Optimized Multidisciplinary Treatment Programs for Nonspecific Chronic Low Back Pain – Cycle 1 2017

LOI Applicant Town Hall
January 25, 2017
Agenda

Overview

Programmatic Requirements

Engagement Requirements

Administrative Requirements

Merit Review Process

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

Ask a question via phone (an operator will standby to take your questions).
Overview
Objective of this PCORI Funding Announcement (PFA):

- Address *critical* clinical and healthcare delivery choices faced by patients, their caregivers, clinicians, or delivery systems.

In this PFA we seek to fund:

- Randomized clinical trials
- Well-justified observational studies

Available Funds and Duration:

- A total of $50 million (direct and indirect)
- Up to $10 million in total direct costs
- Projects should be completed within 5 years
What is a CER Trial?

- Answers a practical, real-world comparative effectiveness research question
- Assesses whether two or more options differ in effectiveness when administered as they would be in real life, and the 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
What is the comparative clinical effectiveness of optimized, multidisciplinary nonsurgical treatment programs involving combined or sequenced interventions for patients with nonspecific chronic LBP?
Priority Research Question PICOTS

- **Population:** Adults with chronic nonspecific LBP on ≥ 50% of days in the past 6 months despite current treatment
- **Intervention:** A structured, optimized, multidisciplinary program of nonsurgical treatments used in combinations or sequences.
- **Comparator:** An evidence-based care program that is meaningfully different from the primary intervention and meets the same requirements.
- **Outcome:** NIH LBP Task Force outcome measures; healthcare utilization; safety; quality of life; validated general and low back pain-specific disability measures
- **Time:** 5-year study period, including 12-month follow-up
- **Setting:** Where patients with LBP typically receive care.
Responsive Applications

- Investigators must address the priority research question.
  - Other investigator-initiated projects will **not** be considered responsive to this PFA

- Applicants should provide a convincing empirical and/or clinical rationale for the multidisciplinary interventions being compared.
  - Each treatment package being compared should include two or more component interventions which are documented to be efficacious or in common use.

- Heterogeneity of Treatment Effects (HTEs) should be examined (e.g., overweight/obesity, psychosocial stressors, mental health conditions, older adults, or other clinical characteristics with an accompanying strong rationale.)
Justification for the Design Elements of Proposed Studies

- 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.
Research Activities Not Supported by this PFA

- Pilot studies
- Efficacy trials
- Cost-effectiveness analyses
- Direct comparisons of the costs of care between two or more alternative approaches
- Evaluation of new or existing decision-support tools
- Studies of the natural history of disease, instrument development, pharmacodynamics, and fundamental science of biological mechanisms
- Evaluation of new or existing decision-support tools
- Development of clinical prediction or prognostication tools
Methodology Standards

In any study, methods are critical. PCORI’s Methodology Committee developed Methodology Standards to which patient-centered CER must adhere.

Methodology Standards: 11 Specific Categories

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects
- Data Networks
- Data Registries
- Adaptive and Bayesian Trial Designs
- Causal Inference
- Studies of Diagnostic Tests
- Systematic Reviews
LOI Requirements

• Four page limit – See requirements for font size and type, margins, and line spacing.
  – LOIs that exceed four pages will not be reviewed.
• All references should be included as in – text citations.
• In the LOI, provide a realistic estimate of the study’s budget.
• Only LOIs deemed most responsive (programmatically and administratively) will be invited to submit an application.
Changes from LOI to Application

• If invited to submit a full application, please contact PCORI (pfa@pcori.org) if you wish to make any significant changes to the proposed study including the following:
  – PI
  – Institution
  – Study design
  – Budget or period of performance
  – Research question
  – Aims
  – Comparators

• PCORI must approve any changes before the full application is submitted
Preparing Your Application

• To begin, all applicants should:
  • Thoroughly read the funding announcement and review the PCORI Application Guidelines Document
  • Review the PCORI Research Plan Template
  • Have a copy of your approved LOI readily accessible
  • Carefully consider the feedback you received on your LOI
Milestones/Deliverables Template

