

**Patient Centered Outcomes Research: Where are We Now?
Stakeholder Perspectives on PCORI Implementation to Date
October, 2013**

Introduction

Three years into the creation of the Patient-Centered Outcomes Research Institute (PCORI) under the Affordable Care Act, the Partnership to Improve Patient Care (PIPC) embarked on a mission to evaluate PCORI's progress in meeting its statutory mandate to prioritize, conduct, and communicate comparative effectiveness research (CER) in ways that are patient-centered – or to quote PCORI, identify what it means to do “research done differently”.¹ PIPC members have long-supported the creation of a new independent institute by Congress to conduct CER, and have remained engaged stakeholders throughout PCORI's existence. Although at times critical, PIPC's ultimate goal is the success of PCORI in meeting the vision of the statute that created it - to conduct truly patient-centered CER. Therefore, we recognize the tremendous effort of the PCORI Board of Governors, its Methodology Committee, and the PCORI staff to change the very culture of research by engaging patients and their providers at every step of the research process to better meet patient needs at the point of health care decision-making.

Legislative History and Intent

The Patient Protection and Affordable Care Act (ACA) created a new context for comparative clinical effectiveness research. The health reform law established a private nonprofit, tax-exempt corporation—a Patient-Centered Outcomes Research Institute—to “assist patients, clinicians, purchasers, and policy-makers 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....”² To do so, the law directs the Institute to contract with various stakeholders—including government agencies, academic institutions, or private sector research entities—to conduct patient-centered CER that will help patients, clinicians, and policy makers make well-informed decisions about health care.

While the exact structure was debated during deliberation of health reform, what is clear was that Congress wanted to change the culture of research so that patients and stakeholders would play a leading role in determining a research agenda that is most useful to them at the point of healthcare decision-making. Members on both sides of the aisle called for CER to be conducted using an open and transparent process, and to consider differences in how people respond to treatment. They sought a focus on clinical effectiveness -

¹ Selby, Joe. “Investing in Research Done Differently.” Executive Director's Corner, Patient-Centered Outcomes Research Institute, June 5, 2012. <<http://www.pcori.org/executive-directors-corner/research-done-differently/>>

² Patient Protection and Affordable Care Act, Pub.L. No. 111-148, 124 Stat. 119 (2010), amended by Health Care and Education Reconciliation Act of 2010, Pub.L. No. 111-152, 124 Stat. 1029 (2010) at § 3506.

not cost - and denounced a “one size fits all” approach for our healthcare system. The result was the creation of a research institute that preserved patient choice and promoted innovation – the two hallmarks of our healthcare system.³

Purpose of White Paper

In our evaluation, PIPC wanted a fresh perspective on how PCORI was being perceived by key stakeholders and people directly engaged in PCORI. We recognize it has been a tremendous effort to establish a new CER institute and advance a new research model. Therefore, this project is intended to provide constructive input to PCORI and other interested stakeholders in the form of an objective assessment of the most prevalent issues in PCORI’s implementation to date.

White Paper Sources and Methodology

In this project, we had the assistance of David Introcaso, Ph.D. who served as the Evaluation Officer for the Agency for Healthcare Research and Quality (AHRQ) and as a Public Health Analyst in the Office of Assistant Secretary for Planning and Evaluation (ASPE). We identified Dr. Introcaso as an expert in the field during our review of his thoughtful and insightful comments submitted to PCORI related to its first proposed national priorities for research in 2012.⁴ PIPC later engaged him to assist in the development of this White Paper by conducting key stakeholder interviews, as well as by providing his insight on PCORI’s progress.

Our methods for conducting this evaluation included several sources of information. First, Dr. Introcaso conducted fifteen phone interviews between January 16 and March 26, 2013. Interviewees included former Congressional staff members, or those involved in the drafting the ACA and relevant Institute provisions; select PCORI Board members, consultants, contractors and PCORI program participants, AHRQ staff, and interested stakeholders and individuals with a background or expertise in comparative effectiveness research. To ensure confidentiality, any findings and notes were de-identified.

