



Comparative Effectiveness Research

Implications for Innovation in U.S. Health Care

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BACKGROUND

Proposals to expand government-funded comparative effectiveness research (CER) programs are a key element of the health care reform agenda under consideration by President Obama and the 111th Congress.

Much of the debate on CER has centered on issues of implementation and the impact on patients, providers, payers and manufacturers. There has been relatively little discussion on how CER might more broadly impact innovation across the health care system.

To address this issue, NEHI has launched an initiative to identify how CER could impact innovation in the U.S. health care system.

WHAT IS CER?

CER is the comparison of two or more health care products to determine their relative clinical effectiveness as demonstrated by patient outcomes. CER may be as simple as comparing two drugs or devices, but the concept can be expanded to include the comparison of procedures, treatments or entire care delivery systems. The purpose of CER is to fill the evidence gaps in medical decision making in order to provide higher-quality patient care. Some CER proponents would like it also to focus on the cost effectiveness or cost utility of comparative products and services (i.e. including both product and service costs along with resulting improvements in patient quality of life).

WHY NOW?

Proposals to expand CER center on achieving two goals:

- 1. Improving the 'evidence gap' in medical practice:** According to a recent Institute of Medicine panel, fewer than half of all care decisions made in the U.S. are based on 'adequate' scientific evidence. Regulatory and reimbursement policies do not subject medical products and services to a systematic comparison with reasonable alternatives.
- 2. Reducing high rates of health care spending:** The continued rise in U.S. health care spending – at rates well in excess of GDP, tax revenue and household income – has spurred interest in improving the value of dollars spent.

The debate around CER has resulted in its inclusion in the American Recovery and Reinvestment Act (ARRA) of 2009, signed into law by President Obama on February 17th. The ARRA allocates \$1.1 billion dollars for comparative effectiveness research over various federal agencies, including the Department of Health and Human Services (HHS), the Agency for Healthcare Research and Quality (AHRQ), and the National Institutes of Health (NIH). While funding levels have been set, the work is just beginning to determine the goals, scope, priorities and use of CER.

Additionally, several proposals are still on the table that relate to CER. For example, Senate Finance Committee Chairman Max Baucus (D-MT) and Senate Budget Committee Chairman Kent Conrad (D-ND) have introduced, and may reintroduce, legislation to create a private, nonprofit Health Care Comparative Effectiveness Research Institute.

The CER Debate

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MAJOR ISSUES

What will be compared? While most CER discussion concerns drugs and medical devices, procedures are also a potential target for CER, as are organizational, financial and delivery systems in health care.

Who will produce the data? Comparative effectiveness data is not a requirement of current regulatory or coverage processes of the FDA and CMS. Proposals to expand CER must stipulate how and when comparative data will be created or synthesized, who will be responsible for creating, assembling and disseminating data, and at whose cost.

What is the standard of evidence? Because findings from randomized-controlled trials (RCTs) – considered the gold standard for clinical evidence – may not reflect effectiveness as seen in real-world clinical practice, alternative forms of data and analysis may prove useful for CER.

Who sets priorities? An effective CER process will need to establish clear and equitable ground rules to set priorities on which drugs, devices or procedures will be targeted and when.

Who conducts the research? Comparative effectiveness analysis could be assigned to one organization or many, including public, private or non-governmental entities, depending upon how CER is funded and how findings will be used.

How will CER findings be translated to practice? The debate will determine whether CER will be utilized solely to improve clinical decision making or also to guide spending decisions. Effective dissemination of findings will be critical to educating physicians and patients on treatment decisions; the real test will be if and how those findings are put into practice in the clinical setting.

WHAT IMPACT WILL CER HAVE ON INNOVATION?

Critical issues pertaining to CER and innovation include:

- At what point (or points) in the lifecycle of an innovation would it be subject to CER review? Before or after coverage and reimbursement decisions? After how much market exposure?
- Will CER allow clinicians to experiment with novel or unanticipated uses of existing technologies?
- Will innovations be evaluated discretely, or in the context of the real-world care practices and delivery systems in which they are used?
- How will ongoing scientific breakthroughs, such as developments in the biological sciences, biostatistics, health information technology and analytical methods, affect CER and, in turn, innovation?

“The use of some form of comparative effectiveness research in the United States seems inevitable. We must carefully examine what its impact will be on health care innovation.”

-Wendy Everett, ScD, President, NEHI