

VIEWPOINT

The PCORI Perspective on Patient-Centered Outcomes Research

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The Patient-Centered Outcomes Research Institute (PCORI) was established as part of the US Patient Protection and Affordable Care Act of 2010 to fund patient-centered comparative clinical effectiveness research, extending the concept of patient-centeredness from health care delivery to health care research. In the United States, patient-centered outcomes research is new and not defined in the legislation, and the rationale is unclear to many. In this Viewpoint, we address 2 related questions: What does patient-centeredness in research mean? Why conduct patient-centered outcomes research?

The essence of the PCORI definition of patient-centered outcomes research is the evaluation of questions and outcomes meaningful and important to patients and caregivers. The definition rests on the axiom that patients have unique perspectives that can change and improve the pursuit of clinical questions.

Relevant to both the definition and rationale is the hypothesis that including the perspectives of end users of the research, which include patients, physicians, and other health care stakeholders, will enhance the relevance of research to actual health decisions these end users face. In turn, increased relevance is hypothesized to improve uptake of the evidence and improve the likelihood that patients will achieve the health outcomes they desire.

Although this proposed causal chain is readily understood in relation to questions of direct interest to patients, the value of including the patient perspective for questions more distal to patient decisions, such as those related to health system design or health policy, is less clear. Also unknown is whether inclusion of the patient perspective for research earlier in the translational continuum¹ enhances the value of the information obtained from such research. PCORI considers research patient-centered if the focus includes outcomes that matter to patients, whether the study compares specific individual-level patient treatment options, for example corticosteroids vs anti-tumor necrosis factor α , or system-level care options, such as competing strategies for reducing emergency department visits for patients with chronic health conditions. Demonstrated relevance to patients is required for PCORI funding.

Active solicitation of patient views about choices of outcomes or comparators in research has been limited to date² and is one aspect of engaging patients in the research process. As a condition for funding, PCORI requires engagement with patients and other relevant health care stakeholders in all of its funded research and views engagement in research as an important component of patient-centered research. The evidence base supporting the specific benefits of active engagement

of patients in research is limited but evolving and shows both positive and negative effects. Beneficial outcomes of engagement include improved relevance of study results to patients,³ improved research recruitment and retention rates,⁴ and improved content and construct validity of measures.⁵ Negative consequences include added financial and staff resources needed to establish and maintain engagement and a sense of work burden among patient participants.⁶ PCORI intends to contribute to this evidence through evaluation of the effects—whether positive, negative, or neutral—of engaged models of research.

Why can researchers or clinicians alone not provide the patient perspective? Experiences and training determine a person's worldview,⁷ and patient-centeredness is at its heart a question of including the worldview of patients. Because of their training and orientation, researchers and clinicians can be at a disadvantage for representing the patient perspective. Patients—individuals whose worldview about health is centered on the *experience* of health care—may more accurately and comprehensively capture the patient perspective than those whose worldview centers on inquiry about health or health care. Getting the patient view directly from patients also reduces the risk of representing only those on the upside of the power asymmetry inherent in interactions between patients and researchers or patients and clinicians.

PCORI is currently funding a range of strategies for incorporating the patient perspective. No specific approach is required or preferred in patient-centered outcomes research and in the research that PCORI funds. Research teams must determine which stakeholders best represent the perspectives that can enhance the project and face the challenges of preparing stakeholders for research (eg, through preparation sessions) and ensuring that viewpoints are expressed (eg, through skilled facilitation).⁸ Traditional methods of incorporating target population perspectives such as focus groups, interviews based on robust sampling, and online forums are often appropriate, even with patients or other stakeholders on the research team. Clinicians can exert a real and valuable influence on the deliberations of patients and researchers. This influence does not invalidate the patient-centeredness of questions and may in fact enrich the questions.

To further ensure the patient-centeredness and end-user relevance of research it funds, PCORI merit review brings together scientists, patients, and other health care stakeholders as reviewers, incorporating these multiple perspectives into funding decisions. Typically, half the reviewers are scientists and half are patients or other stakeholders. In-person merit review

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meetings involve reviewers in discussion of the 5 PCORI merit review criteria: "impact of the condition on the health of individuals and populations, potential for the study to improve health care and outcomes, technical merit, patient-centeredness, and patient and stakeholder engagement." Although the first 3 criteria are standard for medical research funding, the last 2 are unusual in the United States. Scientist reviewers take the lead in discussions of technical merit. All reviewer scores are averaged, and all reviewers share responsibility for evaluating proposal quality.

Taking a patient-centered approach in both the research and the funding review processes is intended to produce research that looks beyond the questions and measures found "under the lamp post," within the researcher's field of view. For example, a proposal to study comparative effectiveness of asthma treatments for Latino youth with FEV₁ as the only outcome would not be funded by PCORI, whereas a project to study asthma treatment using FEV₁ and parental report of asthma control as outcomes was funded.

Inclusion of the patient perspective in research need not, and must not, reduce the rigor of the research. Patient input informs the design of research components that are already a standard part of studies, such as inclusion/exclusion criteria, comparators, and outcomes. The special training and skill set of researchers guide the approaches to study design, data collection, and analysis, just as the experience and perspectives of patients, clinicians, and other stakeholders help to make the research more patient-centered and relevant to health care decisions. Physician input is

needed because patient-centered outcomes research is intended to influence clinical practice. There are practical reasons to incorporate patient-centeredness in research, including improved choice of research questions and improved selection and refinement of outcomes through elicitation of stakeholder perspectives, enhanced accrual and participant retention strategies, and more appropriate dissemination and implementation strategies for findings. The promise of speeding implementation is particularly salient for PCORI, which has a legislative mandate to improve practice and reduce practice variation and disparities. PCORI proposals must include an implementation plan. Clinicians have a role in putting results into practice and should be aware of the intention of patient-centered research to enhance relevance and, ideally, trust in results by those who use them.

To help identify and encourage best practices, PCORI is actively collecting information from awardees about research engagement in their projects: what types of patients or other stakeholders are engaged, when in the research process the partners are included, and the ways in which engaged research is influencing the practice and outcomes of the research.

PCORI funds research based on the belief that incorporating the patient perspective into health care research is inherently valuable and that including the end user of research in the research process enhances usefulness and speeds the uptake of research into practice. Patient-centered outcomes research is poised to substantially change how clinical questions are asked, how answers are pursued, and how those answers are used.

ARTICLE INFORMATION

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