of the Medicare Prescription Drug Price Negotiation Act (S 3, 110th Cong., 1st sess.) pertained to comparative effectiveness; this bill was considered but not approved by the Senate in April 2007. In that same Congress and session, the Enhanced Health Care Value for All Act (HR 2184) was introduced in May 2007, and section 904 of the CHAMP Act (HR 3162) passed the House in August 2007.[13] The recently passed ARRA includes less detail about placement, governance, and other aspects; I return to it below.

Because the CHAMP Act included many of the provisions from HR 2184 (Enhanced Health Care Value), I review it here and compare it to S 3408 (Baucus-Conrad) regarding scope of work, placement, governance, and financing.

■ **CHAMP provisions.** The CHAMP Act includes a broad spectrum of treatments and research methods. The activity would be placed in a new center within AHRQ, and the information produced would be disseminated to the general public. An advisory commission, which would broadly reflect stakeholders' and interested parties' concerns, would oversee all activities, with a clinical advisory panel for each research priority. Medicare would fund up to $490 million. Insured and self-insured people would also contribute, with a default contribution of $2 per covered person in FY 2011, which would provide total funding of up to $375 million in FY 2011.

■ **Baucus-Conrad provisions.** S 3408 (Baucus-Conrad), like HR 3162 (CHAMP), directs the research to include a broad spectrum of treatments and to

disseminate the information to the public and stakeholders. S 3408 also includes the HHS secretary and the NIH director on the governing board, in addition to AHRQ and a broad range of stakeholders and interested parties. The most significant difference between S 3408 and HR 3162 is the placement of the center; S 3408 establishes a new nonprofit corporation, the Health Care Comparative Effectiveness Research Institute, which would be completely outside of government rather than in AHRQ. The financing would include a contribution from Medicare phased in to $1 per beneficiary starting in 2013, with an increase tied to medical inflation. Private plans would contribute $1 per covered life in 2013, with the amount also increased by medical inflation.

Other Stakeholders' Positions

■ **MedPAC.** Stakeholders have taken a variety of positions on these issues. The most important is probably that of the Medicare Payment Advisory Commission (MedPAC), because of its advisory role to Congress. MedPAC's recommendations are similar to most of the important provisions in the House bills.[14] The full range of technology is included for consideration, a full range of methods are advocated, transparency and objectivity are emphasized, and a combination of public and private funding is included, although no amount is specified. MedPAC has taken no position on placement or governance, but its 2008 report discusses the design of a governance board to promote independence, objectivity, and stability; the alternatives of where to house a comparative effectiveness entity; and ways to fund it. MedPAC discusses the value of information on cost and cost-effectiveness but does not include any role for such analysis within the entity—yet does not preclude such a role, either. MedPAC also suggests no decision-making role for the entity and, like the discussion here, presumes that the primary role would be to generate findings on comparative effectiveness and disseminate them to patients, providers, and payers, who would then decide how to use the information.

■ **Physician and consumer groups.** Many other stakeholders, including various physician and consumer groups, have also supported the need for comparative effectiveness research. The major distinctions have to do with the degree of stakeholder involvement and the direct consideration of cost and cost-effectiveness in the analysis. Some private-payer and consumer groups have pressed for directly including costs, while most patient advocacy groups and physician and industry groups have resisted such inclusion. The American College of Physicians, however, has publicly advocated the inclusion of cost-effectiveness analyses as part of comparative effectiveness research.[15]

■ **Blue Cross and Blue Shield.** The Blue Cross Blue Shield Association (BCBSA) has proposed a different structure to produce objectivity, credibility, and independence from the political process. It proposes that a comparative effectiveness research center be a congressionally chartered corporation, like the Corporation for Public Broadcasting—similar to the structure proposed by the Baucus-

Conrad bill. The directors of the center would be congressional appointees. The BCBSA believes that a structure outside government would not only better protect the work of the center from political interference but would also enable contributions from the privately insured to be described as an assessment rather than a tax. The latter is turning out to be an issue for at least some Republicans, who have raised concern about the "mystery midnight tax" associated with the comparative effectiveness research provisions of HR 3162.

