Cycle 3 2017: Broad PCORI Funding Announcements (PFAs)

Applicant Town Hall
October 19, 2017
Agenda

- Programmatic Overview
- Administrative Overview
- Merit Review Criteria
- Questions and Answers

Submit questions via the Question box in GoToWebinar

Ask a question via phone at the end of the presentation
About PCORI
pcori.org
Why PCORI?

• For all the advances it produces, research still has not answered many questions patients face.
• People want to know which treatment is best for them.
• Patients and their clinicians need information they can understand and use.
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.
Broad PFAs: Research Goals
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?”
What does PCORI look for?

- Patient Centered
- Comparative
- Scientific Rigor
- Immediately Impactful
- Burden on the U.S.
- Chronic or Multiple Chronic Conditions
- Cross Cutting Research
- Rare and Understudied Conditions
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
  – Communications and dissemination CER
  – CER to reduce/eliminate health and healthcare disparities

• All applicants should:
  – Explain how the research is comparative
  – Name the comparators
  – State why the comparisons are important
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.
Broad PFAs: Overview and Research Areas of Interest
Assessment of Prevention, Diagnosis, and Treatment Options

• Comparative studies in prevention, screening, diagnosis, treatment, and long-term management that have not been adequately addressed previously.

• Consideration of treatment effect modifiers, including demographic, biological, clinical, socioeconomic and other factors, within proposed studies.

• Comparative studies addressing rare diseases.

Available Funds: Up to $32 million
Total Direct Cost: $2 million
Maximum Project Period: 3 years
Research Areas of Interest

• High priority topics as identified by systematic reviews, evidence of gaps in clinical guidelines, or other credible evidence reviews.
• Comparisons of the effectiveness of two or more strategies for prevention, treatment, screening, diagnosis, or management that are known to be efficacious but have not been adequately compared in previous studies.
• Areas deemed non-responsive to the PFA:
  – Studies to establish efficacy for a new clinical strategy
  – Pilot studies intended to inform larger efforts
  – Studies to develop decision aids
  – Studies of the natural history of disease
  – Studies comparing the roles of community health workers or patient navigators
Studies compare alternative features of healthcare systems that optimize quality, outcomes, and efficiency of patient care.

Major categories of innovation:

- Information technology (IT) or other electronic technologies
- Organizational structures and policies (such as standing orders)
- Incentives for patients (financial, behavioral) and providers (behavioral)
- Personnel (including multidisciplinary teams, peer navigators, community health workers)

Available Funds: Up to $16 million

Total Direct Cost: $5 million in large awards / $2 million in small awards

Maximum Project Period: 5 years in large awards / 3 years in small awards
Research Areas of Interest

• Head-to-head comparisons with or without "usual care" that impact the following broad outcomes?
  – Patients’ access to care, high quality of care, support for self-care, and coordination of care across healthcare settings
  – Professional decision-making on the basis of patients’ personal values
  – Experiences that are important to patients and their caregivers
  – Efficiency of healthcare delivery, as measured by the amount of ineffective, duplicative, or wasteful care provided to patients (e.g. Choosing Wisely)
The Communication and Dissemination Research (CDR) program seeks to fund projects that address critical knowledge gaps in the communication and dissemination process:

1. the communication and dissemination of research results to patients, their caregivers, and clinicians

2. the communication between patients, caregivers, and clinicians

To enable patients and caregivers to make the best possible decisions in choosing among available options for care and treatment.

Available Funds: Up to $8 million
Total Direct Cost: $2 million
Maximum Project Period: 3 years
Research Areas of Interest

• Communication strategies to promote the use of health and health care CER evidence by patients and clinicians

• Dissemination strategies to promote the use of health and health care CER evidence by patients and clinicians

• Explaining uncertain health and health care CER evidence to patients and clinicians
Addressing Disparities

- Inform the choice of strategies to **reduce** or **eliminate** disparities
- Focus on areas of importance where there are critical disparities that disadvantage members of a particular group and limit their ability to achieve optimal, patient-centered outcomes
- We are **not** interested in studies that describe disparities; instead we seek studies that identify best options for eliminating disparities

