Patient-Centered Outcomes Research in Radiology:

Trends in Funding and Methodology

Christoph I. Lee, MD, MSHS, Jeffrey G. Jarvik, MD, MPH

The creation of the Patient-Centered Outcomes Research Trust Fund and the Patient-Centered Outcomes Research Institute (PCORI) through the Patient Protection and Affordable Care Act of 2010 presents new opportunities for funding patient-centered comparative effectiveness research (CER) in radiology. We provide an overview of the evolution of federal funding and priorities for CER with a focus on radiology-related priority topics over the last two decades, and discuss the funding processes and methodological standards outlined by PCORI. We introduce key paradigm shifts in research methodology that will be required on the part of radiology health services researchers to obtain competitive federal grant funding in patient-centered outcomes research. These paradigm shifts include direct engagement of patients and other stakeholders at every stage of the research process, from initial conception to dissemination of results. We will also discuss the increasing use of mixed methods and novel trial designs. One of these trial designs, the pragmatic trial, has the potential to be readily applied to evaluating the effectiveness of diagnostic imaging procedures and imaging-based interventions among diverse patient populations in real-world settings.

Key Words: Patient-centered outcomes research; pragmatic trials; comparative effectiveness research.

The Patient Protection and Affordable Care Act (PPACA) of 2010 harkened a new era of health care reform with the overall goals of improving quality while decreasing costs associated with US health care. An important aspect of the new law is the requirement for tangible generalizable evidence that can clarify the relative effectiveness of different health interventions for common health conditions (1). The generation of such evidence will be led by the newly established Patient-Centered Outcomes Research Institute (PCORI) and guided by a rigorous framework for conducting comparative effectiveness research (CER) with a patient-centered approach aimed at identifying the most efficient way to deliver the right care to the right patient at the right time (2). This article provides an overview of the evolution of CER as defined and funded by the federal government, a review of PCORI's research priorities and funding opportunities relevant to radiologists, and an introduction to research methodologies that will be critical for radiologists to master and adopt to effectively compete for and obtain research funding for patient-centered outcomes research (PCOR).

Definitions of CER and PCOR

The concept of CER is not new, but its definition provided by the federal government has evolved over the last two decades. In 1996, the Agency for Health Care Policy and Research (AHCPR, now the Agency for Healthcare Research and Quality) defined CER as the determination of effectiveness of treatments for both specific medical conditions and broader system-level interventions, including cost analyses linked to health-related quality of life (3). In 2009, with the creation of the Federal Coordinating Council for Comparative Effectiveness Research (FCCCER) under the American Recovery and Reinvestment Act (ARRA), the definition for CER was broadened to include the prevention, diagnosis, and monitoring of health conditions and included a broader array of interventions beyond pharmaceuticals, such as medical devices and diagnostic testing (4). Also in 2009, the Institute of Medicine (IOM) has given a similar definition for CER 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" (4,5).

Acad Radiol 2014; 21:1156–1161

From the Department of Radiology, University of Washington School of Medicine, Seattle, Washington (C.I.L., J.G.J.); Department of Health Services, University of Washington School of Public Health, Seattle, Washington (C.I.L., J.G.J.); and Comparative Effectiveness, Cost and Outcomes Research Center, Seattle, Washington (C.I.L., J.G.J.). Received January 9, 2014; accepted January 28, 2014. Financial Disclosure: The authors declare no conflicts of interest directly related to the subject of this manuscript. J.G.J. has the following financial disclosures: He has served on the Comparative Effectiveness Advisory Board for General Electric through October 2012. He is a cofounder and stockholder of PhysioSonics, a high-intensity–focused ultrasound company and receives royalties for intellectual property. He is also a consultant for HealthHelp, a radiology benefits management company. Finally, he is a Co-Editor of Evidence-based Neuroradiology published by Springer Publishing. C.I.L. has the following financial disclosure: He serves on the clinical advisory board of Castlight Health, a health care transparency company. He has been a paid consultant to General Electric. **Address correspondence to:** C.I.L. e-mail: stophlee@uw.edu

http://dx.doi.org/10.1016/j.acra.2014.01.027

Interestingly, both the 2009 FCCCER and IOM definitions of CER did not include cost analyses. However, both did expand on the 1996 AHCPR language by referring to a broader target audience for CER beyond just policymakers to include patients, physicians, and payers. According to the IOM, CER's purpose is "to assist consumers, clinicians, purchasers, and policymakers to make informed decisions that will improve health care at both the individual and population levels." In 2010, with PPACA, the definition of CER was once again modified to include "interventions, protocols for treatment, care management, and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in, individuals" (6). Similar to the 2009 definitions, the PPACA excluded cost effectiveness as an explicit outcome (7). However, PPACA does allow PCORI to fund studies measuring downstream health care utilization and overall spending.