Interview discussion topics focused on the prevailing issues facing PCORI. The most relevant topic of conversation involved perceptions of PCORI’s progress in meeting its patient-centered mandate, and whether it was adequately engaging patients and other stakeholders. More specifically, interviewees discussed their perceptions of PCORI’s processes for identifying research priorities, a research agenda, and other activities.

Second, PCORI’s website, including various attached reports, slide presentations, blog entries, etc., was exhaustively reviewed. Additionally, PIPC transcripts of PCORI Board of Governor’s meetings were studied. And PCORI staff provided written responses to a set of approximately 20 questions.

Lastly, our analysis drew from available literature and resources related to CER, including reports from various Congressional, AHRQ, CBO, CRS, GAO, IOM, NIH and other related sources; as well as scholarly, literature and

³. Senator Max Baucus (MT), et al. "Comparative Effectiveness Research Funds." *Congressional Record* 155: 24 (Feb. 6, 2009) p. S1796. <<http://www.gpo.gov/fdsys/pkg/CREC-2009-02-06/pdf/CREC-2009-02-06-pt1-PgS1774-5.pdf>>

⁴ Introcaso, David. “PCORI Comments on the Draft National Priorities and Research Agenda” <<http://www.pcori.org/assets/Marwood-Group-02-29-121.pdf>>

documents from various related advocacy, industry, patient and trade association websites.

What We Learned

Patient Centeredness

PCORI's patient-centered mission is a key criteria differentiating itself from existing federal agencies that conduct CER. Throughout its enabling legislation, PCORI is directed to be "patient-centered" and incorporate the patient perspective through each phase of the research process. In identifying research priorities, PCORI is mandated to take into account "patient needs, outcomes and preferences", including the requirement to factor in potential differences in various sub-populations and quality-of-life preferences. Additionally, through key advisory panels and committees, patients and providers are meant to help shape specific research designs and projects. And because patients will ultimately be the explicit audience of PCORI's research findings, the statute clarifies that PCORI's purpose is to assist patients, clinicians and others and to establish a process to solicit feedback from patients and others "about the value of the [research] information disseminated."⁵

Although PCORI has adopted a definition of the term "patient-centered outcomes research," they have not yet established how they will evaluate their work to determine the extent to which their engagement efforts have in fact produced "patient-centered outcomes research." While PCORI's public statements and stakeholder engagement activities indicate their genuine desire to be "patient-centered", more must be done to truly engage patients and providers throughout the research process.

To expand support and understanding of CER, PCORI is working diligently to partner patients and researchers through programs such as their new Challenge Grants and the upcoming Pipeline to Proposals projects.⁶ Yet, there remain some indications that PCORI is struggling with a cultural bias toward traditional researchers in the development of its research priorities, its research agenda, and its plans for dissemination. Therefore, it is important that these programs succeed in their effort to keep patients engaged at every steps of the research process, where patients and providers are expected to play a more predominant role in driving what is researched and how it is communicated.

Patient Engagement

Stakeholders, particularly patient stakeholders, often articulate the promise of PCORI to change the culture of research to better engage patients and providers, and therefore generate information that patients and providers find useful. Although PCORI has conducted a vast array of activities to engage patients and clinicians to date, it is unclear how the input PCORI is receiving is being factored into PCORI's research program.

Since its creation, PCORI has invested significant amounts of time and resources on efforts to become more patient-centered and to better engage patients in the research process. Our analysis found that PCORI has taken a variety of steps to provide opportunities for patients and other stakeholders to give input – ranging

⁵ Patient Protection and Affordable Care Act, §937(c)

⁶ PCORI Challenge Initiative <<http://www.pcori.org/funding-opportunities/challenge/>> and PCORI Pipeline to Proposals <<http://www.pcori.org/funding-opportunities/other-contract-research/pipeline-to-proposal-awards-initiative/>>

from establishing a Patient Engagement Advisory Panel,⁷ which advises the Institute on matters related to patient engagement, to making significant investments that allow for patients and other stakeholders to attend key PCORI meetings. Yet, through a review of public documents, it is difficult to assess the impact of such input on its PCORI's day-to-day operations and decision-making. Without true patient engagement, PCORI risks having patient stakeholders question the value of their input. Therefore, we are encouraged that PCORI is responding to concerns that they better train patients and other stakeholders to effectively engage in PCORI's work, particularly those serving as stakeholder reviewers.⁸