**Available Funds:** Up to $8 million

**Total Direct Cost:** $2 million

**Maximum Project Period:** 3 years
Research Areas of Interest

• Compares benefits and risks of treatment, diagnostic, prevention, or service options, with a focus on eliminating disparities

• Compares and identifies best practices for tailoring evidence-based interventions to patient populations at risk for disparities

• Targeted Populations of Interest:
  – Racial and ethnic minority groups
  – Low-income groups
  – Residents of rural areas
  – Individuals with special healthcare needs, including individuals with disabilities
  – Patients with low health literacy/numeracy and/or limited to English proficiency
  – LGBT persons
Patient and Stakeholder Engagement
Patients and Other Stakeholders

PCORI Community

- Patient/Consumer
- Caregiver/Family Member of Patient
- Purchaser
- Hospital/Health System
- Clinician
- Industry
- Policy Maker
- Hospital/Health System
- Training Institution
- Patient/Caregiver Advocacy Org
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**
  – Active engagement between scientists, patients, and stakeholders
  – Community, patient, and caregiver involvement already in existence or a well-thought out plan
Patient and Stakeholder Engagement

• Evidence that patients, caregivers, clinicians, and other stakeholders have been and will be engaged in:
  – Formulating the research questions
  – Defining the characteristics of study participants, comparators and outcomes
  – Selecting the important outcomes to be assessed
  – Monitoring study conduct and progress
  – Designing plans for dissemination of study results
• Clear statement of the roles and the decision-making authority of all patient and stakeholder research partners
The Engagement Rubric

The rubric is intended to provide guidance to applicants, merit reviewers, awardees, and engagement/program officers (for creating milestones and monitoring projects) regarding engagement in the conduct of research. It is divided into three segments:

1. Planning the Study
2. Conducting the Study
3. Disseminating the Study Results
Budgeting

- Financial compensation of partners
- Expenses of partners (transportation, childcare, caregiver)
- Budgeting for program staff dedicated to engagement tasks
- Costs of engagement meetings and events (travel, food, audio visual)
- Additional time and resource to incorporate partner feedback into various project process
Many members of the patient and stakeholder community have requested that PCORI make the names of partnering individuals and organizations available to credit the contributions of the full research team adequately.

You should provide PCORI only those names of patient or stakeholder partners for whom you have obtained appropriate permission to disclose their identity to PCORI and for PCORI to use their names in public communications.

If partners wish to remain anonymous, you may use pseudonyms or categorical descriptors (e.g., caregiver to husband with COPD, breast cancer survivor of 20 years).

If you are selected for funding, the individuals and organizations you provided (including those described by pseudonym or categorical descriptor) will be listed on the project description page along with the other information about your project (such as abstract and PI).
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**
  https://www.pcori.org/research-results/about-our-research/research-methodology/pcori-methodology-standards
Administrative Requirements
Timeline – Cycle 3 2017

- **PFA Posted:** October 3, 2017
- **Letter of Intent (LOI) Deadline:** October 31, 2017
- **Application Deadline:** February 6, 2018
- **Merit Review:** April 2018
- **Awards Announced:** August 2018
- **Earliest Start Date:** October 2018
Using the PCORI Online System

- PCORI’s system for collecting, reviewing, managing Letters of Intent, Applications, Reviews, Contracts:
  https://pcori.force.com/engagement

- Register as a New User and create your LOI as soon as possible, if appropriate

- Please note that the PI and AO cannot be the same person

- Enter information into all required fields in the system

- PCORI Online Training Resources
Letters of Intent to Apply: Eligibility to Submit

- 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.
Letter of Intent (LOI): Requirements

• Submit your LOI through PCORI Online

• An LOI is required and must be submitted prior to the deadline.

• To submit an LOI, download the **PFA-specific Letter of Intent Template** from the [Funding Center](#) to begin your LOI.

• You must answer all questions.

• Do not upload additional documents as part of your LOI. Letters of endorsements or support are not accepted at this stage.

