Ellen Sigal, president of Friends of Cancer Research and a member of the Patient-Centered Outcomes Research Institute's governing board, says initial opposition to comparative effectiveness research within the cancer community turned to support after cancer advocates realized that the research techniques could lead to a better understanding of which therapies work best for individual patients.

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The Political Fight Over Comparative Effectiveness Research

The creation of a public-private institute to direct new comparative effectiveness research represents a challenging new chapter in America's on-again, off-again support for determining what works in health care.

BY JOHN K. IGLEHART

In a country where the virtues of smart shopping are often heralded, you might not think it especially controversial that Americans should want to purchase effective health care. Thus, embedded in the Patient Protection and Affordable Care Act is a new public-private initiative to determine which therapies, care management, delivery models, and even public health programs accomplish the most good. Yet so controversial was the initiative that it spent weeks in surgery before materializing in the final reform package.

On one level, its survival illustrates how Congress's Democratic majority ultimately prevailed over Republican rhetoric about a "government takeover" and rationing of health care. On another level, it's a story of how the Democrats succeeded only after making a series of accommodations with private interests[1]—ranging from patient advocacy groups to members of the medical-industrial complex, including pharmaceutical and device manufacturers.

The Affordable Care Act created the Patient-Centered Outcomes Research Institute, an unusual public-private nonprofit enterprise that will fund comparative effectiveness research, the topic of this October 2010 thematic issue of *Health Affairs*. The institute will draw on a dedicated trust fund of dollars from the Medicare program and contributions from private insurers. Initial expectations are that its annual funding may grow to about $500 million within a few years, depending on how future federal budget battles play out.

The law charges the institute with establishing a comparative effectiveness research agenda set by private stakeholders, rather than by the government or purely science-minded researchers. In setting that agenda, these stakeholders are charged with focusing on the most common and widespread conditions, with a particular emphasis on chronic disease. Congress also directed the institute to pay close attention to subpopulations of patients, such as racial and ethnic minorities, to determine which groups could derive the most benefit from various treatment approaches.

Overcoming Rationing Rhetoric

The Affordable Care Act's passage was preceded by months of oft-voiced fear and loathing over these comparative effectiveness research provisions. The backlash began early in the Obama administration, when the president proposed to allocate $1.1 billion for such research as part of the stimulus legislation, the American Recovery and Reinvestment Act of 2009. Many Republicans, private foundations that support their causes, and conservative pundits went into rhetorical overdrive.

Rep. Tom Price (R-GA), a physician, sent out an "alert" through the Republican Study Committee that claimed comparative effectiveness research legislation would strip doctors and patients of the right to make health care decisions, creating "a permanent government rationing board" that would prescribe care.[2]

Other outspoken Republican critics of comparative effectiveness research included House Minority Leader John Boehner of Ohio; Rep. Phil Gingrey of Georgia, another physician; Sen. John Kyl of Arizona; and Sen. Pat Roberts of Kansas.

TOWN HALLS AND PUNDITS Amid increasing concern at public "town hall" meetings about health reform in the summer of 2009, conservative pundits also weighed in. One was Betsy McCaughey, a former lieutenant governor of New York. McCaughey linked the increased comparative effectiveness research funding in the stimulus law to another of the law's provisions: giving incentives for doctors and hospitals to adopt electronic health records. She asserted that by promoting both initiatives at once, the federal government planned to monitor individual patient care decisions via surreptitious electronic means and then punish those physicians who failed to comply with rationing guidelines.

Radio talk-show host Rush Limbaugh became McCaughey's megaphone, disseminating her message to millions of listeners.[3] Columnist and commentator George Will criticized the manner in which comparative effectiveness research was expanded "stealthily" through the stimulus law, asserting that an activity that "would dramatically advance government control—and rationing—of health care, should be thoroughly debated."[4]

LIKE BRITISH HEALTH CARE Physician Scott Gottlieb, a former deputy commissioner of the Food and Drug Administration (FDA) who is closely identified with segments of the pharmaceutical industry, warned that "incorporating an explicit tie between the results of research and coverage decisions will put us squarely on a path that more closely resembles the process used in Britain—with all of its shortcomings on access, innovation and health outcomes."[5]

Analysts at two conservative foundations were also critics of comparative effectiveness research. Michael Cannon of the Cato Institute maintained that "government provision of comparative-effectiveness information may do little or nothing to increase efficiency compared to a policy of laissez faire."[6] Kathryn Nix of the Heritage Foundation said that with Donald Berwick directing the Centers for Medicare and Medicaid Services (CMS), "the likelihood of [comparative effectiveness research's] being used to deny coverage based on cost becomes ever more worrisome."[7]

A History Of Support

The irony that comparative effectiveness research was now deemed so diabolical wasn't lost on political observers and health policy specialists. For one thing, this type of research is scarcely new. Cancer researchers, for example, point out that the classic trial of a new cancer intervention is almost always a comparative effectiveness study, in which a new therapy is compared to the standard treatment that current professional consensus deems appropriate for a particular patient.