Research Methods

PCORI's enabling legislation created a Methodology Committee of 15 members who are "experts in their scientific field" tasked with improving "the science and methods for comparative clinical effectiveness research." The legislation allows the Committee to consult and contract with the IOM and others with relevant expertise, as well as with appropriate stakeholders in the conduct of their work. Within 18 months after the establishment of PCORI, the committee was directed to develop "methodological standards for research" and a "translation table that is designed to provide guidance and act as a reference for the Board to determine research methods that are most likely to address each specific research question."⁹

Our analysis found that the PCORI Methodology Committee's role has evolved significantly over the last two years. Since its creation, the PCORI Methodology Committee has undertaken significant additional contributions to PCORI's work, including the development of a definition for "patient-centered outcomes research" and developing methods for setting research priorities and incorporating the patient perspective into research. Although the Board approved a condensed version of methodological standards in November, 2012, the Methodology Committee has not yet finalized its entire report, nor has it completed a translation table. The breadth of activities undertaken by the Methodology Committee could create challenges for focusing on the areas of work required by Congress, to develop methodological standards for research and a translation table.

Research Priorities

In its establishing statute, PCORI was directed to "identify national priorities for research" and develop findings meant to improve the evidence base that clinicians and patients need to make well-informed choices. During Congressional debate, it was clear that the intent of the law was to direct PCORI, through the priority-setting process, to identify specific diseases, tests, treatments, and care strategies. However, our analysis revealed that PCORI's broadly defined research priorities and broad funding announcement process were not consistent with the intent of its statute to be more specific. This broad funding announcement process, based on the broadly defined priorities, continues to challenge PCORI in attracting both patient-centered and rigorous applications, and in creating a cohesive research agenda. A strong criticism of PCORI is that the broad funding announcement process is an investigator-initiated process for selecting a research agenda, as opposed to initiating research topics from stakeholders, especially patients and providers.

⁷ PCORI Patient Engagement Advisory Panel. <<http://www.pcori.org/get-involved/pcori-advisory-panels/advisory-panel-on-patient-engagement/>>

⁸ PCORI Required Training. <<http://www.pcori.org/funding-opportunities/funding-announcements/training/>>

⁹ Patient Protection and Affordable Care Act, §6301(d)(6)

However, PCORI has taken important steps to address concerns expressed in its early implementation that the national priorities for research and research agenda were too broadly defined. PCORI has added new processes for targeted funding announcements utilizing ad hoc workgroups and advisory panels, and has articulated its intent to shift funding over time toward targeted topics. As evidence of this change, at its September 2013 Board Meeting, PCORI laid out its plan to shift its research agenda focus by allocating more funds for targeted research that responds to specific therapeutic or health research questions. Additionally, this type of targeted funding will be supported by 55 percent of PCORI's 2014 research budget, highlighting a positive shift in PCORI's agenda-setting process to align with its statutory mandate.¹⁰

Advisory Panels

PCORI has established two tracks for developing targeted funding announcements using advisory panels as authorized by its statute. First, PCORI's Board of Governors called on their staff to develop a short-term accelerated process to select targeted topics that could be developed into targeted funding announcements using ad hoc workgroups. Additionally, PCORI also recognized the importance of a more transparent long-term process utilizing advisory panels for developing a list of targeted topics for research initiated through stakeholder engagement, as opposed to topics generated by the research community. In March 2013, the PCORI Board of Governors approved four advisory panels:

- Advisory Panel on Addressing Disparities;
- Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options;
- Advisory Panel on Improving Healthcare Systems;
- Advisory Panel on Patient Engagement.¹¹

While engaging stakeholders will better inform PCORI's research agenda, concerns remain that the breadth of expertise on the advisory panels reflecting the very broad priorities is a potential hindrance in developing the best questions for research. Because the advisory panels reflect the broad national priorities for research chosen by PCORI, panel members will not always have expertise on the specific topics under consideration for targeted funding announcements. The panels also have been criticized for including more researchers than clinicians and providers, a potential indication of the cultural bias toward researchers to identify the best research topics. Therefore, as PCORI shifts larger portions of their funding to targeted funding announcements, refinements to the advisory panel process will likely be needed to ensure appropriate expertise is available to match a more targeted research agenda.

