Cycle 1 2017: Pragmatic Clinical Studies to Evaluate Patient-Centered Outcomes

Applicant LOI Town Hall

January 26, 2017
Agenda

- Welcome
- About PCORI
- Research Goals
- Overview
- Programmatic Requirements
- Administrative Requirements
- Applicant Resources
- Questions?

Submit questions via the chat function in Meeting Bridge.

Ask a question via phone (an operator will standby to take your questions).
Today’s Presenters

Stanley IP, MD  
Associate Director  
Clinical Effectiveness and Decision Science

Jean Slutsky, PA, MSPH  
Chief Engagement and Dissemination Officer

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Administrator  
Contracts Operations
About PCORI
Pcori.org
Our Mission

PCORI helps people make informed health care decisions, and improves health care delivery and outcomes, by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers and the broader health care community.
Focus on Comparative Clinical Effectiveness Research (CER)

CER includes:

- Studies that compare health outcomes and the clinical effectiveness, risks, and benefits of two or more approaches to healthcare
  - Clinical effectiveness research
  - Improving healthcare delivery CER
  - CER to reduce/eliminate health and healthcare disparities
  - Communication and dissemination research

All applicants should:

- Explain how the research is comparative
- Name the comparators
- State why the comparisons are important to decision-makers
What is the Starting Point of Comparative Effectiveness?

• Examine the choices people make about the options for managing a disease

• Consider how compelling it is to make a choice among these options

• Consider how the need to compare these options could inform the focus of new research
  • Heterogeneity of the patient population
  • Understanding the important benefits and harms
  • Clarity about gaps in the current evidence base

• Engagement with partners facilitates these steps
What is Patient Centered Outcomes Research?

- Examines comparative effectiveness questions: comparison of options for managing a specific clinical condition
- Features collaboration involving researchers, patients, and other stakeholder partners
  - Identifying the research questions
  - Defining outcomes that are important to patients
  - Leveraging partnerships to ensure project success
- Can use various designs and approaches
  - Randomized controlled trials
  - Prospective registries
  - Other observational designs
Research We Do Not Fund

PCORI does not fund studies of cost-effectiveness analysis (CEA).

Examples of CEA

- Research that conducts a formal CEA in the form of dollar-cost per quality-adjusted life-year (including non-adjusted life-years) to compare two or more alternatives

- Research that directly compares the overall costs of care between two or more alternative approaches as the criterion for choosing the preferred alternative
Research We Do Not Fund

PCORI does not fund research whose findings will include

- development of clinical practice guidelines
- coverage recommendations
- payment
- or policy recommendations

NOTE: PCORI does fund studies that explore the burden of costs on patients—for example, out-of-pocket costs.
PFA Overview: Pragmatic Clinical 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 clinical trials that use efficient approaches
- Large 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
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.

• Project 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
• 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
The Case of Usual Care

• “Usual care” is typically a suboptimal comparator for CER studies.
• It is ill-defined, difficult to quantify, and subject to considerable geographic and temporal variations, thus limiting interpretability, applicability, and reproducibility.
• If the applicant proposes “usual care” as a rational and important comparator in the proposed study, then it must be described in detail, coherent as a clinical alternative, and properly justified as a legitimate comparator (e.g., usual care is guidelines-based).
• Additionally, it should be accompanied by an explanation of how the care given in the usual care group will be measured and how appropriate inferences will be drawn from its inclusion.
Sources for Topics of Interest

- PCORI Priority topics (updated 01/2017; refer to the PFA)
- Special Emphasis topics
- 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.
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 currently presents considerable decisional uncertainty.

• These options have been shown to be efficacious, effective, or are commonly used.

• Plans for partnership with relevant national and/or regional professional and stakeholder organizations.
Research Activities Not Supported in the Pragmatic Clinical Studies PFA

- Studies developing or conducting efficacy evaluation of decision aids
- Efficacy trials
- Evidence syntheses
- Research focusing on costs of care or cost-effectiveness
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.
PCORI Methodology Standard* RQ1 – Identifying Gaps in Evidence

• “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 Pragmatic Clinical Study

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

See:

What PCORI looks for when reviewing LOIs?

- Importance and relevance of the topics to PCORI priorities, as evidenced by critical gaps identified by clinical guidelines developers and/or recent relevant systematic review.
- Clarity and credibility of applicants’ responses to the LOI questions such as well-described comparators, clear research methods (e.g., study design, sample size, effect size)
- Programmatic fit and balance
Engagement
Patient-Centeredness vs. Patient Engagement

- **Patient-Centeredness**
  - Does the LOI mention outcomes (both benefits and harms) important to patients?
  - Are the interventions being proposed for comparison available to patients now?

