

Comparative Clinical Effectiveness Research

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Background

The Institute of Medicine (IOM) defines comparative effectiveness research (CER) as the study of methods, including alternative approaches, to “prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care” and to inform decision-making by “consumers, clinicians, purchasers, and policy makers.”^[1] CER is an important tool for generating evidence necessary to improve the quality of health care and to assure the greatest value from the nation’s investment in the health care system. Although U.S. health expenditures as a percent of gross domestic product vastly exceed those of other wealthy nations, researchers have documented that U.S. adults and children receive only half of all recommended health care.^[2] Leading health system reform analysts have thus advocated that the U.S. follow the example found in other nations and adopt a national CER policy.^[3]

In the American Recovery and Reinvestment Act of 2009 (ARRA), Congress began to move toward the establishment of such a national policy.^[4] ARRA appropriated funding totaling \$1.1 billion for CER and created a Federal Coordinating Council (FCC) to make recommendations related to federal CER initiatives. The FCC, in its 2009 Report to the President and Congress, described the purpose of CER as being “to provide information that helps clinicians and patients choose which option best fits an individual patient’s needs and preferences.”^[5] In its report, the FCC defined CER, set forth a prioritization strategy, and made initial recommendations regarding expenditure of CER funding, including research synthesis and development of the tools essential for CER (such as clinical registries and clinical data networks).

ARRA also directed the U.S. Department of Health and Human Services (HHS) to contract with IOM to recommend national CER priorities.^[6] In its June 2009 priorities report, the IOM expanded traditional CER to include not only comparison of clinical interventions (drugs or other forms of clinical treatment), but also alternative strategies that reflect population and community interventions.^[7] The IOM also identified 100 “initial priority topics” for comparative effectiveness research.^[8] The IOM list included both clinical treatment “head to head” comparisons (for example, different types of treatments for atrial fibrillation) as well as research into the effectiveness of population health interventions (for example, regulating foods sold in schools) associated with such chronic conditions as obesity.

During the debate over ARRA, the use of evidence generated by CER was a critical issue. The Conference Committee stated that funds could be used to “conduct or support research to evaluate . . . medical treatments and services . . .” but clarified that CER could not be used to “mandate coverage or reimbursement” policy.^[9]

Changes Made by the Health Reform Law

P.L. 111- 148, §§ [6301](#) and [10602](#)

The law:

- Renames CER “comparative clinical effectiveness research (CCER)”
- Defines CCER as “research evaluating and comparing health outcomes and the clinical effectiveness, risk, and benefits of two or more medical treatments, services, and items.” Further defines “medical treatments, services, and items”^[10] to include “health care interventions, protocols for treatment, care management, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in individuals.”^[11]
- Replaces the FCC as of enactment^[12] with a Patient-Centered Outcomes Research Institute (Institute), which operates as a nonprofit corporation rather than as a governmental agency.^[13] Specifies governance by a Board of Governors consisting of the Director of the Agency for Healthcare Research and Quality (AHRQ), the Director of the National Institutes of Health (NIH), and 17 members appointed by the Comptroller General and representing patients, providers, drug and device manufacturers, health services researchers, experts in quality improvement, and federal and state government officials.^[14]
- Specifies that the Institute’s primary purpose is to advance the quality and relevance of evidence that can be used by patients, clinicians, purchasers, and policymakers to make informed health care decisions.^[15]
- Requires the Institute to consider variations in patient subpopulations and disseminate research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of medical treatments, services and items.^[16]
- Specifies the Institute’s duties as:
 - Identifying national priorities for research, taking into consideration factors such as disease incidence, prevalence and burden, gaps in evidence, potential for new evidence to improve care, potential impact on health expenditures, and relevance to patients and providers;
 - Establishing, by majority vote of the Board of Governors, and implementing, a research project agenda in accordance with approved methodological standards that include systematic reviews and assessments of existing and future research, primary research, and other methods identified by the Board;