Research Dissemination

Stakeholders place a high priority on identifying new strategies to inform patient decision-making. Although

¹⁰ PCORI's Research Portfolio: Planning and Investment Strategy, Sept. 23, 2013 <<http://www.pcori.org/assets/2013/09/PCORIs-Research-Portfolio-Planning-and-Investment-Strategy-092313.pdf>>

¹¹ PCORI Board of Governors Webinar Teleconference, March 26, 2013 <<http://www.pcori.org/assets/PCORI-Board-Meeting-Presentation-032613.pdf>>

the statute creating PCORI provides funding to the Agency for Healthcare Research and Quality (AHRQ) from the Patient-Centered Outcomes Research Trust Fund for dissemination purposes, the statute includes certain requirements for dissemination activities to be consistent with PCORI's patient-centered mission. The law requires AHRQ to create informational tools for physicians, health care providers, patients, payers and policy makers and to develop a publicly-available resource database of both government-funded evidence and research from most other sources. In this work, AHRQ is required to include a description of considerations for specific subpopulations, the limitations of the research, and to be transparent about the research methods and how the research was conducted and by whom.¹² The statute also recognized that PCORI would not immediately have research findings to disseminate, and required AHRQ to not only disseminate PCORI's research findings but also to disseminate "government-funded research relevant to comparative clinical effectiveness research..."¹³

However, it is unclear how AHRQ, with a central role in dissemination, is contributing to this effort of developing dissemination strategies that are consistent with the statute's patient-centered mandate. Although an AHRQ representative is typically present during PCORI planning discussions related to how research findings should be communicated and disseminated, it is not clear whether AHRQ' plan to adopt PCORI's ideas for disseminating, communicating and implementing research findings into practice in a manner that meets its patient-centered mandate. To date, AHRQ appears to have used funds from the Patient-Centered Outcomes Research Trust Fund to support its existing dissemination programs.¹⁴

Evaluation

Going forward, it will be important for PCORI to define what success looks like and to develop criteria for self-evaluation. PCORI describes itself, "as a learning organization, with a new and evolving process, we take our first-round experience as an opportunity to assess our approach."¹⁵ Yet, our analysis identifies challenges for PCORI to demonstrate that its patient and stakeholder engagement resulted in patient-centered outcomes research. First, PCORI lacks a consistent and credible feedback loop to demonstrate how input was processed and considered. Although at times it seems that public input is having an impact on PCORI operations, PCORI does not routinely connect the feedback received to its future decisions. Second, although PCORI has recently recognized the need for a framework to evaluate its work, particularly related to patient engagement, to date such framework does not yet exist. Therefore, it is difficult for stakeholders, or PCORI itself, to determine what activities are having a meaningful impact on the development of comparative clinical effectiveness research that is patient-centered. It is important to note that the Government Accountability Office is required to evaluate PCORI in 2015, utilizing the metrics provided to them by PCORI's statute, leaving the PCORI little time to adopt internally a rigorous method for evaluation that could influence the GAO's

¹² Patient Protection and Affordable Care Act, §9511, "Patient-Centered Outcomes Research Trust Fund," assigns 20 percent of the Institute's funding to DHHS. Of this 20 percent, AHRQ receives 80 percent and ASPE 20 percent. While funding was ramped up in fiscal years 2010 and 2011, full funding for AHRQ and ASPE began in fiscal year 2012. Beginning 2012 through 2019 AHRQ receives \$24 million each year and ASPE \$6 million.

