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Politics and Policy of Comparative Effectiveness: Looking Back, Looking Ahead

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Interest in evaluating which health care interventions work best under what circumstances has surged in recent years as policymakers seek tools to moderate the cost of public entitlement programs and to facilitate affordable coverage expansions in an era of rising health spending. Although recent legislation has taken positions on some of the fundamental issues relating to expanded public support for comparative effectiveness research (CER), a number of key political and policy questions have been deferred to another time and venue. Thus, continuing disagreements may pose challenges to the new CER initiative.

Core Controversies Surrounding CER

The renaissance of federal policymakers' interest in what today is called comparative effectiveness research was reflected, legislatively, in the Medicare Modernization Act of 2003 (MMA). MMA charged the U.S. Secretary of Health and Human Services with conducting and supporting research with a focus on outcomes; comparative clinical effectiveness; and appropriateness of devices, pharmaceuticals, and services. The mandate for this research, with its stated objective of improving the quality, effectiveness, and efficiency of health care delivered in publicly financed programs, coincided with expansion of Medicare to include prescription drug benefits. By 2007, many stakeholders were calling for expansion of the small (\$30 million annually) public investment

ABOUT THIS SERIES

This brief is the first in a series from Mathematica's Center on Health Care Effectiveness highlighting issues that can help inform today's difficult health care decisions. Learn more about the center's work at <http://chce.mathematica-mpr.com>.

in CER. Reaching broad consensus on the need for increased public support for CER was in itself no small feat, given the controversies and concerns that torpedoed previous initiatives. Yet disagreements relating to four fundamental questions have persisted throughout recent legislative debates: "How should CER be financed? Who should direct the new CER initiative? How can CER be used?" and even the most basic question of all, "What exactly is CER?"

Although the American Recovery and Reinvestment Act of 2009 (ARRA) and the recently enacted Affordable Care Act (ACA) include provisions that help answer these fundamental questions, the public discourse over the legislation and competing legislative proposals reflected some of the core controversies about CER. The underlying differences in perspective that fueled this debate will likely continue to influence the development, implementation, and impact of publicly funded CER.¹

Four Outstanding Issues

Financing CER

Legislative consensus emerged early on the usually contentious question of financing, partly as a result of well-learned political history. Both the House and Senate reform proposals established a CER trust fund from an assessment on public and private health insurance. Advocates for renewed public support for

CER wished to develop a funding mechanism that was stable and would minimize opportunities for political interference. They remembered the contentious congressional budget process faced by the Agency for Health Care Policy Research (AHCPR, now the Agency for Healthcare Research and Quality, or AHRQ) in the mid-1990s. During the recent debates, certain stakeholders objected to the financing mechanism ultimately enacted in the ACA (a tap on the Medicare Trust Fund and a tax on private health insurance). Some objected to new taxes generally, or to a tax that would increase (however modestly) the cost of health insurance. Others objected to the seemingly unequal burden borne by businesses offering employees insurance, and still others to the idea of Medicare dollars funding research directed by private stakeholders (see governance debate below). Accordingly, there are numerous reasons why resistance to this public support for CER might reemerge, either before the levy goes into effect in 2013, or in the subsequent debate surrounding renewal of the levy now scheduled to expire in 2019.

Using CER Appropriately

Not surprisingly, the issue of how CER can be used, particularly by public programs, was a topic of intense advocacy in the debate over CER-related provisions in health reform.² The legislative process established answers here as well, at least for the time being. As with the MMA, the ACA emphasizes that informing patients and clinicians is an important focus of CER, with the program even being titled “Patient-Centered Outcomes Research.” Furthermore the legislation stipulates that findings from CER cannot, by themselves, determine Medicare coverage policy. Intense controversy about the role of research findings and expert conclusions in narrowing patient care options—exemplified by the heated rhetoric in the midst of the health reform debate about new U.S. Preventive Services Task Force mammography guidelines—is likely reflected in the framing of this legislative language.

Nonetheless, the United States still faces relentless growth in health care costs, potentially accelerated by public financing of coverage expansions for the uninsured. Furthermore, public investment in CER has been substantially motivated by the hopes that more

and better research can improve the value of current health care expenditures. The nation’s ongoing fiscal challenges seem sure to intensify policymaker and ultimately voter concern over ever-growing federal health spending. Therefore, it seems inevitable that controversy will continue on how CER can best be used to “bend the cost curve.”

Directing the Research

Similarly, intense controversy emerged during the ARRA debate over questions of governance, when the new Federal Coordinating Council for Comparative Effectiveness Research was denounced as the vehicle for impending government rationing of health care. This intragovernmental entity, designed to coordinate CER efforts across relevant agencies, got caught up in antigovernment rhetoric and was repealed by the ACA.

The Patient-Centered Outcomes Research Institute (PCORI), conceived by ACA to direct the new CER initiative, was established expressly as an “outside-government” entity, directed by a board comprising representatives of various stakeholders, including pharmaceutical, device, and diagnostics manufacturers. The decision to hand off CER leadership to a private entity was not without its own controversies. One of these is how to manage potential conflicts between public and private interests. With its multi-stakeholder governance of high-stakes health care research, its control of a large tax-supported trust fund, and its complex relationship to—and potentially overlapping mission with—existing federal research agencies, the implementation of this new institute presents other formidable policy challenges as well.