- Assuring that research takes into account differences in the effectiveness of health care treatments, services, and items for various subpopulations, such as racial and ethnic minorities, women, age, and groups of individuals with different co-morbidities, genetic and molecular sub-types, or quality of life preferences;
 - Assuring that the research addresses different treatment modalities; and
 - Making research findings available to clinicians, patients, and the general public within 90 days after the conduct or receipt of research findings. Findings must: convey the research in a manner that is useful to patients and providers; address considerations specific to certain subpopulations, risk factors, and co-morbidities; and identify limitations of the research and what further research may be needed.
- Eliminates the bar against use of research findings to guide coverage and payment, while setting forth restrictions on the use of CER in Medicare administration, as follows:
- Specifies that CER findings should not be construed as allowing the Institute to “mandate coverage, reimbursement or other policies for any public or private payer”^[17];
 - Specifies that CER data must not include any data which would violate the privacy of research participants^[18];
 - Clarifies that nothing in the law “shall be construed as superceding or modifying the coverage of items or services under [Medicare] that the Secretary determines are reasonable and necessary or as authorizing the Secretary to deny coverage of items or services under Medicare solely on the basis of comparative clinical effectiveness research”^[19];
 - Bars the use of CER evidence in a manner that treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill^[20]; and
 - Prohibits the Secretary from using evidence or findings from CCER in determining Medicare coverage “in a manner that precludes or with the intent to discourage, an individual from choosing a health care treatment based on how the individual values the tradeoff between extending the length of their life and the risk of disability,” while specifying that this prohibition does not “limit the application of differential copayments based on factors such as cost or type of service or prevent the Secretary from using evidence or findings in determining coverage, reimbursement or incentive programs based on a comparison of the difference in the effectiveness of alternative health care treatments in extending an individual’s life due to that individual’s age, disability, or terminal illness.”^[21]
- Requires the HHS Secretary to make Medicare, Medicaid, CHIP and other data available to the Institute and authorizes the Institute to seek other public and private entities such as clinical databases and registries subject to applicable laws and standards.^[22]
- Authorizes the Institute to enter into contracts for the management of funds and the conduct of research with “appropriate” agencies and instrumentalities of the federal government and “appropriate academic research, private sector research, or study-conducting entities”^[23] that meet conditions for contracts, including the right of peer-review publication as long as a data use agreement exists with the Institute.^[24]
- Prohibits “subsequent” use of data from original research in “work-for-hire contracts with individuals, entities, or instrumentalities that have a financial interest in the results, unless approved under a data use agreement with the Institute.”^[25]
- Establishes financial and governmental oversight procedures.^[26]

- Directs the Institute to ensure “transparency, credibility, and access” through the use of public comment periods, public forums, and public availability of research findings, the process and methods used to conduct research, and the identity of the entity and the investigators conducting such research along with any conflicts of interest.^[27]
- Directs AHRQ, in consultation with NIH, to create tools for dissemination of research as well as a research data base. Further directs AHRQ to build CCER capacity by establishing a program of CCER research methods training.^[28]
- Establishes a Patient-Centered Outcomes Research Trust Fund (PCORTF) providing for directed spending to support CCER between FY 2010 and 2019.^[29] Funding is derived from general revenues, fees, and Medicare Parts A and B. Amends the Internal Revenue Code to establish the standards and procedures used to assess, collect, and transfer fees collected to the PCORTF.^[30]

Implementation

Agencies

The law does not specify which agency of the federal government has direct responsibility for establishing and incorporating the Institute.

Key Dates

None as of this writing.

Process

The health reform law does not provide specific direction regarding the administrative process used to implement the law suggesting broad authority in the Executive Branch to devise an implementation process while also assigning a role to the Comptroller General in appointing members of the Board of Governors. A wide range of tools can be used to implement the statute, such as publishing regulations in the Federal Register with a public notice and comment period, or using other types of approaches such as posted policy instructions, funding availability announcements, official letters to affected entities, and posted rulings and notices. Agency websites can be checked regularly for updates.

Key Issues

- The Institute: What will be the process, timetable, and allocation of responsibilities in the development of the Institute?
- CER Standards: What process will be used to develop the standards governing permissible and impermissible uses of CER findings and evidence?
- CER and Medicare: What standards will the HHS Secretary develop for incorporating CER findings and evidence (to the extent permissible) into Medicare program operations, including national coverage determinations, cost sharing policies, and payment for treatments and therapies?
- Dissemination Standards: What standards will the Institute and AHRQ develop for the dissemination of research results, including findings, methods, and information regarding the process by which research is conducted?
- CER and Private Research: What standards will be established for private entities that seek to conduct CER?

Recent Agency Action

No action as of the time of this posting.

Authorized Funding Levels

The PCORTF^[31] specifies funding levels by fiscal year (FY) through September 30, 2019, with a specified amount of funds made available to HHS, AHRQ, and NIH to support the dissemination of the research findings of the Institute.^[32] Funding levels are specified as follows.

- Appropriated funds in the amount of \$10,000,000 for FY 2010; \$50,000,000 for FY 2011; \$150,000,000 for FY 2012;^[33]
- For FY 2013, appropriated funds including net revenues received from fees on health insurance and self-insured plans, an amount equal to \$1 multiplied by the average number of individuals entitled to benefits under Medicare Part A or enrolled under Part B, and \$150,000,000 for each FY;^[34] and
- For FYs 2014 – 2019, appropriated funds including net revenues from fees on health insurance and self-insured plans, an amount equal to \$2 multiplied by the average number of individuals entitled to benefits under Medicare Part A or enrolled under Part B (with adjustments for increases in health care spending) and \$150,000,000.^[35]

[1] Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research," Report Brief (Washington, DC: IOM, 2009).

