Cycle 3 2019: Broad PCORI Funding Announcements (PFAs)

Applicant Town Hall
September 25, 2019
Agenda

• About PCORI
• Programmatic Overview
• Patient and Stakeholder Engagement
• Administrative Overview
• Merit Review Process
• Resources
• Questions and Answers

Submit questions via the Question box in GoToWebinar
Today’s Presenters

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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 PFA: 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
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.
Research funded by PCORI must adhere to the [PCORI Methodology Standards](#), which represent minimal requirements for the design, conduct, analysis, and reporting of patient-centered outcomes research.

The 65 standards can be grouped into 2 broad categories and 16 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
- Studies of Complex Interventions
- Qualitative Methods
- Mixed Methods Research
- Individual Participant-Level Data Meta-Analysis (IPD-MA)

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*The first standard for Causal Inference Methods (CI-1) is considered cross-cutting and applicable to all PCOR/CER studies.*
Broad PFA: Overview and Research Areas of Interest
Studies that inform the choice of strategies to reduce or eliminate disparities

Focus on areas of importance where there are critical disparities that disadvantage members of particular groups 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: $3 million
Maximum Project Period: 3 years for all awards
Research Areas of Interest

Addressing Disparities seeks to fund research that:

- 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

Open to all topics, and encourage studies on:

- Improving Perinatal Outcomes for Low-Income African-American Women and Infants
- Diagnosis, Initiation of Treatment, and Retention of African Americans and Hispanics/Latinos along the HIV Care Continuum
- Interventions to Reduce Disparities in Obstructive Sleep Apnea and Insomnia in Racial and Ethnic Minorities

Proposed studies must be justified by well-defined evidence gaps.
Proposed interventions should be justified with a well-defined, explicit causal framework.
Addressing Disparities Target Populations

Research must focus on at least one of the following populations:

• Racial and ethnic minorities
• Low-income populations
• Residents of rural areas
• Individuals with special healthcare needs, including individuals with disabilities
• Patients with low health literacy/numeracy and/or limited English proficiency
• Sexual and gender minorities
Assessment of Prevention, Diagnosis, and Treatment Options

- Direct comparisons of clinical services for identifying, managing or treating specific diseases.
- Proposed studies must be justified by well-defined evidence gaps, and should describe the decisional dilemma for patients, clinicians, and other stakeholders.
- Consideration of treatment effect modifiers, including demographic, biological, clinical, socioeconomic and other factors.
- Comparative **effectiveness** studies addressing rare diseases.

**Available Funds:** Up to $14 million  
**Total Direct Cost:** $3 million  
**Maximum Project Period:** 3 years
Research Areas of Interest

• High priority topics as identified by systematic reviews, evidence gaps called out in clinical guidelines, or other credible evidence reviews.

• Comparisons of the effectiveness of two or more strategies 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
The Communication and Dissemination Research (CDR) priority area 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 $3 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
Improving Healthcare Systems

• Studies comparing the effectiveness of alternative features of healthcare systems that optimize quality, outcomes, and efficiency of patient care.

• Categories of innovation
  • Information technology (IT) or other electronic technologies (e.g., EHR, telemedicine)
  • Organizational structures and policies (e.g., standing orders, integrated health systems)
  • Incentives for patients (financial, behavioral) and providers (behavioral)
  • Personnel (e.g., multidisciplinary teams)

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

• Head-to-head comparisons with or without "usual care" that impact the following 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
  • Efficiency of healthcare delivery, as measured by the amount of ineffective, duplicative, or wasteful care provided to patients

• Test practices that combine evidence-based guidelines with patient incentives, provider incentives, or patient and provider incentives.

• Leverage available healthcare system and research resources.
Leveraging existing resources, PCORnet

• PCORI is interested in new research that derives data from a **wide variety of sources** and that uses study designs appropriate for the goals of the proposed project.

• PCORI has invested in PCORnet, a research infrastructure of health care systems and stakeholders that may prove useful to research applicants.

• For additional information on PCORnet go to [PCORnet.org](https://www.pcornet.org)

• **Applicants that propose to use PCORnet should contact the PCORnet intake team** to schedule a consultation and ensure the proposed use of PCORnet is appropriate and feasible, and that budget materials accurately reflect the cost of using PCORnet resources.
  
  • More information on PCORnet is available here ([https://www.pcornet.org](https://www.pcornet.org)) and consultations can be initiated by completing the intake form located here ([https://pcornet.org/contact-us/](https://pcornet.org/contact-us/)).

• PCORI does not intend for this PFA to support the development of new data networks or patient registries, but rather to **support the effective utilization of existing data resources for proposed new CER studies.**
Patient and Stakeholder Engagement
Patients and Other Stakeholders

PCORI Community

- Patient/Consumer
- Caregiver/Family Member of Patient
- Payer
- Industry
- Policy Maker
- Training Institution
- Hospital/Health System
- Advocate
- Clinician
- Hospital/Health System
- Industry
- Policy Maker
- Training Institution
- Hospital/Health System
- Advocate
- Clinician
Patient-Centeredness vs. Patient Engagement

- **Patient-Centeredness**
  - Addresses questions and outcomes (both benefits and harms) that important to patients
  - The interventions proposed for comparison are currently available to patients
  - Study design is low-burden to participants

- **Patient and Stakeholder Engagement**
  - Demonstrates active engagement with patients and other stakeholders
  - Involves relevant organizations, community, patients, and caregivers through existing relationships or presents a well-thought out plan to establish these partnerships
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 reference previously documented decisional dilemmas, and describe how this consultation informed the proposed research project.

