

HEALTH REFORM

Patient-Centered Outcomes Research Institute: The Intersection of Science and Health Care

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The Patient Protection and Affordable Care Act created the Patient-Centered Outcomes Research Institute (PCORI), a nonprofit corporation that is neither an agency nor an establishment of the U.S. government. PCORI's mission is to support the production of well-validated scientific evidence to assist the nation in making informed decisions about a broad range of health care-related issues. In this Commentary, the directors of the Agency for Healthcare Research and Quality and the National Institutes of Health discuss PCORI's opportunities to contribute to a robust portfolio of scientific inquiry that builds on their agencies' investment in comparative effectiveness research.

SERVING THE NATION

On 3 March 1863, President Abraham Lincoln signed an act that established an independent corporation to “investigate, examine, experiment, and report upon any subject of science or art,” whenever asked to do so by the federal government (1). And so, in the midst of the Civil War, the National Academy of Sciences (NAS) was born, a distinguished body that has served our nation well. More than a century later, on 23 March 2010, President Barack Obama signed a landmark health care reform bill that, among many other things, created another nonprofit corporation—the Patient-Centered Outcomes Research Institute (PCORI)—to carry out a much more narrowly focused, but crucial, scientific mission (Table 1).

As stated in Section 6301 of the Patient Protection and Affordable Care Act (2), PCORI's purpose is “to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence” concerning ways in which a broad range of health conditions can be “prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations.” Neither an agency nor an establishment of the federal government, PCORI is also charged with “dissemination of research findings with respect to the relative health outcomes, clinical effectiveness,

and appropriateness of ... medical treatments, services, and items.” PCORI's ultimate goal is to improve patient outcomes by using the best clinical technology, techniques, and medications in existence, as determined by the best available research evidence.

IMPROVING PATIENT OUTCOMES

The Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH) embrace the establishment of PCORI, which will build on our agencies' longstanding investment in comparative effectiveness research (CER) to provide well-validated evidence-based approaches to medical care that can improve patient outcomes (3). CER is designed to inform health care decisions by providing evidence related to the effectiveness, benefits, and harms of different treatment options for a given condition, including subgroups within that condition. The evidence is generated through research that compares drugs, medical devices, tests, surgeries, or methods to deliver health care.

Historically, NIH and AHRQ have played complementary roles in this field. AHRQ specializes in systematic reviews, comprehensive meta-analyses, secondary data analyses, pragmatic clinical and health systems studies, and innovative dissemination and translation of CER findings. NIH is a recognized leader in supporting CER studies that develop primary evidence of effectiveness, including comparative clinical

Table 1. PCORI at a glance.

Topic	Provisions
Purpose	To assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by identifying and analyzing: <ul style="list-style-type: none"> - National research priorities - New clinical evidence and evidentiary gaps - Relevance of evidence and economic effects
Organization	<ul style="list-style-type: none"> - Nonprofit corporation - Not an agency or establishment of the U.S. government
Funding	<ul style="list-style-type: none"> - FYs 2010–2012: Direct appropriations of \$10 million, \$50 million, and \$150 million per year, respectively - FYs 2013–2019: Trust fund with annual inflow of \$150 million in appropriations plus annual per-capita charges per enrollee from Medicare, health insurance, and self-insured health plans - After FY 2019: No funds available from trust fund
Oversight	<ul style="list-style-type: none"> - Public/private board of governors; 19 members include AHRQ and NIH designees - Standing committee to develop and update science-based methodological standards; includes AHRQ and NIH
Research	<ul style="list-style-type: none"> - Will award contracts for peer-reviewed research - Authorized to enter into contracts with outside entities to manage funding and conduct research. Preference given to AHRQ and NIH, if research is authorized by their governing statutes
Dissemination and transparency	<ul style="list-style-type: none"> - Make research findings publicly available within 90 days - AHRQ, in consultation with NIH, will broadly disseminate research findings - Provide public comment periods on major actions - Establish publicly available resource database

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trials and support for accelerating the path toward personalized medicine.

Together, NIH and AHRQ are uniquely positioned to draw on decades of research experience to help PCORI establish its organizational structure, formulate its methodological standards, and develop research priorities and processes. These will be no small tasks, given the extensive scope and pioneering nature of this new institute, coupled with the urgent need for CER to respond to decision-makers' demands for clinical information and to adapt to the rapidly changing landscapes of biomedical technology and health care delivery. As the directors of AHRQ and NIH, we are eager to take advantage of this unprecedented opportunity to shape CER's future through our contributions as members of PCORI's Board of Governors and its Methodology Committee.

RESEARCH FOCUSED ON PATIENT OUTCOMES

Although many details of PCORI remain to be worked out, one thing is certain: AHRQ and NIH will help to ensure that this new entity lives up to its name as a research institute (4). PCORI can draw on the experience and expertise within AHRQ, NIH, and other federal research agencies to support robust, well-executed scientific research that will ultimately yield improved patient outcomes. Together, these agencies will work with PCORI's leaders to forge an institute renowned for its balanced agenda and scientific rigor.

The Institute of Medicine (IOM) recently issued a report identifying the nation's top 100 priorities for CER (5), which may serve to inform PCORI's ongoing efforts to prioritize CER needs and opportunities. Once PCORI produces its list of CER priorities, the law states that the institute will develop an agenda for initiating research projects aimed at those priorities. AHRQ and NIH will participate in the fashioning of a robust portfolio of scientific inquiry that builds on current and prior federal research and that remains focused on improving the end results for patients (6).

