Large Pragmatic Studies to Evaluate Patient-Centered Outcomes

LOI Applicant Town Hall
August 21, 2014
Agenda

Welcome

Overview

Programmatic Requirements

Administrative Requirements

Resources

Submitting Questions:

Submit questions via the chat function in Meeting Bridge.

Ask a question via phone (an operator will standby to take your questions).
David Hickam, MD, MPH
Program Director, Clinical Effectiveness Research

Patient-Centered Outcomes Research Institute
Overview
Our Focus at PCORI

- Patient-centered
- Answering questions that matter to patients and other clinical decision makers
- Comparisons of outcomes that matter to patients
- Attention to possible heterogeneity of treatment effects
PFA Overview: Large Pragmatic Studies to Evaluate Patient-Centered Outcomes

Objective of this PFA:

- Address *critical* clinical and health-related comparative effectiveness questions faced by patients, their caregivers, and their clinicians

In this PFA we seek to fund or co-fund:

- Large pragmatic (simple) trials
- Large scale observational studies

Available Funds and Duration:

- A total of $90 million (direct and indirect) for this cycle
- Up to $10 million in total direct costs per project
- Projects should be completed within 5 years
Two Types of Applications for the Large Pragmatic Studies PFA

- Applications proposing “clinical comparative effectiveness research (CER)”
- Applications proposing “improving healthcare systems (IHS) CER”

Both types of projects are patient centered outcomes research (PCOR)

- CER model: compare 2 or more options
- Examine outcomes that are important to patients
- Assess the balance of benefits and harms
What is a Pragmatic CER Study?

- Answers a practical, real world comparative effectiveness research question.
- Assesses whether two or more options differ in effectiveness when administered as they are in real life, and it is conducted in a clinical setting that is as close as possible to a real world setting.
- The methodological approach (including study design, outcome measures, and follow-up) is as simple as possible without sacrificing scientific rigor.
Programmatic Requirements
Essential Characteristics of Studies

- Involve broadly representative patient populations in typical clinical care and community settings
- Have strong endorsement and study participation by relevant national or regional patient organizations, professional organizations, and/or payer or purchaser organizations
- Aim to
  - Address prevention, diagnosis, treatment, or management of a disease or symptom
  - Improve the performance of healthcare systems
  - Eliminate health or healthcare disparities
- Have a sample large enough to allow precise estimates of effect sizes and support evaluation of differences in treatment effectiveness in patient subgroups
- Measure health outcomes that are meaningful to the patients
Comparators of Interest

- Specific drugs, devices, and procedures
- Medical and assistive devices and technologies
- Techniques for behavioral modification
- Complementary and alternative medicine
- Delivery-system interventions
- Usual care or no specific intervention, if these are realistic choices for patients (e.g., choosing not to have a procedure for cancer screening)
Sources for Priority Topics

- PCORI priority topics (updated 8/2014; refer to the PFA)
- AHRQ Future Research Needs Projects
- IOM 100 priority topics for CER
- Investigator initiated topics will also be considered.

Researchers must make a strong case for the importance of the proposed research. Describe clearly the evidence gap that the study will fill.
PCORI Priority Topics

- Medical vs. invasive procedures for asymptomatic carotid artery stenosis
- Surgical options for hip fracture in the elderly
- Comparisons of the use of pelvic floor mesh to alternative surgical methods
- Strategies for preventing the progression of episodic acute back pain into chronic back pain
- Treatment strategies for adult patients with migraine headache
- Diagnosis and management of bipolar disorder in children and adolescents
- Management of breast ductal carcinoma in situ (DCIS)
- Multi-component interventions to reduce initiation or promote cessation of tobacco use among high-risk populations with known disparities
PCORI Priority Topics

- Treatment strategies for symptomatic osteoarthritis (OA), including joint replacement
- Treatment strategies for patients with autism spectrum disorder
- Strategies for follow-up of pulmonary nodules identified by imaging studies
- Proton beam therapy for patients with lung, breast, and prostate cancer
- Biologic agents in the management of patients with Crohn’s disease
- Benefits and harms of continuous ambulatory peritoneal dialysis compared with hemodialysis
- Treatment options for people with opioid substance abuse
- Treatment options for patients with multiple sclerosis
PCORI Priority Topics

