

Comparative Effectiveness: Better Value for the Money? August 2008



ALLIANCE FOR
HEALTH REFORM

Comparative effectiveness (“CE”) research is a hot topic these days because it offers the attractive prospect of cutting costs while improving the quality of health care.

Simply stated, CE aims to assess how various procedures or interventions for a given ailment compare with each other. CE is part of a broader movement to make sound science-based evidence the basis for medical practice. Most clinical trials tell us how an individual therapy compares to a placebo. But such studies usually do not provide head-to-head comparisons of two or more therapies.

In other words, most of our current research merely asks whether a given drug or procedure is better than nothing, but tells us little about which therapy among a range of possibilities works best under a given set of circumstances.

The Congressional Budget Office, in a December 2007 report, said CE is “...a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches, such as surgery and drug therapy. The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options.”

Interest on the Hill was boosted by the CBO report, which said that over the long term, comparative effectiveness research would probably reduce health care spending.

CBO suggested in a subsequent report that CE research might help ensure that

costly services will be used only when they offer a clinical benefit greater than that offered by less costly services.

But as with many of the other quality/cost-containment tools, the CE devil is in the details. How well comparative effectiveness programs improve quality or reduce costs will depend on how they are designed and implemented.

Public Sector Approaches to CE Research

The Medicare Modernization Act of 2003 (MMA) gave the federal Agency for Healthcare Research and Quality (AHRQ) a limited mandate to conduct comparative effectiveness research.

The act authorized AHRQ to conduct research to determine the clinical effectiveness and appropriateness of various health services, including prescription drugs. AHRQ organizes this work under its Effective Health Care Program, which contracts with 13 Evidence-based Practice Centers (EPCs) affiliated with universities and private-sector organizations to synthesize existing knowledge.

“The specific language [of the MMA] directs us to improve the quality, effectiveness and efficiency of health care delivered through Medicare, Medicaid and SCHIP,” said AHRQ Director Carolyn Clancy at the Capitol Hill briefing. “So, our focus is on what’s known now, ensuring that programs benefit from past investments in research.”

To fill research gaps and generate new research, AHRQ also works with a separate set of research-based health care organizations with access to large electronic data bases.

Fast Facts

- ▲ Comparative effectiveness research assesses how various medical interventions stack up against each other.
- ▲ The Drug Effectiveness Review Project in Oregon is an example of a comparative effectiveness initiative that provides comparative research on the efficacy and safety of drugs.
- ▲ The U.S. Agency for Health Care Research and Quality (AHRQ) currently has a \$30 million budget to conduct clinical effectiveness research.
- ▲ The legislation that provides AHRQ authority to conduct comparative effectiveness research does not provide the agency with authority to evaluate the cost effectiveness of treatments.
- ▲ Many health plans evaluate clinical evidence to help them determine which drugs, devices and services they will cover.
- ▲ The health reform plans of Senators McCain and Obama both include provisions on comparative effectiveness research.

In April 2008 the Alliance for Health Reform, with support from the Robert Wood Johnson Foundation, held a Capitol Hill briefing on comparative effectiveness research. Panelists were Carolyn Clancy, Agency for Health Care Research and Quality; Karen Ignagni, America’s Health Insurance Plans; Wilhelmine Miller, The George Washington University; and David Nexon, Advanced Medical Technology Association. This issue brief incorporates material from that briefing.



Robert Wood Johnson Foundation

Some Placement Options for a Center for Comparative Effectiveness

Agency for Healthcare Research and Quality

This federal agency currently conducts clinical effectiveness and appropriateness research to improve health care quality for Medicare, Medicaid and SCHIP.

Institute of Medicine or a Similar Entity

The IOM is a quasi-independent agency that depends upon a mix of public and private funds.

Department of Health and Human Services

The IOM suggests that Congress direct HHS to designate an entity that would ensure production of comparative effectiveness information, overseen by a clinical effectiveness advisory board.

Significantly, there is no provision for AHRQ to determine the cost effectiveness of treatment. AHRQ's ability to conduct CE research is limited by its comparatively small budget allotted for this purpose. That budget, initially \$15 million, was doubled to \$30 million in 2008. But this is a relatively tiny amount. By comparison, the National Institutes of Health spends \$28 billion a year on research.

Another CE initiative is the Drug Effectiveness Review Project (DERP), a collaboration of public and private organizations that have joined together to provide comparative effectiveness research on the efficacy and safety of drugs. DERP's reviews are conducted by one of AHRQ's Evidence-based Practice Centers and are coordinated through the Oregon Health and Science University.

DERP members, including 15 states, participate in setting the research agenda. They prioritize classes of drugs in four categories: those that account for a large share of pharmacy budgets; those consisting of multiple drugs; those that are frequently used off-label; and those with new, very costly drugs.

Private Sector CE Efforts

There are also private sector efforts in comparative effectiveness research. The Blue Cross and Blue Shield Association's Technology Evaluation Center (TEC) has been engaged in technology assessment since 1985. TEC conducts reviews of existing clinical evidence to determine the effectiveness and appropriateness of a given procedure, drug or device.