For example, how will this private entity’s board interpret the congressionally mandated “preference” to use AHRQ or the National Institutes of Health (NIH) to conduct research within their authority? Congress has used the term “preference” for decades to guide federal agencies, which have developed relatively specific interpretations relevant to a variety of regulatory and grant-making activities. Preference will now be applied, however, by the staff of a nonprofit corporation responding to a private board of governors. How will they understand the optimal role of existing federal research agency capabilities in implementing

the ambitious research agenda for CER? And how will the federal agencies respond to offers of contracts to conduct research from this new entity?

Ideally, PCORI will use its independence and multi-stakeholder perspective to undertake the politically contentious work of identifying priority topics and research questions for investigation. The institute could then turn over to experts at federal research agencies those elements of CER that require a comprehensive understanding of how to generate high quality research in the public interest. Even under optimal circumstances, this process will not be simple. One risk is that the new institute might become its own quasi-federal research agency governed by stakeholders, rather than scientists, but duplicating many of NIH's and AHRQ's functions and capabilities.

Defining CER

Perhaps the most fundamental question left to this multi-stakeholder board is "What is (and is NOT) CER?" The recent policy debates have reflected conceptions of CER that differ in breadth and scope, resulting in misperceptions and confusion about what can be expected from investments in this research.

One controversy relates to the appropriate scope of inquiry embodied in CER. ACA settled a House-Senate ARRA debate by reinserting the word "clinical" into the term "comparative effectiveness," thereby reverting to MMA terminology. Advocates who preferred this construction perceived that it focused the research on developing information of the greatest relevance to clinicians, precluding research focused on cost comparisons, especially cost-effectiveness analysis. Similarly, ACA includes explicit prohibitions on the use of CER funds to calculate so-called quality-adjusted life years, used in assessing the relative value of interventions. The ACA stance reflects a decision to avoid conflating research findings about the relative effectiveness of alternative approaches with judgments about the value of any incremental improvements found. Such information on marginal cost-effectiveness has proven meaningful in public efforts in which the citizens accept the legitimacy of restricting access because of cost considerations; but the recent debate suggests the U.S. public is not ready. Perhaps continued pressure from health cost

growth will change the public perspective, but for the present, cost-effectiveness evaluation will not be part of ACA-financed CER.

Another dispute relates to whether CER encompasses studies of how care is best delivered. To some policymakers, the core mission of CER is to solve the problem that "little rigorous evidence is available about which treatments work best for which patients."³ Accordingly the CER provisions passed by the House (but not the Senate) in 2007 expressly focused on informing decisions "at the point of care." Subsequent advocacy from an array of stakeholders so broadened this interpretation that, in the ACA, CER includes not just traditional treatments (drugs, devices, and procedures) but also "... delivery, ... integrative health practices, and any other strategies ... used in the ... management ... of illness or injury in individuals."

Some of this expansion in definition reflects a sophisticated understanding that many of the choices faced by clinicians and patients at the point of care go beyond a simplistic "red pill or blue pill" or even "medical therapy versus surgery" formulation. Are inpatient rehabilitation facilities better than home care for recovering function after a hip fracture? Which community services are most helpful to Alzheimer's patients and their caregivers? As the Institute of Medicine noted, there are many high-stakes questions for which evidence could help inform clinician and patient decisions. And perhaps an independent, nongovernmental, multi-stakeholder board can address these questions in a way that is perceived as more legitimate and even-handed than a federal agency or a presidential task force or commission.

But there are many other research questions that do not have the same salience to the point of care. Although practice managers and payers need to know the relative merits of electronic health records or nurse case managers in transforming primary care practices into medical homes, for example, these are not issues that must be decided by individual patients and the clinicians treating them. Similarly, hospital administrators may have an intense interest in research on how best to reduce staphylococcus infections in their facilities, but other stakeholder preferences are likely not critical issues here. Six

hundred million dollars a year is not a lot of money to spend when answering all the point-of-care clinical evidence quandaries (especially in relation to the multi-billion-dollar annual investment in innovation discovery by NIH and biotechnology corporations). Of course, each PCORI trust fund dollar spent to answer a health services research question is one less dollar to resolve a pressing clinical controversy. This can delay needed answers at the bedside and possibly facilitate continued use of a profitable but relatively ineffective product or service. How PCORI defines CER and determines the appropriate scope of its work certainly has implications for how quickly better clinical evidence can be brought to bear to address costly and unnecessary care variations.

Resolution Deferred

The ACA makes an important public investment in CER, but as with other elements of health reform, key political controversies remain, and a number of crucial policy questions have been deferred for resolution at another time and venue. The manner in which these issues are revisited, and the ultimate outcome of the impending debate, may well prove critical to the future role of CER in enhancing U.S. health care.

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Notes

- ¹Rich, E.C. "The Policy Debate over Public Investment in Comparative Effectiveness Research." *Journal of General Internal Medicine*, vol. 24, no.6, 2009, pp. 752–757.
- ²Docteur, E., and R.A. Berenson. "How Will Comparative Effectiveness Research Affect the Quality of Health Care?" Issue brief, Quick Strike Series: Timely Analysis of Immediate Health Policy Issues. Princeton, NJ: The Robert Wood Johnson Foundation and the Urban Institute, 2010.
- ³Orszag, P.R., and P. Ellis. "Addressing Rising Health Care Costs—A View from the Congressional Budget Office." *New England Journal of Medicine*, vol. 357, no. 19, 2007, pp. 1885–1887.

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