Milestones: Significant events or accomplishments within the project; may have deliverables associated with them

Deliverables: Measurable and verifiable outcomes or objects that a project team must create and deliver according to the contract terms
Research Strategy: Overview

- Maximum 20 pages in length
- Use the Research Plan Template as your guide
  - Background
  - Significance
  - Patient Population
  - Recruitment Plan
  - Estimated Racial/Ethnic and Gender Enrollment Table
  - Study Design or Approach
  - Engagement Plan
  - Research Team and Environment
- PLEASE provide all the information requested, as outlined in the template.
Research Strategy: Recruitment Plan

- Discuss past experiences with recruitment of the target population
- Provide preliminary evidence of the potential for successful recruitment
- Provide numbers for the pool of potential participants, those estimated to be eligible, and the expected participation rate
- Discuss barriers to recruitment and how you plan to overcome them

Strategies for successful recruitment
  - Engaged clinical sites
  - Clinical advocates
  - Proactive, experienced research coordinator
  - Alignment and integration of recruitment activities with clinical workflow
PCORI applications may include an appendix for additional materials the investigators think may be useful

- Reviewers will not be required to include the appendices in the review and assessment of the project

Examples of additional materials are:

- Survey instruments
- Papers and publications
People and Places Template: Biosketch

- You may use the NIH biosketch or PCORI’s format
- Biosketches are required for all key personnel
- List all partners within the Key Personnel section
- Patient/Stakeholder Biosketch

Page Limit
5 Per person
People and Places Template: Project / Performance Site(s)

- Demonstrate that the proposed facilities have the appropriate resources required to conduct the project to plan, within budget, and on time.
- Provide a description of the facilities that will be used during the project, including capacity, capability, characteristics, proximity, and availability to the project.
Letters of Support

• Save all letters of support as a single PDF file prior to uploading to the PCORI Online System.

• All letters of support should be addressed to the PI and demonstrate the commitment of key personnel and supporting organizations to your proposed project.

• Letters of support should clearly reflect the substantive involvement and material contribution to be provided by the signatory parties, and are meant to substantiate the commitment of collaboration of all forms.
Engagement Requirements
Patient-Centeredness vs. Patient Engagement

• Patient-Centeredness
  – Research questions and outcomes reflect what is important to patients and caregivers and consider patient preferences.

• Patient Engagement
  – Project includes active engagement among scientists, patients, and stakeholders.
  – Project includes community, patient, and caregiver involvement.
Addressing Engagement

- Applicants should consult with patients and other stakeholders on their decisional dilemma and evidence needs or reference previously documented decisional dilemmas in preparation for the submission of LOIs and applications.
- State the specific clinical decision(s) and/or treatment choice(s) confronted by the decision makers, and explain how the findings from the proposed research will inform those decisions.
- State why this decision, such as choosing between specific treatment strategies, is important to patients and their caregivers.
- Document the uncertainty faced by patients, clinicians, and other decision makers in making this decision.
- Identify the stakeholders you consulted in determining that the proposed study addresses their evidentiary needs for decision making.
- Indicate your commitment to continuing to engage them actively in the conduct of the study.
Addressing Engagement

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

- Engagement in Research website page: [http://www.pcori.org/funding-opportunities/what-we-mean-engagement](http://www.pcori.org/funding-opportunities/what-we-mean-engagement)
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.
Using the PCORI Online System

- Begin the LOI as soon as possible.
- Navigate to PCORI Online [https://pcori.force.com/engagement](https://pcori.force.com/engagement)
- Log into the PCORI system early to address additional LOI questions.
- Please only use **Chrome, Safari browsers and Firefox** to access the system.
- The PI and the AO cannot be the same individual.
- [PCORI Online Training Resources](#)

