Comparative effectiveness research: policy and politics

EDIE E. ZUSMAN, M.D.
Sutter East Bay Neuroscience Institute, Castro Valley, California

Comparative effectiveness research (CER) is the basis for some of the fiercest rhetoric of the current political era. While it is a relatively old and previously academic pursuit, CER may well become the foundation upon which the future of health care in the US is based.

The actual impact of CER on—and uptake among—doctors, patients, hospitals, and health insurers, however, remains to be seen. Political considerations and compromises have led to the removal of key aspects of CER implementation from policy legislation to prevent alienating stakeholders critical to the success of health care reform.

Health care providers, including specialists such as neurosurgeons, will need to understand both the policies and political implications of CER as its practices becomes an indelible part of the future health care landscape.

(http://thejns.org/doi/abs/10.3171/2012.4.FOCUS1298)

KEY WORDS • comparative effectiveness research • socioeconomics • health policy • Affordable Care Act • health care reform

Comparative effectiveness research is defined by the Institute of Medicine as the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care. 8,10,11

Randomized controlled trials, which identify causal relationships between treatments and health outcomes, are not designed to answer more intricate questions about how a new therapy should be considered for use in the context of existing treatment options. Comparative effectiveness research, however, may have the potential to generate more and better evidence on what works best and therefore add to the evidence used in selecting treatments.9

Widely used in other countries, and supported at one time by both Democrats and Republicans in the US, CER has at once been cited as a tool to improve health care quality, control health care costs, and, most controversially, to ration health care.4,7

History

Although a new concept to many in today’s political arena, the roots of effectiveness research can be traced back to mid-18th century Scotland and the “arithmetical medicine” practiced by the graduates of Edinburgh Medical School,4 where 6 treatments for scurvy were tested and compared in a controlled trial. In Paris in the 1830s, Pierre Louis developed a method demonstrating that phlebotomy did not improve survival rates of patients with pneumonia,7 and at the beginning of the 20th century, an American physician, Ernest Codman, founded “outcomes management,” in which he examined hospital efficiency by tracking patient care errors.

American policymakers most often cite CER in England, where in the 1930s health services research gained popularity. Later, after decades of socialized medicine, researchers found evidence of geographic variations in the provision of care including operations such as appendectomy, caesarean section, cholecystectomy, hysterectomy, tonsillectomy, and prostatectomy.2

In the US, geographic variations in prostate care were highlighted in a 1972 paper by Archie Cochrane, leading to concerns that care was delivered differently depending on the recipient’s geographic location. In some areas, there was underprovision of care, whereas in other areas there was overprovision of care, and many of the treatments delivered were deemed ineffective. Since then, various agencies have evolved in the US to conduct and promote research on the evidence of technologies and treatments, as well as their cost effectiveness, and the results have differed. The agencies include the National Center for Healthcare Technology (1978–1981) and the Office of Technology Assessment, which from 1972 to 1995 issued a series of reports on a variety of process- and disease-oriented health care matters.9

Evidence-based medicine, a similar strategy, emerged in the 1980s as a formal approach to using scientific data to inform medical decisions and to assist in the develop-
Development of practice guidelines suggesting validated treatment plans for common medical conditions.

Health insurance providers recognized early on some of the practical applications of CER. For example, in 1985, the Blue Cross and Blue Shield Technology Evaluation Center was among the first agencies to conduct multidisciplinary investigations of comparative effectiveness of novel health care technologies.

The Agency for Healthcare Policy and Research was established in 1989 to “enhance the quality, appropriateness, and effectiveness of health care services and access to care.” The agency undertook a number of initiatives, including development of a clearinghouse for medical evidence on appropriate treatments for conditions like back pain. A series of 15 guidelines related to spinal surgery, for example, drew strong opposition from spine surgeons, who called for elimination of the agency.

The term “policy” was dropped in 1996 from the Agency’s name to emphasize its primary commitments to research and quality, creating the Agency for Healthcare Research and Quality (AHRQ). The agency currently functions as an arm of the Department of Health and Human Services and is a prominent federal funding agency for CER in the US.