- Active involvement by patients and caregivers in the management of **chronic mental illness**
- Integration of mental and behavioral health services into the primary care of the **general population**
- Integration of mental and behavioral health services into the primary care of **persons at risk for disparities** in health care and outcomes
- Effectiveness of innovative strategies for enhancing **patients’ adherence to medication regimens**. Studies should take into account the needs of patients with chronic conditions who are prescribed medications for short- and/or long-term indications
- **Reduction of cardiovascular disease (CVD) risk** in underserved populations such as racial and ethnic minorities and those living in rural communities
- Effectiveness of specific features of **health insurance** on access to care, use of care, and other outcomes that are especially important to patients.
What About Other Investigator Initiated Topics?

- The need for such a topic must be supported by a *critical* gap identified by a credible and recent systematic review.
- Head to head comparison of two or more options that have been shown to be efficacious, effective, or are commonly used.
- Partnership and endorsement from relevant national and/or regional professional and stakeholder organizations.
Research Activities Not Supported in the Large Pragmatic Studies PFA

- Studies of decision aids
- Efficacy trials
- Evidence syntheses
- Cost-effectiveness analyses
- Research that aims to compare the overall costs of care between two or more alternatives and use the results to determine the preferred alternative
PCORI Methodology Standards

- 47 standards in 11 groups.
- The Methodology Standards do not address all issues related to study designs and methods.
- Note that PCORI is not using a specific set of methodological standards for “pragmatic studies.”
  - Consider design tradeoffs (e.g., blinding vs not blinding)
  - Refer to other respected sources for additional guidance.
  - View report and standards here: http://www.pcori.org/research-we-support/research-methodology-standards/
“Gap analysis and systematic reviews should be used to support the need for a proposed study. If a systematic review is not available, a systematic review should be performed using accepted standards in the field (see standard SR-1), or a strong rationale should be presented for proceeding without a systematic review. In the case where a systematic review is not possible, the methods used to review the literature should be explained and justified.”

Justification for the Design Elements of a Large Pragmatic Study

- Suggest reviewing pragmatic–explanatory continuum indicator summary (PRECIS) tool
- Consider tradeoffs
  - Eligibility criteria
  - Flexibility of intervention
  - Range and types of outcomes
  - Follow up intensity
  - Adherence
  - Etc.

Patient-Centeredness vs. Patient Engagement

Patient-centeredness is about whether the project aims to answer questions or examine outcomes that matter to patients/caregivers.

Patient engagement is about having patients/caregivers as partners in research, as opposed to merely being recruited as study participants.
Addressing Engagement

Several approaches to engagement can succeed. PCORI provides many engagement resources for applicants:

- PCORI’s “The Patient and Family Engagement Rubric”

- Sample Engagement Plans

- Engagement in Research website page
  http://www.pcori.org/get-involved/what-is-engagement-in-research/

- PCORI’s Methodology Standards PC-1 to PC-4
Administrative Requirements
Budget and Period Limitations

Funds & Budget
- Direct costs up to $10 million over the life of the project
- Indirect costs: up to 40%
- Institutional base salary up to $200,000
- Indirect costs are capped on subcontracts / sub awards
- The limit for Scientific Travel is $10,000 over the duration of the project. There is no cap on Programmatic Travel.

Period of Performance
- Maximum of 5 years
- Requests to extend are not permitted during any stage
- Do not anticipate receiving a cost OR no-cost time extension.
- Propose realistic timelines accounting for the burdens associated with obtaining IRB approval from multiple sites
Costs of Interventions

- PCORI will not cover costs for clinical care alternatives that are being compared in the project.

- PCORI will consider covering costs for ancillary tasks necessary in the implementation or monitoring of a clinical intervention or strategy as part of the research program.

- Examples include costs for obtaining consent, collecting data, or monitoring that would not normally be performed in routine care.