What's more, for years, many Republicans had heartily supported expanding comparative effectiveness research. During his presidential election campaign in 2008, Sen. John McCain (R-AZ) had squarely backed the research, although he opposed its use by Medicare in making coverage and reimbursement decisions. Former House Speaker Newt Gingrich of Georgia, who among other activities now heads a consultancy known as the Center for Health Transformation, has argued repeatedly that "doctors should have better access to concise, evidence-based and actionable medical information."[8] Other prominent Republican supporters of comparative effectiveness research included Gail Wilensky, *Health Affairs'* adviser on this thematic issue and a former administrator of Medicare and Medicaid who also served as White House health policy adviser to President George H.W. Bush.[9,10]

ELECTION IN 2008 HARDENED OPPOSITION But once Democrats swept the 2008 election, Republican support for comparative effectiveness research vanished. As the party girded for battle over health reform, a clear line of attack was to link the research with the "government takeover of health care" and rationing themes. As health reform legislation worked its way through House and Senate committees during 2009, Democrats had to wield their majorities on the panels to defeat numerous comparative effectiveness research amendments. In general, Republicans offered amendments to reduce spending on effectiveness research and to restrict the uses of such research.

The ranking Republican member of the Senate Health, Education, Labor, and Pensions Committee, Sen. Michael Enzi of Wyoming, was eventually able to negotiate a few changes with senior Senate Democrats, including Finance Committee chair Max Baucus of Montana and Budget Committee chair Kent Conrad of North Dakota. Among them: prohibition of the use of a metric known as quality-adjusted life-years (QALYs).

LACK OF US SUPPORT FOR QALYS Although originally developed as a broader measurement of disease burden beyond mortality, QALYs are now used in cost-effectiveness analyses to aid coverage and reimbursement decisions worldwide.[11] Among those making use of the QALY measures is the National Institute for Health and Clinical Excellence (NICE) of England and Wales, which uses a cost-effectiveness threshold range of £20,000–£30,000—or about $33,000–$50,000—per quality-adjusted life-year as a basis of recommending whether the National Health Service should cover new therapies.

During US health care reform, QALYs quickly became more code language for government-run health care systems and rationing. Thus, Democrats agreed with Enzi on compromise language saying that the Patient-Centered Outcomes Research Institute "shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of health care is cost effective or recommended."[12]

Accommodating Interest Groups

On another front, Senate Democrats and their staffs worked behind the scenes to address a separate set of con-

cerns about comparative effectiveness research. These came from the Partnership to Improve Care, a coalition of patient and industry groups, organized medicine, health services research groups, organized labor, and other stakeholders with a range of worries. Many focused on fears that the research could threaten biomedical innovation and restrict patients' access to costly new treatments. Thus, these organizations lobbied successfully to win seats on the new institute's board.

The Affordable Care Act directs the Government Accountability Office (GAO) to fill all twenty-one slots on the board with representatives of specific interests: consumers; hospitals; industry; nurses; payers; physicians; researchers; surgeons; and two government agencies, the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH). (The GAO is now scheduled to announce those appointments on September 23, 2010.)

Tony Coelho, a former California Democratic representative who heads the Partnership to Improve Patient Care, says that the board's makeup will clearly prove determinative in how the initiative proceeds. "Tell me who gets on the board," he said in a recent interview, "and I can tell you what comparative effectiveness research looks like."[13]

CHANGING VIEWS The views on comparative effectiveness research of some major interest groups—such as patient and research advocacy groups as well as device, diagnostic, and drug manufacturers—evolved over the course of negotiations with Democrats. Cancer groups were a case in point. Ellen Sigal, president of the Friends of Cancer Research and a member of the Patient-Centered Outcomes Research Institute's governing board, noted recently that the "concept of comparative effectiveness research was originally received with mixed emotions within the cancer community." The hesitation, she explained, partially stemmed from the parallels that were drawn with Britain's and other nations' health systems.[14]

However, after cancer advocates examined how comparative effectiveness research "could utilize various study methods and data sources to lead to a better understanding of treatment outcomes in different populations, it became clear that this could be a step toward 'personalized' medicine," Sigal said. And amid exploding knowledge about the unique genetic signatures of different cancers, it has become clearer than ever that comparative analysis of different interventions will be key to understanding which therapies will work best for individual patients.