The AHRQ funding for research to compare outcomes and clinical effectiveness was bolstered in 2003 through a $50 million investment as part of the Medicare Modernization Act. The funds were allocated to conduct and support effectiveness research for Medicare and Medicaid enrollees. In addition, the Department of Veteran Affairs conducts research based on reviews of medical records to determine the clinical effectiveness of treatments, and it funds clinical trials to compare drug therapies. The Centers for Medicare and Medicaid Services also has helped sponsor research on comparative effectiveness, mostly to determine whether to establish separate payment rates for similar treatments.

Although these efforts conceptually served different purposes, the common thread was the integration into decision making of clinical evidence about an intervention or service.

However, while CER has been used by government fairly broadly since then, its policy potential was not fully realized until 2009, when President Obama signed into law the American Recovery and Reimbursement Act, the stimulus bill that included a $1.1 billion allotment for CER.

The funds were divided among 3 federal agencies: the Office of the Secretary of Health and Human Services ($400 million), the National Institutes of Health ($400 million), and the AHRQ ($300 million).

The Patient Protection and Affordable Care Act, the health reform legislation passed in 2010, included creation of an independent, quasi-governmental body called the Patient-Centered Outcomes Research Institute (PCORI) with a diverse panel of board members that funds CER through a trust fund of dollars from the Medicare program and contributions from private insurers.

Research priorities, restrictions, and oversight duties were outlined in additional legislation including the Comparative Effectiveness Research Act of 2009 (CERA), the Patient-Centered Outcomes Research Act of 2009 (PCORA), the Health Care and Education Reconciliation Act of 2010, in addition to the Patient Protection and Affordable Care Act.

The PCORI is charged with determining a CER agenda set by private stakeholders, rather than by the government or by purely science-minded researchers. The group is expected to focus on the most common and widespread conditions, with a particular emphasis on chronic disease. The institute also is charged with paying close attention to subpopulations of patients to determine which groups could benefit most from different treatment approaches.

Topics were diversified to include research into screening, prevention, and monitoring, as well as medical, surgical, and other therapeutic interventions and alternatives. The final portfolio contains a list of 100 major topics that will receive federal CER funding.

Institute commissioners, appointed by the General Accounting Office, include representatives from patient advocacy organizations, health insurance, academic medical centers, private health care providers, drug and medical device manufacturers, and veterans’ and disability rights organizations, among others (http://www.pcori.org/). One highly controversial component regarding PCORI is that in its current structure, it works outside of congressional approval and oversight.

The law specifically prohibits the institute from making clinical, coverage, or reimbursement recommendations on the basis of evidence generated at its direction. This may well have been the political compromise necessary to keep an infrastructure for CER in the health care reform legislation without dividing the stakeholders over another controversial aspect, the implication of using cost and outcomes data to fund or authorize specific health care practices—what some may term rationing.

It is likely, however, that even without PCORI’s direct authority to act on CER results, publishing and promulgating these findings within the health care community for the review of Centers for Medicare and Medicaid Services and insurers, this information will still have a significant impact on health care–funding decisions. For example, the Secretary of Health and Human Services does have authority to use CER findings under certain conditions. Insurers and health systems will have access to CER results and publications and will be able to use these findings in concert with other studies to generate evidence-based guidelines for determining care coverage.

The Goal of CER

Why CER evolved from a mostly scientific pursuit in the 1970s and 1980s into a core piece of highly politicized health care reform in the 21st century relates to its potential in reducing health care costs, which are estimated to increase to 19.1% of gross domestic product, or $4.4 trillion, by 2018. Expenditures have risen in both the private and public health care sectors, due in part to the development and spread of new and more costly medical technologies.

Research has demonstrated that the US has the most expensive health care in the world, and yet by many mea-
Comparative effectiveness research: policy and politics

The insurance industry. Existing system—physicians, hospitals, drug makers, and powerful groups by endorsing a plan that minimized costs be left out of the reform equation. Hasting Center has concluded that efforts to win acceptance by key stakeholders required that certain cost controls be left out of the reform equation. Those efforts will intensify within health care as the US, implementing health care reform, has to absorb 32 million currently uninsured people into the health care system while also improving health care quality and slowing cost increases.