The Way Ahead

The passage of ARRA unexpectedly changed the landscape of the comparative effectiveness discussion, at least for now. The conference agreement includes $1.1 billion for "comparative effectiveness research" over two years. The Senate attempted to include the word "clinical" throughout the bill to clarify that it refers to comparative clinical effectiveness and not cost-effectiveness, but the term "comparative effectiveness" as used by the House prevailed. I regard this as permissive rather than directive: nothing precludes cost-effectiveness information from being funded with the monies for comparative effectiveness research, but nothing requires funds to be used for this purpose, either.

The conference language also makes clear that the funding is to support research and the dissemination of information on comparative effectiveness and not to be used for mandating coverage or reimbursement, by either public or private payers. This continues the important distinction between an information-generating activity and a decision-making entity advocated in this paper and also by MedPAC, among others. The language also emphasizes the importance of taking subpopulations into account rather than relying only on average effects associated with a particular procedure, device, or therapy.

Compared to the CHAMP or Baucus-Conrad bills, there is very little detail in ARRA about governance, placement of activities, or other aspects of how the comparative effectiveness research will be produced or disseminated. The $1.1 billion will be distributed in several allocations: $700 million is for comparative effectiveness research, with $400 million being transferred to the NIH, leaving AHRQ with $300 million for its own use. An additional $400 million is to be allocated at the discretion of the HHS secretary. In addition, the Institute of Medicine (IOM) is directed and funded to provide a report to Congress and the HHS secretary, by no later than 30 June 2009, on setting priorities for comparative effectiveness research—and is to consider input from stakeholders. In a similar spirit of involving the public and stakeholders, the HHS secretary is to provide the public with an opportunity to comment "to the extent possible."

■ **Little guidance about decision making.** Compared to the detailed language about governance in the various earlier bills, there is little in the ARRA conference bill about how decisions will be made. A federal Coordinating Council for Comparative Effectiveness Research is to be made up of the heads of various relevant federal

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entities such as the NIH and AHRQ, whose recommendations the HHS secretary is directed to consider along with those from the IOM.

■ **A dominant role for government.** The primary result of this initial funding for comparative effectiveness research may be to establish and legitimize efforts in this area, with more detailed legislation to follow after two years. These efforts will include an initial round of priority setting along with systematic reviews of existing studies or previously conducted meta-analyses that shed light on comparative effectiveness findings in areas identified by the IOM and the federal coordinating council. Whether conducting this work in AHRQ and the NIH is appropriate for the long term is one of many issues that may be addressed in the follow-on legislation that will be needed to continue funding. This was the strategy in HR 2184 (the Enhanced Health Care Value for All Act): locating the initial efforts within AHRQ, with a governing board evaluation three years later about where to house the activity thereafter. At the least, however, the stimulus bill clearly indicates a dominant role for government at this early stage. Whether or not this will continue for future efforts is unclear, but if government funding remains substantial, it is likely that government will continue to assume an important role going forward.

■ **Responses from drug manufacturers.** There are many unknowns at this stage—about how the comparative effectiveness research will proceed, but also about how the work produced will be received by industry and the American public. The initial response by some parts of the industry as well as some of the political opposition has been disappointing but perhaps not surprising. For years the pharmaceutical industry has claimed that its therapeutics are cost-effective compared to more invasive interventions such as surgery. The purpose of comparative effectiveness research is to consider alternative strategies for treating a medical condition, including medical and surgical procedures, and to identify those that are most clinically effective for various subgroups in the population. At least in principle, this should provide an important opportunity to break down some of the mental silos within which budget making occurs. It would be unlike the focus of comparative effectiveness efforts in the United Kingdom, France, and Australia, which have primarily focused on head-to-head drug-to-drug or device-to-device comparisons.

■ **A mechanism to slow health spending.** Ultimately, the United States will not be able to continue spending increases on health care at the rate of the past four decades. The pressure on the federal budget from increased spending on Medicare and the stresses on employers and employees from unsustainable increases in private-sector health care spending just won't allow it. Comparative effectiveness research combined with changes in reimbursement that reward and encourage the use of the most clinically appropriate and valuable interventions can help slow health

spending while maintaining incentives for valuable medical innovation. It is not a panacea for what ails the U.S. health care system, but when combined with appropriate changes in the reimbursement system, it seems much better than the alternatives.

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NOTES

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