Many health plans evaluate clinical evidence to help them determine the scope of their drug formularies (lists of drugs the health plans cover), and their coverage of devices and services (such as tests and procedures).

International Efforts

While CE may be generating new excitement in the U.S., it is more established in several other countries.

The National Institute for Health and Clinical Excellence (NICE) in Britain is perhaps the best-known example of a body that conducts CE research. Funded by the government, NICE provides guidance to the National Health Service (NHS), Britain's government-run health care system, about the coverage of new therapies (including drugs) and diagnostic services, and promotes clinical best practices. NICE serves the National Health Service in an advisory capacity; the NHS itself determines coverage. The evaluation of technology is conducted by NICE committees comprising clinicians, academics, patient advocates and industry representatives.

Australia is another leader in using CE information. Australia uses technology assessment and cost effectiveness analysis to make coverage determinations about pharmaceuticals. The Pharmaceuticals Benefits Advisory Committee (PBAC) conducts a centralized review of clinical evidence and makes a recommendation. The health minister is in charge of making the coverage determination, but cannot do so without a positive recommendation from the PBAC.

In 2003, Germany established the Institute for Quality and Efficiency. The institute is run by a private foundation, but is federally funded. The institute evaluates the quality and efficiency of drugs and health services, and reviews clinical practice guidelines for diseases. Based on the institute's report, the decision about whether a technology will be reimbursed is made by the Federal Joint Committee, the body that determines the benefit package for about 70 million people in Germany.

Governance of CE

Experts have proposed various structural arrangements for an umbrella comparative effectiveness entity in the U.S. Some would house it in an existing government agency (specifically AHRQ). Because AHRQ is already engaged in comparative assessment, it is a natural fit for those who want to build on existing infrastructure.

Other experts, however, express concern that AHRQ may be unduly influenced by stakeholders who use the political process to influence the agency's decision making.

At the Alliance/Robert Wood Johnson Foundation briefing, Karen Ignagni of America's Health Insurance Plans called for a new CE enti-

ty. The organization “needs to be an independent body insulated from the ... back and forth political discussion,” she said. “That is not to say that it should be on its own in a vacuum. It needs to have the direct participation of manufacturers, of consumers, of payers, of scientists ... the best and the brightest.”

Interest in a new entity to conduct comparative effectiveness studies has been heightened by recent, high-profile endorsements by prominent bodies such as the Institute of Medicine (IOM) and the Medicare Payment Advisory Commission (MedPAC).

In January, an IOM committee recommended that Congress establish a single national clinical effectiveness assessment program to set priorities for, fund and manage systematic reviews of existing CE research. The IOM recommends that Congress direct HHS to designate an entity that would ensure production of unbiased effectiveness information. The HHS secretary would appoint a clinical effectiveness advisory board to oversee the program. The IOM committee’s charge explicitly precluded recommendations regarding cost effectiveness and the placement of the entity.

The new entity should “provide a forum for addressing conflicting guidelines and recommendations, and [should make] an annual report to Congress,” said Wilhelmine Miller of The George Washington University at the briefing. Dr. Miller is a member of the IOM Committee on Reviewing Evidence to Identify Highly Effective Clinical Services.

MedPAC, the body that advises Congress on payment policy for Medicare, also weighed in on CE, recommending an independent entity with an independent oversight board and adequate funding to perform evidence-based research.

IOM as Model for CE Center?

MedPAC suggests that a quasi-independent organization such as the Institute of Medicine (IOM) might make a good model for the center. MedPAC recommends mandatory financing from a combination of public and private sources to provide funding and stability. MedPAC further recommends insulating the center’s funding from the annual congressional appropriations process to ensure its revenue for a longer period.

Challenges in Using CE

Comparative effectiveness research has its limitations. Studies apply only to the population that

is examined. They cannot necessarily be applied to other groups. (However, to some extent, systematic reviews of all of the clinical research on a given intervention help to overcome the limited applicability of single studies.)

Moreover, how a therapy performs under controlled conditions can vary from how well it performs in the real world, depending upon patient adherence to the study’s guidelines, the presence of other health conditions, and interactions with other medications.

The IOM committee observed that the care of individual patients must integrate clinical expertise with evidence from systematic research, and reflect the patient’s values and particular circumstances.

“Comparative effectiveness research typically looks at the impact of a treatment on an average patient within a study population,” said David Nexon of the Advanced Medical Technology Association at the Capitol Hill briefing. “It doesn’t take into account the patient differences...comorbidities, genetic [heritability], race, ethnicity and even income levels [that] can affect treatment,” Nexon said.

In addition, the availability of comparative effectiveness research does not, in and of itself, guarantee that it will be used by practitioners. Once CE research results are known, what incentives do providers have to change practice behavior? How likely are providers to change behavior in a fragmented delivery system where patients transition routinely from one clinical setting to the next?