¹³ Patient Protection and Affordable Care Act, §937(a)(1)

¹⁴ U.S. Department of Health and Human Services, Agency for Healthcare Resources and Quality, "Justification of Estimates for Appropriations Committees," Fiscal Year 2014. <<http://www.ahrq.gov/about/mission/budget/2014/2014.pdf>>

¹⁵ Email correspondence with Bill Silberg, March 18, 2013 and the Institute's blog post, "Picking Up the Pace on Our Path to Focused Research Funding" <<http://www.pcori.org/blog/picking-up-the-pace-on-our-path-to-focused-research-funding/>>

findings.¹⁶ And third, PCORI has utilized a three-year award period for comparative clinical effectiveness research, consistent with federal research agencies, which could be a barrier to demonstrating timely research findings and therefore an impact on the healthcare system before 2019 when PCORI will need to be reauthorized by Congress.

PCORI Funding

PCORI provides specific information about its funding announcements and awards on the website.¹⁷ The bulk of PCORI funding has awarded comparative clinical effectiveness research, around \$370 million in combined broad and targeted funding announcements to date. PCORI spent \$30 million on Pilot Projects. Over \$3 million has been allocated to other purposes, including data infrastructure and dissemination activities. Below is a list of funding awards that have been made publicly available by PCORI:

Patient-Centered Outcomes Research (PCOR) for Treatment Options in Uterine Fibroids: Developing a Prospective Multi-Center Practice-based Clinical Registry, 2013 (targeted funding announcement): \$20 million

Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons, 2013 (targeted funding announcement): \$30 million

National Priorities Funding (broad funding announcements), 2013: \$303 million (up to \$400 million by year end)

Treatment Options for African-Americans and Hispanics/Latinos with Uncontrolled Asthma, 2013 (targeted funding announcement): \$17 million

Improving Methods for Conducting Patient-Centered Outcomes Research (Methodology Committee): \$28 million

National Patient-Centered Clinical Research Network, 2013: \$68 million

Pilot Projects, 2012: \$30 million¹⁸

PCORI Challenge Grants, 2013: \$125,000¹⁹

Eugene Washington PCORI Engagement Awards, 2013: Contract costs not specified²⁰

PCORI Dissemination and Implementation Action Plan: Contract costs not specified²¹

¹⁶ Patient Protection and Affordable Care Act § 6301(g)(2)(A)

¹⁷ PCORI, Funding Center. <<http://www.pcori.org/funding-opportunities/funding-center/>>

¹⁸ PCORI, Pilot Projects. <<http://www.pcori.org/funding-opportunities/pfa-awards/pilot-projects/>>

¹⁹ PCORI, Challenge Initiative, <<http://www.pcori.org/funding-opportunities/challenge/>>

²⁰ Eugene Washington PCORI Engagement Awards. <<http://www.pcori.org/funding-opportunities/eugene-washington-pcori-engagement-awards/>>

²¹ PCORI, Dissemination and Implementation Action Plan. <<http://www.pcori.org/funding-opportunities/other-contract-research/dissemination-and-implementation-action-plan-2/>>

Scientific Survey Services, 2013: Contract costs not specified²²

PCOR/CER Roadmap for State Policymakers, 2013: \$291,272²³

Landscape Review- Understanding Options to Reduce Disparities in Cardiovascular Disease through Comparative Effectiveness Research, Contract costs not specified²⁴

Comprehensive PCORI Training Curriculum Development Project, \$832,573²⁵

Comprehensive Inventory of Research Networks, \$199,236²⁶

Pilot Projects Monitoring Services, \$806,002²⁷

Results Analysis Services (review of public comments), \$184,330²⁸

Survey Research Services, \$245,900²⁹

Review and Synthesis of Evidence for Eliciting the Patient's Perspective in PCOR, \$176,025³⁰

Expert Stakeholder Interviews to Identify Evidence for Eliciting the Patient's Perspective in PCOR, \$125,000³¹

Methods for Setting Priorities in Research (White Papers), \$112,000³²

Analysis of Input Received on Working Definition of "Patient-Centered Outcomes Research," \$90,797³³

