



# Perspective

## The Patient-Centered Outcomes Research Institute — Promoting Better Information, Decisions, and Health

A. Eugene Washington, M.D., and Steven H. Lipstein, M.H.A.

Within the 2000 pages of the 2010 Patient Protection and Affordable Care Act (ACA) is a short section authorizing the creation of the Patient-Centered Outcomes Research Institute (PCORI) —

a research organization dedicated to the support and promotion of comparative clinical effectiveness research. The establishment of PCORI represents the culmination of long-standing interest in comparative effectiveness research, a lengthy legislative gestation, and compromise among varied congressional perspectives and priorities.<sup>1</sup> PCORI responds to a widespread concern that, in many cases, patients and their health care providers, families, and caregivers do not have the information they need to make choices aligned with their desired health outcomes.

PCORI funding is set at a to-

tal of \$210 million for the first 3 years and increases to approximately \$350 million in 2013 and \$500 million annually from 2014 through 2019. With more than \$3 billion to spend between now and the end of the decade, PCORI will support many studies encompassing a broad range of study designs and outcomes that are relevant to patients, aiming to assist people in making choices that are consistent with their values, preferences, and goals.

By statute, PCORI is an independent, not-for-profit, private entity. Its independence and governance structure are meant to ensure that it represents the per-

spectives of the broader health care community, operates with predictable funding, and establishes itself as a credible, trusted source of information for patients, caregivers, and providers. To facilitate efficient use of the new funding, Congress directed PCORI to collaborate with existing federal agencies that engage in outcomes research, particularly the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH).

PCORI's board of governors, appointed last September by the Government Accountability Office (GAO), includes patients and health care consumers, nurses, physicians, and health services researchers, as well as representatives of integrative health care; hospitals and health systems; health plans; self-insured employ-

ers; the pharmaceutical, device, and diagnostic industries; the Veterans Health Administration; and state government. The board also includes the heads of the AHRQ and the NIH. In addition, PCORI has a methodology committee, whose 17 members were appointed by the GAO last January. This committee — which has methodologic expertise in fields including epidemiology, health services research, clinical research, biostatistics, genomics, and research methods — is charged with defining rigorous, high-integrity standards and methods to strengthen the science underpinning patient-centered outcomes research. The board has appointed as executive director Dr. Joe Selby, a family physician with expertise in clinical epidemiology and health services research and a history of leading and conducting research informed by the needs of patients and clinicians.

Since being impaneled, the board has worked to ensure transparency, credibility, and access, holding open board meetings every other month in Washington, D.C., Los Angeles, St. Louis, New York, and Seattle (meeting webcasts and summaries are available at [www.pcori.org](http://www.pcori.org)). These meetings have focused on strengthening the institute's governance, establishing an initial organizational framework, developing a consensus on PCORI's mission, and delineating processes for proposing national research priorities, establishing a research agenda, and producing a methodology report. Ample time is allotted for public comment at each meeting.

The board has adopted the following mission statement: "PCORI helps people make informed healthcare decisions, and im-

proves healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community." These sources of guidance and the involvement of stakeholders in all phases of research are not only distinguishing characteristics of PCORI's mission — they are also essential to broad acceptance of the evidence-based information the research is intended to produce. As one example of how we are pursuing our mission, PCORI board meetings include roundtable discussions with myriad stakeholders, including patients and patient advocates, clinicians, researchers, and industry representatives, to explore their perceptions of the meaning of "patient-centered outcomes research," develop a deeper understanding of outcomes valued by patients, and establish an inventory of the sources to which patients turn for health information. The forums in St. Louis and New York each drew about 100 participants. In Washington, board and methodology committee members met with patients and caregivers and fanned out in small teams across the District of Columbia to meet with 42 organizations representing nursing disciplines, medical specialties, medical technology and research fields, and patient-advocacy groups. In Seattle, the board spent an evening with representatives from health service organizations, health care providers, and comparative effectiveness researchers engaged in collaborations across the Pacific Northwest.

The institute has also launched a speakers' bureau, making gov-

ernors available to participate in national and local meetings and professional conferences with patients, clinicians, caregivers, researchers, industry representatives, health care purchasers, and policymakers. The goal of these communication and outreach activities is to promote the inclusion of all voices in the conversation about providing better information to patients and the people who care for them.

One of the principle duties of PCORI is to identify national priorities for research. In addition to engaging patients and other stakeholders, the priority-setting process will include a set of landscape reviews, pilot projects, and conference grants. The board's work is guided by four questions asked from a patient's perspective:

"Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?"