In addition to EBM, CER has been embraced as a tool to support efficiency practices and ultimately control health care costs by “paying for what works,” eliminating medical care variation, reducing overtreatment, and curtailing the use of expensive care when it is ineffective care. Comparative effectiveness research also is designed to fill knowledge voids from randomized clinical trials with practice-based research, practical clinical trials, and observational studies. The application of evidence derived from CER is anticipated to improve quality of care by providing data related to treatment efficacy, quality of life, and outcomes and to assist clinicians with making informed patient management decisions.

Political Reality

The politics of CER are complex, ranging from economic to philosophical differences of opinion and analysis, and have led to compromises that some argue may undermine the potential benefits of CER as a tool to improve the quality and delivery of health care. Passage of the Affordable Care Act, which enabled government-sponsored use of CER, itself required compromises on many fronts. Michael K. Gusmano of The Hastings Center has concluded that efforts to win acceptance by key stakeholders required that certain cost controls be left out of the reform equation. Seeking to avoid the Clinton-era health reform failure, the Obama administration limited opposition from powerful groups by endorsing a plan that minimized threats to the incomes of those who benefit from the existing system—physicians, hospitals, drug makers, and the insurance industry. Once those groups endorsed the legislation, Republican opposition waned.

Opponents of health care reform measures often argue that rather than reduce spending, reform measures will make health care more costly. As a key tool of health reform, for example, CER aims to eliminate unnecessary care, saving billions while improving quality. Gusmano has argued that the experiences with CER abroad suggest that the efforts are just as likely to increase spending because new medical technologies, even when considered cost effective, usually cost more.

Gusmano added that preventive care, long viewed as a method to reduce spending by keeping people healthy, may improve health, but it often increases costs to employers and others who pay for health care.

The distaste among American policymakers for using cost to evaluate health care technology also means that investments in CER will be less likely to curtail use of technologies that are not cost effective. Patient advocacy groups, too, often oppose curtailing the use of existing technology, even when CER demonstrates that technology is not cost effective. For example, despite evidence based on CER that screening mammography in women between the ages of 40 and 49 years may do more harm than good, a broad spectrum of professionals, advocates, and policymakers rejected the findings.

Convincing consumers and their providers of the value of CER also may be problematic. Many Americans believe they have the best health care system in the world, certain that innovation and expenditures go hand-in-hand with quality and better outcomes.

Whether physicians change the way they treat patients or whether private and public health insurance payers apply CER findings to coverage decisions is also a matter of speculation, according to John K. Iglehart, founding editor of Health Affairs, in “The Political Fight Over Comparative Effectiveness Research.”

Iglehart and others predict that there will be less incentive to use CER because 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.” These qualifications were made in the law in response to concerns that government-sponsored health care studies would ultimately lead to homogenized (one-size-fits-all) treatment recommendations that ignored patient heterogeneity.

Rationing Concerns

Perhaps the most explosive concern has been the perception that CER will be used to ration health care, making it more difficult for people with a lower likelihood of benefiting from certain treatments or procedures to get coverage for those treatments. That view has been taken up by a large and vocal contingent of Republicans.

Although CER became controversial after President Obama took office, the concept had at one time been embraced by both Democrats and Republicans. Sen. John McCain, for example, backed the research during his presidential election campaign in 2008. Former House Speaker Newt Gingrich, of Georgia, agreed that “doctors should have better access to concise, evidence-based and actionable medical information.”

Cancer researchers have pointed out that trials of
new cancer interventions are almost always comparative effectiveness studies, in which new therapies are compared with standard treatments that have been deemed appropriate for certain patients.\textsuperscript{3}

Still, opponents of CER cited worries that it would lead to treatment recommendations for certain conditions that were used to justify cost-control efforts,\textsuperscript{12} and those would lead to indiscriminate coverage restrictions that could hurt patients.

The term “death panels” to describe the potential impact of CER\textsuperscript{1} is used frequently by politicians, including those vying for the 2012 Republican presidential nomination, who have vowed to repeal health care reform laws if elected. They are not alone in their opposition. Already, according to the National Conference of State Legislatures, 17 states have enacted legislation to challenge elements of federal health reform, mostly in opposition to the law’s mandate for health insurance coverage.