- Support for the study by the involved healthcare delivery systems must be documented.
Guidelines for Intervention Cost/Coverage

- Costs for study interventions must be covered by delivery system, payer, manufacturer or developer of the intervention.
- The willingness of one or more of the stakeholder groups to cover treatment costs, even when one of the proposed intervention arms is not currently covered by insurance, will be taken as strong endorsement of the study by the health system or payer and of the likelihood that they will implement or use the study’s findings if definitive.
- This material support for the study by host delivery system, payer or developer should therefore be discussed in the application.
- In exceptional cases, PCORI may consider coverage of the co-payment or coinsurance costs of participating patients when that is necessary to preserve blinding in a study or to assure access to the study for vulnerable populations.
- Contact PCORI with cost questions.
Eligibility to Submit a Proposal

- Any private sector (non-profit or for-profit) research organization
- Any public sector research organization (university or college hospital or healthcare system, laboratory or manufacturer, unit of local, state, or federal government)
- Non-domestic components of organizations based in the US and foreign organizations may apply, as long as there is demonstrable benefit to the US healthcare system and US efforts in the area of patient-centered research can be clearly shown.
- Individuals are not permitted to apply.
Download the **Letter of Intent Template specifically for the Winter 2015 cycle** from the Funding Center to begin your LOI.

LOIs should not be more than 5 pages. References in the form of in-text citations are included in this limit. LOIs that exceed five pages will not be reviewed.

You must answer all questions, including the question on brief justification for the cost (“Will not exceed $10 million” is not a sufficient answer!).

Do not upload additional documents as part of your LOI.

Letters of endorsements or support are not accepted at this stage.

You must upload your LOI as a PDF in PCORI Online.
Formatting:

- Include the Principal Investigator’s (PI’s) full name on every page in the top left corner of the page header.
- Use at least half-inch margins and single spacing.
- Use size 11 Arial, Calibri, or Times New Roman for the main body of the text. Figures and captions may have smaller type.
- Each page must be numbered consecutively for each PDF upload.
- Keep the numbering of the LOI questions within the LOI template.
This is a competitive LOI process:

- An LOI is required and must be submitted prior to completion of an application. To submit an LOI, download the “Letter of Intent Template” in the Applicant Resources and complete the required fields in the PCORI Online System.

- The LOI is due on October 1, 2014, by 5:00 p.m. (ET).

- Only those LOIs deemed most responsive to this PFA will be invited to submit a full application.

- Applicants will be notified by October 31, 2014, whether or not to submit a full application.

Using the PCORI Online System

- An applicant can save information by clicking the ‘Save and Review’ button on the save and review page.
- A PI can add an Administrative Official. The PI and the AO cannot be the same individual.
- Users can now reset/change their own password.
- The AO can now send the application back to the PI.
- Please only use **Chrome or Safari browsers** to access the system.
Resources
Resources

Refer to the Pragmatic Studies page in our Funding Center (http://bit.ly/winter2015) for the following resources:

- PFA and Application Guidelines
- PCORI Online User Manuals
- Sample Engagement Plans
- PCORI Online: https://pcori.fluxx.io/
- Methodology Standards: http://www.pcori.org/research-we-support/research-methodology-standards/
## Submission and Key Dates

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<th>When</th>
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<tr>
<td>LOI-Applicant Town Hall #1</td>
<td>August 21, 2014 at 11:00pm ET</td>
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<tr>
<td>LOI due in PCORI Online</td>
<td>October 1, 2014 by 5:00pm ET</td>
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<td>Applicants notified as to whether they have been selected to submit a full application</td>
<td>October 31, 2014</td>
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<td>Applicant Town Hall #2 (if invited)</td>
<td>TBD Late 2014 or early 2015</td>
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<tr>
<td>Application Deadline (by invitation only)</td>
<td>February 3, 2015 by 5:00pm ET</td>
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<td>Merit Review Dates</td>
<td>May 7-8, 2015</td>
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<td>Awards Announced</td>
<td>July/August 2015</td>
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<td>Earliest Start Date</td>
<td>TBD 2015</td>
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Questions and Answers

Submit questions via the chat function in Meeting Bridge

Ask a question via phone (press 7)

Contact Us:
• E-mail us at pfa@pcori.org
• Schedule a call at http://bit.ly/programmatic_inquiry
• Call 202-627-1885 (one week before due date)