"What are my options, and what are the benefits and harms of those options?"

"What can I do to improve the outcomes that are most important to me?"

"How can the health care system improve my chances of achieving the outcomes that I prefer?"

To provide answers, the institute will conduct outcomes research assessing the benefits and harms of preventive, diagnostic, therapeutic, or delivery-system interventions, highlighting comparisons and outcomes that people care about and experience, such as survival, function, symptoms, and health-related quality of life. People's life circumstances vary, as do their biology, lifestyles, behaviors, and other important factors affecting health status. PCORI-sponsored research will

produce information tailored to diverse settings and individuals to capture the attributes of a broad range of patients.

Our landscape reviews will assess the country's current capacity for conducting patient-centered outcomes research and what is currently known about engaging patients and other stakeholders in the research process, including the dissemination of findings. PCORI's pilot projects (announced on September 28, 2011) will focus on developing, testing, and evaluating novel approaches that inform the national priorities; bring together various stakeholders; translate research into practice; identify gaps in evidence that most affect vulnerable populations; examine the prognostic information available to patients and providers; and elucidate behaviors, lifestyles, and choices within patients' control that may affect their outcomes. The PCORI conference grants are aimed at bringing together diverse perspectives to address discrete problems related to engaging patients in research and health care decisions, creating a patient-centered research agenda, and disseminating results effectively.

Concurrently, PCORI's methodology committee will review and summarize state-of-the-art

methods and standards for the conduct of patient-centered outcomes research. This work includes a detailed assessment of methods for patient engagement; establishment of research priorities; use of clinical data systems, including those covering usual practice settings; study design; and statistical analysis. Once national priorities have been drafted, vetted with the public, and approved by the board, a research agenda will be developed. We expect this agenda to deploy a full research arsenal, including randomized trials, observational outcomes studies based in registries or databases derived primarily from clinical care, and data syntheses.

By statute, 20% of the institute's funding is provided to the secretary of health and human services — most of it for the AHRQ, to support dissemination of relevant research findings to patients, providers, and other stakeholders, as well as for training and career development in comparative clinical effectiveness research. The importance of PCORI's work in the communication, dissemination, and uptake of its research findings cannot be overstated.

Most important, as we mark PCORI's first anniversary this month, the institute is poised to

lead a national movement that meaningfully involves patients and incorporates their voices in producing trusted, evidence-based information, promoting better decisions and ultimately better health for all.

Disclosure forms provided by the authors are available with the full text of this article at [NEJM.org](http://NEJM.org).

Dr. Washington is the chair, and Mr. Lipstein the vice-chair, of the board of governors of the Patient-Centered Outcomes Research Institute. Other members of the board include Debra Barksdale, Ph.D., R.N., Kerry Barnett, J.D., Lawrence Becker, Carolyn Clancy, M.D., Francis Collins, M.D., Ph.D., Leah Hole-Curry, J.D., Allen Douma, M.D., Arnold Epstein, M.D., Christine Goertz, D.C., Ph.D., Gail Hunt, Robert Jesse, M.D., Ph.D., Harlan Krumholz, M.D., Richard E. Kuntz, M.D., Sharon Levine, M.D., Freda Lewis-Hall, M.D., Grayson Norquist, M.D., M.S.P.H., Ellen Sigal, Ph.D., Harlan Weisman, M.D., and Robert Zwolak, M.D., Ph.D. Methodology Committee members include Sherine Gabriel, M.D. (chair), Sharon-Lise Normand, Ph.D. (vice-chair), Naomi Aronson, Ph.D., Ethan Basch, M.D., Alfred O. Berg, M.D., M.P.H., David Flum, M.D., M.P.H., Steven Goodman, M.D., Ph.D., Mark Helfand, M.D., M.P.H., John Ioannidis, M.D., D.Sc., Michael S. Lauer, M.D., David O. Meltzer, M.D., Ph.D., Brian S. Mittman, Ph.D., Robin Newhouse, Ph.D., R.N., Sebastian Schneeweiss, M.D., Sc.D., Jean R. Slutsky, P.A., M.S.P.H., Mary Tinetti, M.D., and Clyde Yancy, M.D.

This article (10.1056/NEJMp1109407) was published on September 28, 2011, at [NEJM.org](http://NEJM.org).

1. Wilensky GR. The policies and politics of creating a comparative clinical effectiveness research center. *Health Aff (Millwood)* 2009; 28:w719-w729.

Copyright © 2011 Massachusetts Medical Society.