METHODOLOGY MATTERS

An important tool for guiding the scientific vision of PCORI-supported research will be the institute's standing Methodology Committee. The law states that this committee—which comprises no more than 15 scientific experts, including designees of the AHRQ and NIH directors, in fields ranging from

genomics to health services research—will “work to develop and improve the science and methods of comparative clinical effectiveness research” by establishing “methodological standards for research.”

Among the many areas likely to fall within the purview of the Methodology Committee are clinical outcome measures, research designs, modeling, risk-adjustment parameters, statistical protocols, quality of data and evidence, and the conduct of studies. Because CER draws on many cross-cutting fields that employ different assumptions, study methods, standards of evidence, and degrees of empirical rigor, we have before us an unprecedented opportunity to refine the precision of selecting the best methodological approach for a specific question, and to encourage continued methodological innovations. We believe, however, that it may be most useful for this committee to provide the field with general guidance, rather than taking a highly prescriptive approach.

POWER OF PEER REVIEW

Just as peer review plays a pivotal role for AHRQ and NIH, peer review must form the scientific heart of PCORI. The law states that PCORI shall ensure that there is a peer-review process for all primary research that it funds. According to the language of the statute, the review will include experts in the scientific field relevant to the research being evaluated and will be designed to avoid bias and conflicts of interest on the part of reviewers.

More precision will ultimately be needed in the peer-review plan for PCORI. For example, the current language would allow peer review to be conducted by for-profit research enterprises or by medical journals. As most researchers can attest, there is broad variability in the quality of peer-review processes among journal editorial boards, as well as among for-profit research enterprises. High-quality review processes cannot be guaranteed without the inclusion of objective criteria, appropriate infrastructure, and other essential elements of adequate peer review. To flesh out the law's current bare-bones framework for peer review, we suggest that PCORI consider making use of AHRQ's or NIH's highly regarded peer-review system (7). How to structure and implement the peer-review process, including issues pertaining to reviewers' compensation and workload, will be among the many challenges facing PCORI as it moves forward.

At present, the questions to be pursued in PCORI's peer-reviewed CER projects are extensive, with possibilities running the gamut from drugs to medical devices to prevention strategies. In the past, most CER studies have focused on the comparison of two or more interventions aimed at addressing a particular medical condition in a relevant population. However, recent advances in our understanding of the basic mechanisms of diseases and individual differences have created new opportunities for CER to identify subsets of patients within a relevant population and match them with the therapies most likely to be safe and effective for them. Moreover, both the IOM and the Federal Coordinating Council for CER concluded that CER includes “care-delivery interventions,” such as disease or care management, hospital discharge planning, and patient self-management for chronic illness. Although health services researchers have addressed these issues for decades, doing so in a comparative framework offers exciting and unexplored opportunities. Other CER challenges include addressing the needs of patients with multiple co-morbidities and incorporating new insights on multifactorial disease etiology.

FUNDING FACTS

PCORI will be funded through the Patient-Centered Outcomes Research Trust Fund, which will receive money from a variety of funding streams, the mix of which will change over the course of time. For fiscal years (FYs) 2010–2012, the trust fund will receive only direct appropriations from general federal revenues. For FYs 2013–2019, the funding will come from \$150 million in annual appropriations, along with an annual per-capita charge per enrollee from Medicare and other health insurance plans. The per-capita charge, which will start at \$1 and rise to \$2, is expected to raise approximately \$2.6 billion through FY 2019, according to the Congressional Budget Office (8). That would place PCORI's annual funding level in the realm of \$500 million or more.

As for how the money will be used, the new institute will have the authority to enter into contracts to manage funding and conduct research. Such contracts may be awarded to federal agencies, academic institutions, or the private sector. However, AHRQ and NIH will receive preference if the research to be conducted or managed under such a contract is authorized by our governing statutes.

SPREADING THE WORD

Once the results of CER studies are published by PCORI, AHRQ's Office of Communication and Knowledge Transfer, in consultation with NIH, will broadly disseminate the research findings. One way AHRQ will accomplish this is by producing informational tools for physicians, health care providers, patients, payers, and policy-makers. In addition, AHRQ will develop a publicly available database of evidence and findings from published, government-funded CER.

The importance of effective dissemination cannot be overestimated. As AHRQ's annual reports to Congress on the state of quality of care and disparities in care document in great detail, the gap between the best possible care and that which is routinely delivered remains substantial (9).

SCIENCE FOR SOCIETY

The section of the Affordable Care Act that created PCORI accounts for just 43 of the act's more than 2400 pages. However, as the slim bill that established NAS proves, legislative length is not always a good yardstick of a research organization's potential impact

on society. Just as it took a dedicated band of scientists to form what evolved into a venerated academy to advise the United States on matters of science, our country today stands in need of researchers willing to devote their time and energy to advising on issues that are absolutely vital to Americans' personal health care decisions.

The General Accountability Office is accepting Letters of Nomination from the scientific community as well as from health care providers, insurers, the biomedical industry, and health care consumers for the PCORI Board of Governors through 30 June 2010. We look forward to the research community's participation in the first of what are likely be many opportunities to serve the nation in the name of the science of health reform (10).

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