• Only those LOIs deemed most responsive (programmatically and administratively) to this PFA will be invited to submit a full application.
From LOI to Application

• Full applications are invited based on the information provided in the LOI.

• Changes to the following require PCORI’s approval:
  • Principal investigator
  • Institution
  • Research question(s)
  • Specific aims
  • Study design
  • Comparators
  • Budget/period of performance
Invited to Apply: Tips for Success

- Adhere to the Application Guidelines for the appropriate PFA and funding cycle
- Start and submit application early
- Have a copy of your approved LOI readily accessible
- Ensure that all team members can see the application in the system (check during the LOI stage)
- Inform your AO of your intent to submit
- Clearly describe comparators for the study
- Document evidence of efficacy/effectiveness for the intervention and comparator(s) and/or demonstrate that they are in widespread use
- Justify your power calculations based on prior evidence of anticipated effect sizes
- Clearly demonstrate the feasibility of the study
  - Show that have the team to do this and you are the right team
  - Define and support your recruitment and retention plan
  - Document that sites are already committed to participating
  - Include realistic timelines for site start-up, IRB approval, and recruitment
- Submit the completed application on/before the due date by 5:00 PM ET
2017 PCORI Methodology Standards

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

The 48 standards can be grouped into 2 broad categories and 12 topic areas.

**Cross-Cutting Standards**
- Formulating Research Questions
- Patient Centeredness
- Data Integrity & Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects

**Design-Specific Standards**
- Data Registries
- Data Networks
- Causal Inference Methods*
- Adaptive & Bayesian Trial Designs
- Studies of Medical Tests
- Systematic Reviews
- Research Designs Using Clusters

*The first standard for Causal Inference Methods (CI-1) is considered cross-cutting and applicable to all PCOR/CER studies.
Application Components & Templates

• As seen in the Application Guidelines
  – Projection Information (Abstracts)
  – Budget and Budget Justification
  – Milestones
  – People and Places
  – Research Plan
  – Letters of Support
  – Resubmission Letter (if applicable)
Application Components: Budget & Justification

• In PCORI Online, for the Budget tab complete the following sections:
  – Detailed Research Project Budget for Each Year of the Research Project Period
  – Detailed Peer-Review Budget for Peer-Review-Related Costs
  – Budget Summary for Entire Project

• In the Templates and Uploads tab, upload the **Budget Justification Template** for the prime applicant and each subcontracted organization for the entire Research Project Budget and Peer-Review Budget for all research and peer-review-related costs. Include the federally negotiated or independently audited indirect cost rate letter (prime contractor) and fringe benefit rate policy verification document (prime contractor).
Application Components: Milestones/Deliverables

- Milestones
  - Significant events, deliverables, tasks, and/or outcomes that occur over the course of the project that mark progress toward the project’s overall aims

- Deliverables
  - Measurable and verifiable outcomes or products that a project team must create and deliver according to the contract terms

- See Appendix 1 of the Application Guidelines for examples of milestones.
Application Components: People & Places Template

• **Leadership Plan Template (Dual PI Applications only)**
  - Describe the governance and organizational structure of the leadership team and the research project;
  - Delineate the administrative, technical, scientific, and engagement responsibilities for each PI and the rationale for submitting a dual-PI application;
  - Discuss communication plans and the process for making decisions on scientific and engagement direction;
  - Describe the procedure for resolving conflicts.
  - **Note:** If this template is applicable, it should be uploaded as the first section of the People and Places Template.
Application Components: People & Places (cont.)

- **Leadership Plan (if applicable):** 5 pages
  - Required for Dual-PI Applications
- **Project/Performance Site(s) and Resources:** 15 pages
  - Provide a description of the facilities that will be used during the project, including capacity, capability, characteristics, proximity, and availability to the project.
- **Professional Profile/Biosketch:** 5 pages per individual
- **Patient/Stakeholder Partner Profile/Biosketch:** 5 pages per individual
Application Components: Research Plan

• **Research Strategy:** *(REVISED LIMIT) 12 pages*
  – Provide all the information requested, as outlined in the template:
    • Specific Aims
    • Background
    • Significance
    • Study Design or Approach

• **Research Team & Environment:** *(NEW) 2 pages*
  – Describe the research team’s capabilities to accomplish the goals of the proposed research project and the appropriateness of the research environment to conduct the study.