The major change in the definition of CER under PPACA is the intended primary audience–patients. Likely because of misperceptions of CER and it being distinguished from cost-effectiveness research, PPACA rebranded CER as PCOR (8). PCOR is intended, first and foremost, to inform patient health care decision making. Specifically, PCOR focuses on the outcomes most important to patients themselves, rather than outcomes important to other stakeholders such as payers, physicians, or researchers. These patient-centered outcomes include measures such as symptom relief, improvements in function, improvements in health-related quality of life, and survival. In addition to increasing patient centeredness of end points, another evolution in CER and PCOR under PPACA is an increasing focus on the relevance of outcomes research to direct clinical practice and the need to conduct CER in "real-world" settings (9,10).

Federal Funding of CER and PCOR

In 2009, the ARRA committed $1.1 billion to CER, including the creation of a federal coordinating council on CER, the FCCCER. The IOM and FCCCER jointly developed 100 top priority topics for spending the ARRA investment that eventually funded 400 CER projects. These efforts served as a prelude to the investment in CER promised by PPACA and the creation of PCORI and the PCOR Trust Fund. The PCOR Trust Fund included initial start-up funding of $10 million in 2010, $50 million in 2011, and $150 million in 2012. Starting in 2013 and continuing through 2019, $150 million per year will be placed into the PCOR Trust Fund from general revenue appropriations and additional funds from annual per capita Medicare and insurance plan charges (11). These funds, expected to exceed $500 million per year by 2015, will be more stable than funds originating solely from the annual congressional appropriations process. Because the overwhelming majority of the PCOR Trust Fund will fund research studies through competitive application processes governed by PCORI, they represent a large potential source of CER funding for radiology health service researchers.

PCORI is the independent nonprofit corporation established by PPACA that "helps people make informed healthcare decisions, and improves 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" (12). Governed by a 21-member board that includes representation from all major stakeholders including providers, researchers, payers, and patients, PCORI will spearhead the nation's CER efforts. In addition to the governing board, several committees provide focus for key areas of work. One of these is the PCORI Methodology Committee, which has developed methodology standards outlining the minimal requirements to follow in the conduct of PCOR (13). The alignment of radiology health services research objectives and methodologies with those of PCORI will be critical for obtaining the competitive funding.

National CER Priorities and Radiology-Specific Opportunities

Beginning with the ARRA, the IOM and FCCCER outlined national priorities for CER investigation (5). The initial list of the top 100 priority CER topics published in 2009 included the following topics directly relevant to radiology: comparison of imaging studies for diagnosis and staging of cancer; comparison of imaging modalities for breast cancer detection; comparison of screening tools for colorectal cancer; comparison of imaging modalities for acute neurologic and orthopedic conditions; and comparison of diagnostic imaging performance between radiologists and nonradiologists (5,14). PCORI expanded on these initial research priorities and drafted five national priorities after obtaining major stakeholder and public input. These broad priorities include the following: 1) assessment of prevention, diagnosis, and treatment options to see which works best for different people with a particular health problem; 2) comparing health system-level approaches to improve access and care; 3) comparing approaches to providing CER information to patients and their providers; 4) identifying and addressing disparities in prevention, diagnosis, and treatment effectiveness across patient populations; and 5) building data infrastructure and improving analytic methods for conducting PCOR. Unlike the IOM national priorities list, the PCORI list does not define specific disease conditions or interventions. Thus, conceivably, the investigation of diagnostic imaging procedures and radiology reporting can fall under all five of these broad priority areas, opening the door to larger opportunities for radiology-specific PCOR studies.

In the initial request for applications sent out in 2011, PCORI received 856 applications for 40 initial pilot project awards (15). As of September 2013, PCORI has granted 197 awards totaling more than $273.5 million to fund