Pragmatic Clinical Studies and Large Simple Trials to Evaluate Patient-Centered Outcomes

LOI Applicant Town Hall Webinar
Washington, DC
February 12, 2014 at 11:00am ET
About PCORI
Our Work Answers Patients’ Questions

Given my personal characteristics, conditions and preferences...

“What should I expect will happen to me?”

“What are my options and what are the potential benefits and harms of those options?”

“What can I do to improve the outcomes that are most important to me?”

“How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”
Our Focus

Comparative Clinical Effectiveness Research

- 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
Programmatic Requirements
PFA Overview: Pragmatic Clinical Studies and Large Simple Trials to Evaluate Patient-Centered Outcomes

Objectives of this PFA:
- Address *critical* clinical and health-related comparative effectiveness questions faced by patients, their caregivers, and their clinicians
- Test novel methodological approaches to seek answers to the above questions in real-world environments *efficiently* and in a *rigorous* manner

Available Funds and Duration:
- A total of $90 million (direct + indirect) for this cycle
- Up to $10 million in total direct costs per project
- Projects should be completed within 5 years

In this PFA we seek to fund or co-fund:
- Pragmatic clinical trials
- Large simple trials
- Large scale observational studies
Essential characteristics of funded head-to-head studies

- Involve broadly representative patient populations
- Have strong endorsement and study participation by relevant 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
- Take place within typical clinical care and community settings
- Have a sample large enough to allow precise estimates of effect sizes and support evaluation of potential differences in treatment effectiveness in patient subgroups
- Measure health outcomes that are meaningful to the patient population under study
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 topics of interest

- PCORI priority topics (updated 1/2014)
- IOM 100 priority topics for CER
- AHRQ Future Research Needs Projects

Investigator initiated topics will also be considered. Researchers must make a strong case for the importance of the topic.
PCORI Priority Topics

- Diagnosis and management of bipolar disorder in children and adolescents
- Management of breast ductal carcinoma in situ (DCIS)
- Reduction of cardiovascular disease (CVD) risk in underserved populations such as racial and ethnic minorities and those living in rural communities
- Strategies for preventing the progression of episodic acute back pain into chronic back pain
- 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.
PCORI Priority Topics

- Effectiveness of specific features of health insurance on access to care, use of care, and other outcomes that are especially important to patients.
- Treatment strategies for adult patients with migraine headache
- 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
- Treatment options for people with opioid substance abuse
- Treatment options for patients with multiple sclerosis
- Proton beam therapy for patients with lung, breast, and prostate cancer
Definitions

What is a pragmatic clinical trial?
- A trial that takes place in a real world clinical population and setting, and aims to collect information to help make treatment decisions.

What is an explanatory trial?
- A trial that is designed to increase the probability of obtaining maximal effect from an intervention of interest in an experimental population and setting. The results will show whether the intervention can work (efficacy) but not whether it will work in everyday clinical practice (effectiveness).
Domains of the Pragmatic–Explanatory Continuum Indicator Summary (PRECIS)*

- Participant eligibility criteria
- Flexibility of intervention
- Practitioner expertise needed in applying the experimental intervention
- Flexibility of comparator intervention
- Practitioner expertise needed in applying the comparator intervention
- Follow-up intensity
- Nature of the primary outcome
- Participant compliance of intervention
- Practitioner compliance with protocol
- Type of primary outcome analysis

*Thorpe et al. CMAJ. 2009:180:E47-E57
What about other investigator initiated topics?

The need for such a topic must be supported by a critical gap identified by a credible systematic review.

Partnership and endorsement from relevant professional and stakeholder organizations
“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.”

Funding Exclusions

- Explanatory (efficacy) trials
- Cost-effectiveness analyses
- Direct comparisons of costs of care between the two or more alternatives
Administrative Requirements
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.
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 has been increased to $10,000 over the duration of the project.

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.
Letter of Intent (LOI)

This is a competitive LOI process:

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

- All applicants will be notified of their status by April 7, 2014.

- Download the **Technical Abstract Template** from the Funding Center to begin your LOI.
  - LOIs should not be more than 5 pages.
  - Adhere to the formatting and template requirements in the Application Guidelines.
  - You must upload your LOI as a PDF in PCORI Online.

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<th>What</th>
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<tr>
<td>LOI-Applicant Town Hall #1</td>
<td>February 12, 2014 at 11:00am ET</td>
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<td>LOI due in PCORI Online</td>
<td>March 7, 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>April 7, 2014</td>
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<td>Applicant Town Hall #2</td>
<td>TBD Summer 2014</td>
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<td>Application Deadline (by invitation only)</td>
<td>August 8, 2014 by 5:00pm ET</td>
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<td>Merit Review Dates</td>
<td>November 2014</td>
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<td>Awards Announced</td>
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<td>Earliest Start Date</td>
<td>April 2015</td>
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Using the PCORI Online System

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

Visit the Funding Center:
- Application Guidelines
- FAQs
- PCORI Online User Manuals
- Sample Engagement Plan

Schedule a call with a Program Officer:
- Call 202-627-1884
- Visit http://www.pcori.org/about-us/contact-us/

Contact our Helpdesk:
- E-mail pfa@pcori.org
Questions

Please use this time to ask any programmatic and administrative questions you may have about the PFA or LOI submission process.

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

Submitting Questions:
Submit questions via the chat function in Meeting Bridge.

Ask a question via phone (press 7 on your phone).