

Comparative Effectiveness Research

The Patient Protection and Affordable Care Act (ACA) established a non-profit, tax exempt corporation, known as the “Patient-Centered Outcomes Research Institute” (PCORI) to provide comparative effectiveness information to assist patients, clinicians, purchasers, and policy makers in making informed health decisions. The PCORI Board of Governors approved on March 5, 2012, the following working definition of “patient-centered outcomes research”:

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. This research answers patient-centered questions such as:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the potential benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

PCORI is required to prioritize the healthcare areas to address, engage in research and evidence synthesis efforts, and disseminate its findings to all stakeholders in an understandable manner. In May 2012, PCORI approved a National Priorities for Research and Research Agenda. The function of the Institute is solely informational; it is specifically precluded from making mandates regarding coverage, reimbursement or other policies for any public or private payer. Nonetheless, it is expected that both private and public payers will over time use the comparative effectiveness information from this trusted source in various policy decisions.

The federal government is permitted only to use the evidence and findings from the Institute to make a Medicare coverage determination if the process is iterative (based on multiple sources), transparent, includes public comment and considers the effect on subpopulations. Furthermore, the federal government is prohibited from using this information in determining Medicare coverage, reimbursement, or incentive programs in a manner that would preclude or have the intent to discourage individuals from choosing health care treatments based on how the individual values the tradeoff between extending the length of life and the risk of disability. The enabling legislation also specifically prohibits the Institute from using cost-effectiveness analyses (e.g. quality adjusted life years (QALY) for establishing as a threshold what health care is cost-effective or recommended.

On December 18, 2012, PCORI announced its first 25 grant awards totaling more than \$40 million over three years for comparative effectiveness research. The projects approved for funding covered a wide spectrum of the healthcare system and included ways to improve care for people with such health problems as bacterial and viral infections, cardiovascular disease and stroke, certain cancers, chronic kidney disease, chronic pain, depression and other serious mental illness, and pediatric diabetes, as well cross-cutting proposals investigating how to improve care for people with multiple conditions.

How is the Institute governed?

The Institute, which was formally established in 2010, is governed by a Board of Directors consisting of the Directors of the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health (NIH) and an additional 17 members appointed by the Comptroller General representing patients and health care

consumers, physicians and providers, private payers, pharmaceutical, device, and diagnostic manufacturers or developers, representatives of quality improvement or independent health service researchers, and representatives of the federal government or the states. The selection process for the Board of Directors began in spring of 2010 with appointments finalized in September 2010. Members to the Institute's important Methodology Committee were appointed in January 2011 and Joe V. Selby, MD, M.P.H was appointed the Institute's first Executive Director in May 2011. The processes of the Institute must be transparent, include strong conflict-of-interest safeguards, and allow stakeholders and other individuals to provide informed and relevant information with respect to any determinations, to review draft proposals of the determinations and to submit public comments with respect to draft proposals.

How is the Institute funded?

The Institute is funded through federal appropriations from the Treasury of \$10 million and \$50 million for years 2010 and 2011 respectively. Beginning in 2013 and for each year after, the federal contribution from the Treasury will be \$150,000. Also beginning in 2013, the Institute will receive additional funding of \$1.00 (\$2.00 starting in 2014) from the Medicare Trust Fund for each beneficiary covered under Medicare A for the year, and a similar amount from each insured and self-insured health plan contract offered in the private sector during that year. By 2014, total funding for the Institute from all sources is estimated to be approximately \$500 million.

How will the Institute specifically affect my practice and my patients?

This Institute is expected to be a trusted source of information about which clinical strategies work best, under what circumstances and for whom for both you and your patients.

The AHRQ and National Institutes of Health currently support such comparative effectiveness efforts and publish their findings in such journals as the College's *Annals of Internal Medicine*. In addition, AHRQ, through its Effective Health Care program, currently publishes each year a limited number of guides focused on the needs of physicians and patients that synthesize available comparative effectiveness research on selected clinical issues. The guides are available at: <http://www.effectivehealthcare.ahrq.gov/index.cfm/guides-for-patients-and-consumers/>. The creation of the Institute will significantly increase the scope and volume of this much needed information. Furthermore, both AHRQ and NIH will be significantly involved in the Institute in the form of representation on the Board of Directors and priority status for research contracts offered by the Institute that fall under their purview.

How can I stay informed of the Institute's activities?

PCORI maintains a website (<http://www.pcori.org/>) that provides information regarding its activities, a list of upcoming meetings and events, and a portal through which to provide input.