²² PCORI, Scientific Survey Services. <<http://www.pcori.org/assets/PCORI-PCO-SCISRV20131.pdf>>

²³ PCORI, PCOR/CER Roadmap for State Policymakers. <<http://www.pcori.org/funding-opportunities/other-contract-research/pcorcer-roadmap-for-state-policymakers/>>

²⁴ PCORI Landscape Review. <<http://www.pcori.org/funding-opportunities/other-contract-research/landscape-review-understanding-options-to-reduce-disparities-in-cardiovascular-disease-through-comparative-effectiveness-research/>>

²⁵ PCORI, Comprehensive PCORI Training Curriculum Development Project. <<http://www.pcori.org/funding-opportunities/other-contract-research/comprehensive-pcori-training-curriculum-development-project/>>

²⁶ PCORI Comprehensive Inventory of Research Networks. <<http://www.pcori.org/funding-opportunities/other-contract-research/comprehensive-inventory-of-research-networks/>>

²⁷ PCORI, Pilot Projects Monitoring Services. <<http://www.pcori.org/funding-opportunities/other-contract-research/pilot-projects-monitoring-services/>>

²⁸ PCORI, Results Analysis Services. <<http://www.pcori.org/funding-opportunities/other-contract-research/results-analysis-services/>>

²⁹ PCORI, Survey Research Services. <<http://www.pcori.org/funding-opportunities/other-contract-research/survey-research-services/>>

³⁰ PCORI, Review and Synthesis of Evidence for Eliciting the Patient's Perspective in PCOR. <<http://www.pcori.org/funding-opportunities/other-contract-research/review-and-synthesis-of-evidence-for-eliciting-the-patients-perspective-in-pcor/>>

³¹ PCORI, Expert Stakeholder Interviews to Identify Evidence for Eliciting the Patient's Perspective in PCOR. <<http://www.pcori.org/funding-opportunities/other-contract-research/expert-stakeholder-interviews-to-identify-evidence-for-eliciting-the-patients-perspective-in-pcor/>>

³² PCORI, Methods for Setting Priorities in Research. <<http://www.pcori.org/research-we-support/methods-for-setting-priorities-in-research-white-papers-2/>>

³³ PCORI, Analysis of Input Received on Working Definition of "Patient-Centered Outcomes Research." <<http://www.pcori.org/funding-opportunities/other-contract-research/analysis-of-input-received-on-working-definition-of-patient-centered-outcomes-research/>>

Recommendations

1. Changing the culture of research will require PCORI to include stakeholders, particularly patients and providers, who are the primary end-users of comparative clinical effectiveness research in every step of the research process. Therefore, PCORI should embrace the expertise of specific patient and provider communities to identify research priorities, to be partners in research, and to develop dissemination strategies for research relevant to their needs. PCORI should also create incentives for researchers to pursue research topics that patients and providers identify as useful to their health care decision-making.
2. PCORI should establish meaningful, specific national research priorities, and “enter into contracts for the management of funding and conduct of research” per its authorizing statute, thus continuing to shift away from the investigator-initiated research model.
3. Advisory panels were authorized by Congress as “expert advisory panels” including “representatives of practicing and research clinicians, patients...” and others. Therefore, PCORI should create expert advisory panels that have a depth of expertise on the specific topic that will be developed into a targeted funding announcement to ensure that the resulting research is useful to patients and their providers.
4. PCORI’s Methodology Committee should focus its work on the activities mandated by its authorizing statute, to develop methodological standards for research and a translation table.
5. PCORI should continue its efforts to develop improved strategies for dissemination and communication of research findings, in consultation with patients, providers and other stakeholders. In addition, AHRQ should collaborate closely with PCORI to develop and adopt strategies for dissemination that meet the statute’s patient-centered mandate for disseminating PCORI research findings.
6. PCORI should adopt an evaluation model that looks critically at the impact of PCORI’s stakeholder engagement and other activities on its mission to be truly patient-centered. PCORI should also develop criteria for self-evaluation consistent with the measures for success defined by its statute for the five-year review of PCORI by the Comptroller General.