With the Patient-Centered Outcomes Research Institute structured as an independent, quasi-governmental body with its own board, "we felt that the [new institute had] a proper level of independence and flexibility, sustainable funding, as well as the necessary ability to take advantage of the diverse expertise that exists across multiple federal agencies and other organizations," Sigal said. So her group joined with other organizations that had supported comparative effectiveness research from the start, including AARP, the AFL-CIO, and Consumers Union. The last group initially favored expanding the comparatively modest comparative effectiveness research program that already existed within AHRQ, as called for in the House-passed version of health reform legislation, but later signed onto the Senate template that ultimately became law.

GAINING PHRMA'S SUPPORT Negotiations with device and drug manufacturers were particularly critical, since these companies had little taste for research that would necessarily compare one company's products against another's, often in head-to-head trials. These organizations also worried about the erection of even subtle barriers against technological innovation—not to mention full-throated government rationing.

Ian Spatz, a private consultant who was Merck's vice president for global health policy, noted in a conversation that the traditional view of pharmaceutical companies "has been that every comparative effectiveness research proposal is a slippery slope that leads to NICE—and NICE is seen as pure evil." However, after drug makers achieved most of their legislative goals, Pharmaceutical Research and Manufacturers of America (PhRMA) came out in support of the comparative effectiveness research initiative. PhRMA's chief comparative effectiveness research legislative goals were to make certain that research findings were transparent, that the administrative agency was an enterprise that operated independently of government, and that its members were represented on the board.

In an interview, Richard Smith, PhRMA's senior vice president for policy, said, "By including the full range of stakeholders in its governance, by defining the scope of research to include the full range of treatment options and the organization, delivery, and management of care, the institute is charting a different, more positive course than agencies in other countries which focus on cost-effectiveness and impose centralized restrictions on access to care." Two Eli Lilly executives agreed with that assessment, writing in a recent article, "In our view, the companies that will survive and thrive in this new environment will be those that embrace comparative effectiveness research (CER) as the next logical step in the progression of requiring evidence and recognize it as a necessary input for a value-driven healthcare system."[15]

In a letter to Senators Baucus and Conrad, Stephen Ubl, CEO of the Advanced Medical Technology Association (AdvaMed), also wrote in support of the comparative effectiveness research legislation.[16] Ubl applauded the openness of the legislative process and the law's assurance that the research entity "does not make coverage decisions and the studies conducted do not include coverage recommendations or clinical practice guidelines."

COALITIONS BROUGHT IN Private organizations that Democrats corralled to support their comparative effectiveness research vision were not always on the same page, however. One issue that proved divisive was potential conflicts of interest among the new institute's board members, as well as institute policies for publishing the results of whatever research it ultimately funds.

In the end, a coalition of groups that included the AFL-CIO, the Center for American Progress, Consumers Union, the National Partnership for Women and Families, and the Pacific Business Group on Health successfully pressed for language that required all institute

board members to disclose any direct financial benefit that could redound to them based on the findings of any comparative effectiveness study. The coalition also joined medical research groups to help persuade the Senate Finance Committee staff to modify language that they asserted gave industry too strong a voice in determining whether research results could be published.[17] But even here, industry won a small victory. According to the legislation, the institute's twenty-one-member board, which includes three industry representatives, will be able to impose penalties on researchers whose published results are deemed to be not "entirely consistent with the evidence."[18]

Institute Marks New Chapter

The creation of the institute represents a challenging new chapter in America's on-again, off-again support for determining what works in health care. On the one hand, results published in *Health Affairs* from a recent survey suggest that many members of the public may stand with some Republicans in their opposition to medical evidence of any sort—other than what their personal physicians say is needed.[19] On the other hand, by embracing a public-private model of governance, Congress created an unusual entity that will be the beneficiary of millions of dollars in taxpayer revenues and introduce a new model of research priority setting.

Many threshold questions remain. Will physicians actually change their practices based on comparative effectiveness research findings, or will additional incentives be necessary to alter their professional behavior? Will private insurers and federal programs—especially Medicare—apply these findings to their coverage decisions, even though the Affordable Care Act stipulates that its language should not be construed as permitting institute-funded research to "mandate coverage, reimbursement, or other policies for any public or private payer"?[18]

These questions and others are likely to play out over many years—assuming that the Affordable Care Act and the institute survive a possibly difficult period just ahead. With some twenty states suing in court to overturn provisions of health reform law, and House Republicans vowing to try to repeal the legislation if they win a majority in the November 2010 elections, the debate over the role of comparative effectiveness research in US health care is clearly far from over. ■

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