Some critics argue that comparative effectiveness research merely takes a snapshot of available competing treatments and cannot take into account developing technologies.

Others point out that CE studies may not take into account legitimate differences in patient preference, and fear they may be used to deny coverage for safe and effective treatments.

AHRQ’s Experience

AHRQ’s experience highlights the potential and the limitations of comparative effectiveness research. Said Carolyn Clancy: “I think there’s been some concern that the ultimate outcome could be perceived by someone as a giant thumbs up or giant thumbs down on a particular service. In fact, we find that that’s rarely the case....”

“We think the reports actually...help clinicians and health care organizations refine the process of identifying more rapidly which



ALLIANCE FOR HEALTH REFORM

Acknowledgements

This publication was made possible by a grant from the Robert Wood Johnson Foundation. The Alliance is grateful for that support.

The Alliance also thanks Lisa Swirsky, the author of this paper, and Alliance intern Lindsey Cook, for her assistance.

The Alliance is a nonpartisan, not-for-profit group committed to the education of journalists, elected officials and other shapers of public opinion, helping them understand the roots of the nation's health care problems and the trade-offs posed by various proposals for change.

Design by Yael Konowe of Yael Design, Reston, Va.

Printed on recycled paper. © 2008



Alliance for Health Reform
1444 I Street, NW, Ste 910
Washington, D.C. 20005
Phone 202/789-2300
Fax 202/789-2233
www.allhealth.org

patients are most likely to benefit from particular services, so that access to effective treatments is actually maximized.”

Next Steps

The push for comparative effectiveness has gained traction among policy makers, including presidential candidates. The health reform proposals of Sen. John McCain and Sen. Barack Obama both mention comparative effectiveness as a way of reining in costs while improving quality, though neither offers details as to how.

Congress added comparative effectiveness language to the SCHIP legislation that passed both House and Senate but was vetoed by President Bush.

More recently, the Comparative Effectiveness Research Act of 2008, spon-

sored by Sens. Kent Conrad (D-N.D.) and Max Baucus (D-Mont.), would establish an institute to evaluate the effectiveness of different drugs and medical devices that can be used to treat the same medical problem.

For the moment, there appears to be genuine interest in testing whether this quality/cost strategy will deliver on its promise. With health reform on the horizon and budgetary concerns ever primary, comparative effectiveness is unlikely to fade as an issue.

For the sources used in writing this issue brief, please send an email to info@allhealth.org.

To download the webcast, transcript, podcast and resource materials from the briefing on which this paper was based, please go to www.allhealth.org/briefing_detail.asp?bi=125.

Expert Sources

- ▲ **Nancy Barrand**, Robert Wood Johnson Foundation 877/843-7953
- ▲ **Tanisha Carino**, Avalere Health 202/207-3677
- ▲ **Carolyn Clancy**, Agency for Healthcare Research and Quality 301/427-1200
- ▲ **Jill Eden**, Institute of Medicine 202/334-2191
- ▲ **Scott Gottlieb**, American Enterprise Institute 202/862-5885
- ▲ **Stuart Guterman**, The Commonwealth Fund 202/692-6735
- ▲ **Karen Ignagni**, America's Health Insurance Plans 202/778-3200
- ▲ **Mark McClellan**, The Brookings Institution 202/741-6567
- ▲ **Mark E. Miller**, MedPAC 202/220-3700
- ▲ **Wilhelmine Miller**, George Washington University 202/416-0777
- ▲ **Marilyn Moon**, American Institutes for Research 301/592-2101
- ▲ **David Nexon**, Advanced Medical Technology Association 202/783-8700
- ▲ **Peter Orszag**, Congressional Budget Office 202/226-2700
- ▲ **Sean Tunis**, Center for Medical Technology Policy 410/963-8876
- ▲ **Gail Wilensky**, Project HOPE 301/656-7401

Websites

- ▲ **Alliance for Health Reform** www.allhealth.org
- ▲ **Advanced Medical Technology Association** www.advamed.org
- ▲ **AHIP Center for Policy and Research** www.ahipresearch.org
- ▲ **AHRQ Effective Health Care Program** www.effectivehealthcare.ahrq.gov
- ▲ **Alliance for Better Health Care** www.chsr.org/abhc.htm
- ▲ **BCBS Office of Policy and Representation** www.blueadvocacy.org
- ▲ **Congressional Budget Office** www.cbo.gov
- ▲ **Consumer Reports Best Buy Drugs** www.crbestbuydrugs.org
- ▲ **ECRI Institute** www.ecri.org
- ▲ **Institute of Medicine** www.iom.edu
- ▲ **MedPAC** www.medpac.gov
- ▲ **NHS National Institute for Health and Clinical Excellence (UK)** www.nice.org.uk
- ▲ **Oregon Evidence-Based Practice Center** www.ohsu.edu/epc
- ▲ **Robert Wood Johnson Foundation** www.rwjf.org

For additional experts and websites on this and other subjects, go to www.allhealth.org.