![Browsers Logos]
Budget Information
Budget Templates: Overview

Three budget sections must be submitted as part of the online application process:

- Detailed Budget
- Budget Summary
- Budget Justification

NOTE: A detailed budget is needed for each year of the program. Complete each budget section for the prime applicant and any/each subcontractor.
Budget Justification

- Narrative that fully supports and explains the basis for the information in the Budget Detail
  - Provide sufficient detail to understand the basis for costs, the reason that the costs are necessary, and an explanation for major cost variances
  - Use the budget template to tell PCORI why the costs are reasonable for the work to be performed

- Breakdown of costs proposed for each consortia or contractor

- Must specify any other sources of funding that are anticipated to support the proposed research project

- **Provide quotes, indirect cost rate letter, fringe benefit policy**
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.
What happens to your application after you submit it?
Programmatic Screening

- Study deviates from approved LOI
- Study includes cost-effectiveness analysis
- Study is not responsive to PFA and/or does NOT address the PFA’s “Research Areas of Interest”
- Study is not comparative
Merit Review
Building an Inclusive Merit Review

• Panels include 3 reviewer types to bring diverse perspectives to the merit review process.

• Each application is reviewed by 3 scientists, 1 patient, and 1 other stakeholder.

• The panel chair facilitates discussion and promotes a culture of mutual respect and understanding among reviewer types.
Reviewers evaluate the strengths and weaknesses of an application based on the following six criteria:

<table>
<thead>
<tr>
<th>Crosswalk of PCORI Merit Review Criteria with NIH Criteria</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>SIGNIFICANCE</strong></td>
</tr>
<tr>
<td>1. Potential for the study to fill critical gaps in evidence</td>
</tr>
<tr>
<td>2. Potential for the study findings to be adopted into clinical practice and improve delivery of care</td>
</tr>
<tr>
<td><strong>APPROACH</strong></td>
</tr>
<tr>
<td>3. Scientific merit (research design, analysis, and outcomes)</td>
</tr>
<tr>
<td>4. Investigator(s) and environment (NEW)</td>
</tr>
<tr>
<td><strong>PCORI-only Merit Review Criteria</strong></td>
</tr>
<tr>
<td><strong>PATIENT-CENTEREDNESS/ENGAGEMENT</strong></td>
</tr>
<tr>
<td>5. Patient-centeredness</td>
</tr>
<tr>
<td>6. Patient and stakeholder engagement</td>
</tr>
</tbody>
</table>
Multi-phase process for review

- Preliminary (online) review
- In-Person review
- Post-Panel review (PCORI program staff)
### Submission and Key Dates

<table>
<thead>
<tr>
<th>What</th>
<th>When</th>
</tr>
</thead>
<tbody>
<tr>
<td>LOI-Applicant Town Hall</td>
<td>January 25, 2017 at 11:00am ET</td>
</tr>
<tr>
<td>LOI due in PCORI Online</td>
<td>February 14, 2017 by 5:00pm ET</td>
</tr>
<tr>
<td>Applicants notified as to whether they have been selected to submit a full application</td>
<td>March 15, 2017 by 5:00pm ET</td>
</tr>
<tr>
<td>Application Deadline (by invitation only)</td>
<td>May 17, 2017 by 5:00pm ET</td>
</tr>
<tr>
<td>Merit Review Dates</td>
<td>July 2017</td>
</tr>
<tr>
<td>Awards Announced</td>
<td>November 2017</td>
</tr>
<tr>
<td>Earliest Start Date</td>
<td>January 2018</td>
</tr>
</tbody>
</table>
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)
Questions and Answers

Submit questions via the chat function in Meeting Bridge

Ask a question via phone (press 7)

Contact Us:
• Schedule a call at http://bit.ly/programmatic_inquiry
• Call 202-627-1884 (programmatic inquiries)
• E-mail us at sciencequestions@pcori.org
• Call 202-627-1885 (administrative and technical inquiries)
• E-mail us at pfa@pcori.org