• Applications should identify patients and stakeholders consulted in determining that the proposed study addresses their evidentiary needs for decision-making and indicate how patients and stakeholders will be actively engaged as partners throughout the conduct of the study.
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:

Planning the Study

Conducting the Study

Disseminating the Study Results

PCORI Patient and Family Engagement Rubric

I. Overarching Concepts

- The rubric specifically focuses on patient and family engagement in research to help illustrate promising practices emerging in this relatively new area of engagement in research. The term “patient partners” is intended to include patients (those with first-hand experience), family members, caregivers, and the organizations that represent them who are representatives of the population of interest in a particular study.

- Although the rubric is called the Patient and Family Engagement Rubric, there is an expectation that engagement of other stakeholders (e.g., clinicians, payers, or hospital administrators) that are relevant to a particular study will also be evaluated.

- The rubric is intended to provide guidance to applicants, merit reviewers, awardees, and engagement/program officers (for creating milestones and monitoring progress) regarding patient and family engagement in the conduct of research. It is not intended to be comprehensive or prescriptive. Instead, it provides a variety of options to incorporate engagement, where relevant, into the research process. Applicants can choose to include some, but not all, activities, and can include additional innovative approaches not listed here.

- The rubric is based on the promising practices identified in the first three rounds of PCORI awards. It is also consistent with PCORI’s Methodology Standards for patient-centricness and its PCORI Engagement Principles.

- The rubric is structured into four sections: Planning the Study, Conducting the Study, Disseminating the Study Results, and PCORI Engagement Principles.

- The rubric provides guidance to help applicants “show their work” when describing the details of how patient and family input will be incorporated throughout the entire research process.
Engagement Resources

Please visit PCORI's website for updated content and resources on the Public and Patient Engagement pages: [www.pcori.org/engagement](http://www.pcori.org/engagement)

- The value of engagement and planning for effective engagement in research
- Definitions and examples of different stakeholders
- Engagement Resources
  - Engagement Rubric
  - Compensation Framework
  - PCORI in Practice webinars
- Engagement in Health Research Literature Explorer
Budgeting

Things to Consider:

• 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).
Administrative Requirements
Using the PCORI Online System

• Register as a New User and create your LOI as soon as possible
• Please note that the PI and AO cannot be the same person
• Please only use Chrome, Safari, and Firefox browsers to access the system

https://pcori.force.com/engagement
PCORI Online Training Slides
PCORI Online Application Cheat Sheet
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

- 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
Application Components: Research Plan

• **Research Strategy:** 12 pages (upload as a single pdf file)
  • Provide all the information requested, as outlined in the template:
    * Specific Aims  * Significance
    * Background  * Study Design or Approach

• **Research Team & Environment:** 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.

• **Protection of Human Subjects:** 5 pages
  • PCORI follows the Federal Policy for the Protection of Human Subjects (45 CFR part 46), including the Common Rule.
Application Components: Research Plan (cont.)

• **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.

• **References Cited: 10 pages**
  - List the full bibliographical citation for each reference. Each reference must include the names of all authors, the article title and journal or book title, the volume number, the page numbers, and the year of publication.

• **Appendices: 10 pages (optional)**
  - 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: no page limit (upload as excel file)**
  - Applicants must complete each column of this checklist, as appropriate, and include it with the Research Plan PDF upload.
Additional Application Components

- **Milestones/Deliverables**: no page limit (upload as excel file)
- **People & Places Template** (upload as a pdf file)
  - Professional/Patient/Stakeholder Biosketch: 5 pages per individual
  - Project/Performance Site(s) and Resources: 15 pages
- **Leadership Plan Template** (Dual PI Applications only): 5 pages
- **Subcontractor Detailed Budget Template**: (upload as a excel file)
- **Budget Justification Template**: no page limit (upload as a pdf file)
- **Letters of Support**: no page limit (upload as a pdf file)
- **Resubmission letter**: 1 page (if applicable) (upload as a pdf file)
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

- Complete and upload the following templates into PCORI Online:
  - **Subcontractor Detailed Budget Template**: For each project year, complete a Detailed Budget for each subcontractor organization proposed in your application. All personnel information must be entered in the Personnel tab corresponding to that year in this template. Add additional rows for personnel as needed.
  - **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).
Merit Review
What happens to your application after you submit it?
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
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 (research design, analysis, and outcomes)
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.
## Submission and Key Dates

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<tr>
<th>What</th>
<th>When</th>
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<tr>
<td>LOI Deadline</td>
<td>October 1, 2019 by 5:00 pm ET</td>
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<tr>
<td>Application Deadline</td>
<td>January 14, 2020 by 5:00 pm ET</td>
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<td>Merit Review Dates</td>
<td>April 2020</td>
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<td>Awards Announced</td>
<td>July 2020</td>
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<td>Earliest Start Date</td>
<td>November 2020</td>
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Resources

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)
Thank you
Ask a question via the question box in GoToWebinar

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