- **Patient and Stakeholder engagement**
  - Does the LOI mention intent to build an interdisciplinary study team that includes appropriate patient and stakeholder representation in consultation with PCORI?
Evidence of appropriate engagement of relevant patients and other stakeholders

- Applicants are expected to consult with patients and other stakeholders on their decisional dilemma and evidence needs or to reference previously documented decisional dilemmas in preparation for the submission of LOIs.
- Identify the patients and stakeholders you consulted in determining that the proposed study addresses their evidentiary needs for decision-making and indicate your commitment to continuing to engage them actively in the conduct of the study.
Engagement Resources

– PCORI’s “The Engagement Rubric”
– Sample Engagement Plans
– Compensation Framework
– Engagement Budgeting
– Engagement in Research website page
  http://www.pcori.org/funding-opportunities/what-we-mean-engagement
– PCORI’s Methodology Standards PC-1 to PC-4
Administrative Requirements
Application Requirements: Who Can Apply?

- Any private sector research organization
  - Non-Profit Organizations
  - For Profit Organizations

- Any public sector research organization
  - Universities/Colleges
  - Hospitals or Healthcare Systems
  - Local, State, or Federal Government
  - Laboratories

- Foreign Organizations
- Nondomestic Components of Organizations based in the US

**NOTE**
PI must be an employee of the prime applicant institution. Individuals are not eligible to submit research applications to PCORI.
Competitive LOI Process

This is a competitive LOI process:

• An LOI is required and must be submitted prior to completion of an application.

• The LOI is due on February 14, 2017, 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 March 15, 2017, whether or not to submit a full application.

• Please refer to the PFA, Application Guidelines, and PCORI Online Training Resources in the Funding Center here: http://www.pcori.org/funding-opportunities/announcement/pragmatic-clinical-studies-evaluate-patient-centered-outcomes-1
Using the PCORI Online System

- Apply through PCORI Online (https://pcori.force.com/engagement)
- Access the website using Chrome or Safari browsers only
- Create a new request and begin the LOI as soon as possible
- The PI and AO cannot be the same individual
- Enter information into all required fields in the system
- PCORI Online Training Resources
Letter of Intent (LOI)

• Download the **Letter of Intent Template specifically for the Cycle 1 2017 PCS PFA** from the Funding Center to begin your LOI.

• LOIs must not be more than 3 pages excluding references. PCORI suggests including all references as in-text citations using American Medical Association citation style, but other citation styles are accepted. LOIs that exceed the page limit 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. (The header may fall within the top margin, but the body text should not begin closer than a half-inch from the edge of the page.)
- Use font size Calibri 11 for the main body of the text.
- Keep the numbering of the LOI questions within the LOI template.
# Submission and Key Dates

<table>
<thead>
<tr>
<th>What</th>
<th>When</th>
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<tbody>
<tr>
<td>LOI-Applicant Town Hall</td>
<td>January 26, 2017 at 2:30 PM ET</td>
</tr>
<tr>
<td>LOI due in PCORI Online</td>
<td>February 14, 2017 by 5:00 PM ET</td>
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<tr>
<td>Applicants notified as to whether they have been selected to submit a full application</td>
<td>March 15, 2017</td>
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<tr>
<td>Applicant Town Hall (if invited)</td>
<td>TBD</td>
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<tr>
<td>Application Deadline (by invitation only)</td>
<td>May 17, 2017 by 5:00 PM ET</td>
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<tr>
<td>Merit Review Dates</td>
<td>July 2017</td>
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<tr>
<td>Awards Announced</td>
<td>November 2017</td>
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<tr>
<td>Earliest Start Date</td>
<td>January 2018</td>
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Resources
Where can I find help?

Visit pcori.org/apply
- Application Guidelines
- FAQs
- PCORI Online User Manuals
- Sample Engagement Plans

Schedule a Call with a Program Officer
- Submit a request at pcori.org/content/research-inquiry
- Call 202-627-1884 (programmatic inquiries)
- E-mail sciencequestions@pcori.org

Contact our Helpdesk
- E-mail pfa@pcori.org
- Call 202-627-1885 (administrative and technical inquiries)
Q&A

Ask a question via the Q & A function in Meeting Bridge.

Ask a question via phone (an operator will standby to take your questions).

If we are unable to address your question during this time, e-mail the Helpdesk at pfa@pcori.org.
Thank You