The Patient-Centered Outcomes Research Institute (PCORI) National Priorities for Research and Initial Research Agenda

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The Patient Protection and Affordable Care Act of 2010 created the Patient-Centered Outcomes Research Institute (PCORI) to fund and promote comparative clinical effectiveness research (CER) that will ‘assist patients, clinicians, purchasers, and policymakers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis.’ CER is not a new concept, but appreciation of its potential for providing patients and their clinicians with uniquely valuable information on what works, tailored to the clinical situation and to patient priorities, has increased rapidly in recent years.

The research institute founded by this legislation was named to emphasize the critical importance of a patient-centered perspective in conducting this research. The PCORI Board of Governors determined early on that taking this name seriously, placing patients at the center of CER and actively engaging clinicians and other stakeholders was an ideal strategy to ensure that the PCORI research agenda stays focused on practical questions, relevant outcomes and study populations, and the possibility that treatment effects may differ across patient populations.

The institute developed a definition of patient-centered outcomes research that emphasizes the voice of the patient in assessing health care options. The PCORI mission statement commits to producing and promoting high-integrity research that is “guided by patients, caregivers, and the broader healthcare community.” The institute’s first funding announcement solicited projects focused on methods for engaging patients and other stakeholders in all aspects of the research process. Merit review of more than 800 responses to this announcement is being conducted by the Center for Scientific Review of the National Institutes of Health (NIH). PCORI has modified the process in 2 ways. First, stakeholders, including patients, caregivers, and clinicians, were invited via the PCORI website to sign up if they were interested in participating in the merit reviews. A total of 48 individuals were subsequently recruited, trained by the NIH, and included as voting members of the 16 study sections (3 per section). Second, proposed scoring criteria included an additional criterion—extent of patient engagement.

The founding legislation required PCORI to develop national priorities for research and a research agenda, and to post both for a 45- to 60-day public comment period before major funding for research could begin. The development process extended from July to December 2011. It included examination of other recent prioritization efforts and consideration of 9 criteria cited in the statute (Box). The PCORI definition of patient-centered outcomes research, and of input received through discussions with stakeholder groups, including patients and their caregivers, clinicians, hospitals and health care systems, payers, the life sciences industry, and the research community. The process produced 5 national priorities (Box) and the research agenda, which are available on the PCORI website.

The national priorities encompass many research areas cited by earlier priority-setting groups, but add a patient-centered perspective. Helping patients make informed health care choices will not be achieved solely by producing more CER evidence on prevention, diagnostic, or treatment strategies. New evidence is unlikely to be rapidly adopted unless the systems in which patients and clinicians make decisions are improved, removing current barriers to acting on the new information. Better methods for making CER results available and for communication about those results between patients and their clinicians are needed. Similar to studies evaluating health disparities, CER seeks evidence on possible differences in the effectiveness of treatments, in preferences for various outcomes, and in information needs across various populations. CER evidence can be further purposed to reduce or eliminate health disparities among vulnerable populations. Another priority addresses the need to build and sustain a national research infrastructure that facilitates learning from clinical experience. It calls for building clinical research databases, improving analytic methods for conducting this research, and training...

Statutory Criteria for PCORI-Funded Research
1. Effect on the health of individuals and populations
2. Probability of improbability through research
3. Inclusiveness of different populations
4. Current gaps in knowledge/variation in care
5. Effect on health care system performance
6. Potential to influence decision making
7. Patient-centeredness
8. Rigorous research methods
9. Efficient use of research resources

Proposed National Priorities for Research
1. Assessment of options for prevention, diagnosis, and treatment
2. Improving health care systems
3. Dissemination and communications research
4. Addressing disparities
5. Accelerating patient-centered outcomes research and methodology

*Details on both the criteria and the national priorities for PCORI-funded research may be found at http://www.pcori.org/provide-input/

The PCORI research agenda provides further specification about the kinds of studies of interest under each priority. Rather than identifying particular conditions or interventions, this agenda calls for studies across the broad range of conditions and decisions faced by patients. Proposed studies must also be aligned with the 9 criteria specified in the statute, which emphasize the burden of illness, the current level of uncertainty faced by patients and clinicians, and the potential of the research to reduce that uncertainty. The agenda calls for close coordination between PCORI and other funders of CER, including the NIH, the Agency for Healthcare Research and Quality, and the life sciences industry, to ensure that PCORI funding complements rather than duplicates research sponsored by others. It also proposes further specification of the agenda in collaboration with patients, clinicians, and the full range of the PCORI stakeholders.

Transparency is a key principle for PCORI and the lively public comment period included collection of input through an online survey, discussion with more than 190 individuals through 24 focus groups conducted throughout the United States, and a national webcast Patient and Stakeholder Dialogue midway through the comment period. Analysis of this input and resulting revisions to the priorities and agenda are under way and the initial funding announcements are expected in May 2012. Applicants will be instructed to explain how their proposed research aligns with the statutory criteria and to describe how patients and other stakeholders will be engaged in and benefit from the research. The initial portfolio of funded research is expected to cover a range of conditions and interventions, to be exemplary of stakeholder-engaged research, and to be highly aligned with the criteria.

At the same time, PCORI will begin convening multi-stakeholder conferences, workshops, and advisory panels to consider whether specific research areas within each priority may be particularly aligned with PCORI criteria and deserving of more targeted PCORI funding. Over time, it is likely that an increasing portion of PCORI funding will be directed to such high-priority research questions or areas.

The proposition that greater involvement of patients, clinicians, and others in the research process could help re-orient the clinical research enterprise, reduce clinical uncertainty, and speed adoption of meaningful findings holds great promise, but remains to be tested. PCORI will test this hypothesis. The underlying imperative is to improve patients’ care experience, decision making, and health outcomes. Patients as well as the physicians and other health care professionals who care for and about them are invited and encouraged to join in this effort.

Conflict of Interest Disclosures: All authors have completed and submitted the ICMJE Form for Disclosure of Potential Conflicts of Interest and none were reported.

REFERENCES