As a result of the various concerns, legislators worked to ensure that the Secretary of Health and Human Services could only use CER findings in coverage determinations with certain caveats, namely that coverage decisions based on CER must be developed in a transparent manner and be subject to public- and peer-review processes.\textsuperscript{9}

The law also prohibits use of a QALY to set a threshold for decision making. Quality-adjusted life year measures are used by the National Institute for Health and Clinical Excellence (NICE) in England and Wales, which uses a cost-effectiveness threshold range of about $33,000–$50,000 per QALY as a basis of recommending whether the National Health Service should cover a particular therapy.\textsuperscript{3}

In the US, QALYs became code language for government-run health care systems and rationing. Ultimately, Democrats agreed to language in the law that the PCORI “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 threshold to establish what type of health care is cost effective or recommended.”\textsuperscript{11}

Whether omission of QALY and a prohibition of coverage recommendations will prevent use of CER for these purposes, however, is not clear.

Writing in the Journal of Managed Care Pharmacy, Prasun Subedi, Ph.D., Director of Worldwide Policy, and Eleanor M. Perfetto, Ph.D., Senior Director of Reimbursement and Regulatory Affairs for Pfizer, Inc., suggested that private payers will use the PCORI findings in their own economic evaluations and will make coverage and reimbursement determination based on the assessments.\textsuperscript{9}

**Effects on Innovation and Personalized Care**

Beyond coverage and cost-control concerns are those suggesting that CER will lead to restrictions on coverage that would translate into reduced investment in health care innovations.

In a 2009 report to President Obama and Congress, the Coordinating Council for CER, established to foster coordination of CER conducted or supported by the federal government, summarized that expansion of CER had 3 implications: that the results would better inform health care decisions; that CER represents an investment in steps to improve quality and value of health care for all; and that CER has the potential to drive high-value innovation and enable the practice of more personalized medicine based on subgroups of patients.\textsuperscript{3}

Nevertheless, skepticism is pervasive. The Partnership to Improve Patient Care, a coalition of 36 industry, patient advocacy, and clinician organizations, for example, has raised concerns that CER will not take adequate account of individual patient differences and may impede development and adoption of improvements in medical care and “stymie progress in personalized medicine.”\textsuperscript{11}

According to Alan M. Garber,\textsuperscript{1} a staff physician at the Veterans Affairs Palo Alto Health Care System and a Stanford University health care policy researcher, those concerns are unfounded. He has argued that rather than impeding personalized medicine, CER can hasten discovery of the best approaches to personalization with better information for managing individual patient needs.

Garber\textsuperscript{1} cited obstacles to adoption of personalized approaches such as genomic testing because of a lack of adequately designed studies assessing their clinical utility. He suggested that CER could be used to establish the evidence needed to determine the appropriate use of genetic tests to identify a patient’s risk for certain diseases or the potential benefit of certain treatments. Rather than rely solely on findings of formal studies with clinical judgment, physicians could make more informed decisions about individual patient treatment with well-designed comparative effectiveness studies.

**Conclusions**

Despite differences in opinion about how comparative effective research will ultimately inform and influence health care delivery, its use will likely expand as the nation moves to ensure that every American receives health care coverage, that the rate of health care expenditures slows, and that the quality of care improves for all consumers. No sector of the medical establishment, including specialty care like neurosurgery, will be unaffected. Even with continued political opposition, restrictions for CER use included in current health care policy, and both consumers’ and physicians’ rejection of CER as a means to guide treatment decisions, economic realities will likely drive its use nevertheless.

**Disclosure**

The author reports no conflict of interest concerning the materials or methods used in this study or the findings specified in this paper.

**References**


E. E. Zusman

Neurosurg Focus / Volume 33 / July 2012
Comparative effectiveness research: policy and politics


Accepted April 3, 2012.
Please include this information when citing this paper: DOI: 10.3171/2012.4.FOCUS1298.
Address correspondence to: Edie E. Zusman, M.D., Sutter East Bay Neuroscience Institute, 20055 Lake Chabot Road, Suite 110, Castro Valley, California 94546. email: zusmane@sutterhealth.org.