[2] Elizabeth A. McGlynn, Steven Asch, John Adams, Joan Keesey, Jennifer Hicks, Alison DeCristofaro, Eve A. Kerr, "The Quality of Health Care Delivered to Adults in the United States," *New England Journal of Medicine* 348, (26):2635-45, June 26, 2003.

[3] Gail R. Wilensky, "The Policies and Politics of Creating a Comparative Clinical Effectiveness Research Center," *Health Affairs* (28(4): w719-w729, July/August 2009).

[4] The American Recovery and Reinvestment Act of 2009 (ARRA), Pub. L. No. 111-5, 111th Congress, 1st sess. (2009).

[5] Federal Coordinating Council for Comparative Effectiveness Research Report to the President and Congress, available at

<http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf> June 30, 2009 (Accessed May 21, 2010).

[6] ARRA, Title VIII.

[7] Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research," Report Brief (Washington, DC: IOM, 2009), available at

<http://www.iom.edu/~media/Files/Report%20Files/2009/ComparativeEffectivenessResearchPriorities/Stand%20Alone%20List%20of%20100%20CER%20Priorities%20-%20for%20web.ashx> (Accessed May 21, 2010).

[8] Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research," Report Brief (Washington, DC: IOM, 2009), available at

<http://www.iom.edu/~media/Files/Report%20Files/2009/ComparativeEffectivenessResearchPriorities/Stand%20Alone%20List%20of%20100%20CER%20Priorities%20-%20for%20web.ashx> (Accessed May 21, 2010).

[9] U.S. House of Representatives, "Joint Explanatory Statement of the Committee of the Conference," www.house.gov/billtext/hr1_cr_jes.pdf (accessed April 30, 2010).

[10] Id. at [§ 6301\(a\)](#) adding new § 1181(a)(2)(A) to the Social Security Act.

[11] Id. at [§ 6301\(a\)](#) adding new § 1181(a)(2)(B) to the Social Security Act.

[12] Id. at [§ 6302](#)

[13] Id. at [§ 6301\(a\)](#) adding new § 1181(b)(1) to the Social Security Act.

[14] Id. at [§ 6301\(a\)](#) adding new § 1181(f) to the Social Security Act.

[15] Id. at [§ 6301\(a\)](#) adding new § 1181(c) to the Social Security Act.

[16] Id. at [§ 6301\(a\)](#) adding new § 1181(c) to the Social Security Act.

[17] Id. at [§ 6301\(a\)](#) adding new § 1181(j)(1)(A) to the Social Security Act.

[18] Id. at [§ 6301\(a\)](#) adding new § 1181(d)(8)(A)(v) to the Social Security Act.

[19] Id. at [§ 6301\(c\)](#) adding new § 1182(b) to the Social Security Act.

[20] Id. at [§ 6301\(c\)](#) adding new § 1182(c)(1) to the Social Security Act.

[21] Id. at [§ 6301\(c\)](#) adding new § 1182(d)(1) to the Social Security Act.

[22] Id. at [§ 6301\(a\)](#) adding new § 1181(d)(3) to the Social Security Act.

[23] [§ 6301\(a\)](#) adding new § 1181(d)(2)(B)(i) of the Social Security Act.

[24] [§ 6301\(a\)](#) adding § 1181(d)(2)(B)(ii) to the Social Security Act.

[25] [§ 10602\(1\)\(B\)](#), amending § 1181(d)(2)(B)(iv) of the Social Security Act.

[26] Id. at [§ 6301\(a\)](#) adding new § 1181(g) to the Social Security Act.

[27] Id. at [§ 6301\(a\)](#) adding new § 1181(h) to the Social Security Act.

[28] Id. at [§ 6301\(b\)](#) adding new § 937(a) to the Public Health Service Act.

[29] Id. at [§ 6301\(a\)](#) adding new § 1181(c) to the Social Security Act; [§ 6301\(e\)](#) adding new § 9511(a) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.

[30] Id. at [§ 6301\(e\)](#) adding new § 9511(b-f) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.

[31] Id. at [§ 6301\(e\)](#) adding new § 9511(a) to Subchapter A of chapter 98 of the Internal

Revenue Code of 1986.

[32] Id. at [§ 6301\(e\)](#) adding new § 9511(d)(2) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.

[33] Id. at [§ 6301\(e\)](#) adding new § 9511(b)(1) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.

[34] Id. at [§ 6301\(d\)](#) adding new § 1183 to the Social Security Act; [§ 6301\(e\)](#) adding new § 9511(b)(1) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.

[35] Id. at [§ 6301\(d\)](#) adding new § 1183 to the Social Security Act; [§ 6301\(e\)](#) adding new § 9511(b)(1) to Subchapter A of chapter 98 of the Internal Revenue Code of 1986.