• **Dissemination & Implementation:** 1 page
  – Describe how you will make study results available to study participants after you complete the analyses, and possible barriers to disseminating and implementing the results of this research in other settings.
• **Consortium Contractual Arrangements: 5 pages**
  – Describe the proposed components of the research project that will be performed by subcontracted organizations.
  – Explain the strengths that these partners bring to the overall project to ensure successful submission of contract deliverables in accordance with the milestone schedule.

• **Appendices: 10 pages**
  – Applicants can include additional materials that they believe are useful, but reviewers are not required to review the appendix materials in evaluating the application.

• **Methodology Standards Checklist: (NEW) no page limit**
  – Applicants must complete each column of this checklist, as appropriate, and include it with the Research Plan PDF upload.
Letters of support should be addressed to the PI to demonstrate the commitment of key personnel and supporting organizations to the proposed project.

Letters of support should be organized in the following manner:

- Letters of organizational support
- Letters of collaboration
- Letters confirming access to patient populations, data sets, and additional resources
Using the PCORI Online System

- Navigate to PCORI Online (https://pcori.force.com/engagement)
- Log into the PCORI system early
- Please only use **Chrome, Safari, and Firefox browsers** to access the system
- The PI and the AO cannot be the same individual
- [PCORI Online Training Slides](#)
- [PCORI Online Application Cheat Sheet](#)
Merit Review
What happens to your application after you submit it?
Administrative Screening

Applicants **must follow** the administrative requirements stated in PCORI’s Application Guidelines.

Applications may be administratively withdrawn for the following reasons:

- Exceeding budget or time limitations
- Not using PCORI’s required templates
- Submitting incomplete sections or applications
Programmatic Screening

Applications may be programmatically withdrawn for the following reasons:

- Deviation from the approved LOI
- Inclusion of cost-effectiveness analysis (CEA)
- Inclusion of development and dissemination of clinical practice guidelines (CPG)
- Not responsive to the program-specific PFA
Merit Review Process

- pcori.org/content/merit-review-process
Application Review

Applications are reviewed against six criteria:

1. Potential for the study to fill critical gaps in evidence.
2. Potential for study findings to be adopted into clinical practice and improve delivery of care.
3. Scientific merit
4. Investigator(s) and environment
5. Patient-centeredness
6. Patient and stakeholder engagement

- Each application is reviewed by three scientists, one patient, and one other stakeholder.
- PCORI’s Board of Governors makes funding decisions based on merit review and staff recommendations.
## Merit Review Criteria

### Crosswalk of PCORI Merit Review Criteria with NIH Criteria

| SIGNIFICANCE                                                                 | 1. Potential for the study to fill critical gaps in evidence  
2. Potential for the study findings to be adopted into clinical practice and improve delivery of care |
|-------------------------------------------------------------------------------|-------------------------------------------------------------------|
| APPROACH                                                                      | 3. Scientific merit (research design, analysis, and outcomes)  
4. Investigator(s) and environment                                             |
| PCORI-only Merit Review Criteria                                               | 5. Patient-centeredness  
6. Patient and stakeholder engagement                                          |
## Submission and Key Dates

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<th>What</th>
<th>When</th>
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<tr>
<td>Application Deadline</td>
<td>February 6, 2018 by 5:00pm ET</td>
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<td>Merit Review Dates</td>
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<td>Awards Announced</td>
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<tr>
<td>Earliest Start Date</td>
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Refer to the funding opportunities page in our Funding Center (http://www.pcori.org/funding-opportunities) for the following resources:

- PFA and Application Guidelines
- PCORI Online User Manuals
- Sample Engagement Plans
- General Applicant FAQs: https://help.pcori.org/hc/en-us/categories/200010230-Applicant-Resources
- PCORI Online: https://pcori.force.com/engagement/
- Research Methodology: http://www.pcori.org/node/4020
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 question box in GoToWebinar

Ask a question via phone at the end of the presentation.

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