“Comparative Effectiveness Research” and “Patient-Centered Outcomes Research” (PCOR) are interchangeable terms that our readers will invariably encounter over the next several years. PCOR seems to have been born at the time of the randomized controlled trials (RCTs) of the 1970s and 1980s, and has matured through legislation including the Medicare Prescription Drug, Improvement and Modernization Act of 2003, which established the Effective Health Care Program at the Agency for Health Research and Quality (AHRQ); the American Recovery and Reinvestment Act of 2009 (ARRA) which allocated $1.1 billion for PCOR; and the Affordable Care Act of 2010 which created the Patient-Centered Outcomes Research Institute (PCORI). The PCORI is charged with establishing national priorities and a research project agenda to develop evidence that will help people make informed health decisions.1

So what is PCOR? The Institute of Medicine (IOM) has defined PCOR as

“The generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care. The purpose of comparative effectiveness research is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels.”

According to the PCORI, patient-centered outcomes research “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.”

One might skeptically ask what sort of research were we doing 10, 20, or even 50 years ago, if it was not related to patient outcomes? However, PCOR does differ fundamentally from what we have done previously. For instance, it is meant to directly compare active treatments, not compare treatment to observation or placebo. The emphasis is on studies involving patients, clinicians, and interventions that are typical of those in usual practice, unlike the classic RCT with its stringent inclusion criteria, performance in academic medical centers, and testing of novel interventions. Furthermore, the focus is not simply on what the physician would consider the desirable outcomes, but those that patients and policymakers would also value. The studies best suited for PCOR are large, include sufficient numbers of subjects for analysis of subpopulations, and include outcomes valuable to patients.

ARRA commissioned the IOM to set national priorities for PCOR and the IOM identified the top 100 disease- and intervention-specific topics.2 Two topics in ophthalmology appeared in the IOM’s top 100, but not until the third (51-75) quartile. They were “Compare the effectiveness of different treatment options (e.g., laser therapy, intravitreal steroids, anti-vascular endothelial growth factor) for diabetic retinopathy, macular degeneration, and retinal vein occlusion” and “Compare the effectiveness of treatment strategies for primary open-angle glaucoma (e.g., initial laser surgery, new surgical techniques, new medical treatments) particularly in minority populations to assess clinical and patient reported outcomes.”

The ophthalmology research community has rapidly responded to the challenge of PCOR (and the allure of the associated research dollars), with work on the former topic addressed by the National Institute of Health–supported Diabetic Retinopathy Clinical Research Network3 and the latter by the Registry in Glaucoma Outcomes Research (ReGOR) study, sponsored by the AHRQ, and currently being conducted by Anne Coleman, Flora Lum, and colleagues at the H. Dunbar Hoskins Jr., M.D. Center for Quality Eye Care of the Foundation of the American Academy of Ophthalmology. The Center “was established as a quality of care and health policy research center that advances the accessibility to and appropriateness of eye care services” with the goal that “The work of the Hoskins Center will help ensure that patients continue to receive high-quality, evidence-based eye care within a tightening economic environment that will demand increased value for any services provided.”

Although PCOR holds out the promise of generating important data that could lead to the provision of higher-quality care at reduced cost, it faces 2 huge hurdles. The first is that PCOR is only funded until 2019, so the Institute has little time to produce timely, practice-changing results that will build Congressional support for PCOR and hence a renewal of its funding. The leaders of the PCORI correctly point out that a first step in producing quality research is to ensure that proper research methods are used,4 but the clock is ticking as these methods are determined. Clinical research takes time, and one can envision how the performance of a 3-year trial, with a year of planning and a year of enrollment (both optimistic estimates) plus a year of data analysis, not to mention the time required for publication, already comes close to the expiration date of initial funding for the PCORI. Critics note that PCORI’s actions to date have not conveyed a sense of urgency or strategic direction and leave the issue of determining the highest-priority research questions and specific research methods entirely to the research community, to patients, and to stakeholders.1

The second hurdle facing PCOR is the problem of getting recommendations adopted and then changing physician and patient behavior. Timbie et al5 list 5 reasons that many PCOR studies have not changed practice. These include financial disincentives to change, if the change would result in less revenue; ambiguity of study results (if the results aren’t clear-cut, why change?); cognitive bias in the interpretation of new information, which include confirmation, pro-intervention, and pro-technology biases; failure of the research to address the needs of end users and limited use of decision support in making decisions.
Historically, the rapidly growth of health care costs in the United States has not produced uniformly better health outcomes, nor is it clear how much quality will be sacrificed if and when health care expenditures are reigned in. However, only through the rapid generation of results pertinent to patient-related outcomes and the demonstration that these results can be implemented can we hope to increase the value of our health care dollars. The task is daunting, but the goal is well worth the fight.

References

1. Sox H. The patient-centered outcomes research institute should focus on high-impact problems that can be solved quickly. Health Aff 2012;31:2176–82.


