

Cycle 3 2018: Improving Methods for Conducting Patient-Centered Outcomes Research (PCOR)

Applicant Town Hall
October 17, 2018

Agenda



- Programmatic Overview
- Administrative Overview
- PCORI Policy Update
- Merit Review Criteria
- Questions and Answers
 - [Submit questions via the question box in GoToWebinar](#)

Today's Presenters



Emily Evans, PhD MPH
Senior Program Officer,
Clinical Effectiveness &
Decision Science



Michele Lee Clements, CRA
Contract Administrator



Jason Gerson, PhD
Senior Program Officer,
Clinical Effectiveness &
Decision Science



Laura Sheahan, PhD
Merit Review Officer

1.



Programmatic Overview

Programmatic Overview



Emily Evans, PhD MPH

Senior Program Officer, Clinical Effectiveness & Decision Science

Cycle 3 2018: Methods PFA Overview



- PCORI seeks to fund applications that make a significant *methodological* contribution to PCOR/CER
- The Cycle 3 2018 Methods PFA is available at: <https://www.pcori.org/funding-opportunities/announcement/improving-methods-cycle-3-2018>

Available Funds & Project Duration:

- Up to \$750,000 in total direct costs per project
- Projects should be completed within 3 years

Cycle 3 2018 PFA: Programmatic Priorities

Resubmissions and New Applications

- Methods Related to Ethical and Human Subjects Protections (HSP) Issues in PCOR/CER
- Methods to Improve Study Design
- Methods to Support Data Research Networks
- Methods to Improve the Use of Natural Language Processing



*Research supported by the
Methods PFA*

General Guidance: Methods LOIs & Applications



- **Background and Significance**

- Identify, explain, and provide support for the specific anticipated *methodological* contributions to PCOR/CER.
 - Projects that simply apply best methods to a particular domain, seek to disseminate an approach, or develop infrastructure will not align with PCORI programmatic priorities.

- **Study Design or Approach**

- Provide a detailed description of the methodological work that is planned (e.g., theoretical development, simulation studies, data collection and analysis, empirical analyses, etc.).
- Applications must adhere to all relevant PCORI Methodology Standards.

General Guidance: Methods LOIs & Applications

continued



- **Evaluation**

- Describe and justify a plan for evaluation of the methods, including identification and examination of underlying assumptions.
- Applications must justify why the chosen data sources are optimal for the project (rather than just convenient to obtain).

- **Engagement (full application)**

- Engagement plans should be tailored and justified with respect to the specific aims, methods, and targeted end-users.
 - *Note:* Engagement is not required for Methods proposals but lack of engagement should be justified.

2018 PCORI Methodology Standards



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 54 standards can be grouped into 2 broad categories and 13 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

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

2.



Administrative Overview

Administrative Overview



Michele Lee Clements, CRA

Contract Administrator

LOI and 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

Research Plan Template

- **Research Strategy: Maximum 12 pages**
 - Provide all information requested, as outlined in the template:
 - Objectives
 - Background
 - Significance
 - Study Design or Approach
 - Engagement Plan (*should not exceed 1 page of the 12 page Research Strategy*)
- **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.

Research Plan Template

continued

- **Dissemination & Implementation Potential: 1 page**
 - Describe how you will make study results available to study participants after you complete the analyses (as applicable).
 - Describe 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.

Research Plan Template

continued



- **References: 10 pages**
- **Appendices (optional): 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.
- **What Has Changed for the Cycle 3 2018 Funding Cycle**
 - The Methodology Standards Checklist is an Excel file that now has a dedicated upload field in PCORI online. It should not be uploaded with the Research Plan Template. Applicants must complete Column D, and completed subsequent columns, as appropriate.

People and Places Template



- **Leadership Plan Template (Dual PI application): 5 pages**
 - 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.

People and Places Template

continued



- **Professional Profile/Biosketch: 5 pages per person**
 - Required for all key personnel
 - Use NIH biosketch or PCORI's format
 - List all partners within the Key Personnel section
- **Patient and/or stakeholder biosketches: 5 pages per person**
- **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

Letters of Support

- 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

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
 - See Appendix 1 of the Application Guidelines for examples of milestones
- **Deliverables**
 - Measurable and verifiable outcomes or products that a project team must create and deliver according to the contract terms
- **What Has Changed for the Cycle 3 2018 Funding Cycle**
 - Milestones will now be collected through the Milestone Template, which should be uploaded to PCORI Online **as an Excel file**, not a PDF, instead of being entered directly into PCORI Online. **Remember to use the Methods-specific version of the Template.**
 - Save the milestone file as "Milestones_PILastName.xls" and upload according to the Application Guidelines.

- **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
 - **What Has Changed for the Cycle 3 2018 Funding Cycle:** The Subcontractor Detailed Budget Template for each subcontractor for each project year is now required and must be uploaded in PCORI online as an excel file.
 - Include the federally negotiated or independently audited indirect cost rate letter (prime contractor) and fringe benefit rate policy verification document (prime contractor)

Using the PCORI Online System

- **Navigate to PCORI Online** (<https://pcori.force.com/engagement>)
 - Please only use Chrome, Safari, and Firefox browsers to access the system



- **Reminders:**
 - Log into the PCORI system early
 - The PI and the AO cannot be the same individual
- **Resources**
 - [PCORI Online Training Slides](#)
 - [PCORI Online Application Cheat Sheet](#)

Tips for Success



- **Programmatic**

- Adhere to the Application Guidelines for the appropriate PFA and funding cycle
- Have a copy of your approved LOI readily accessible
- Start and submit application early

- **Administrative**

- 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
- Submit the completed application on/before the due date by **5:00 PM ET**

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

3.



PCORI Policy Update



Jason Gerson, PhD

Senior Program Officer, Clinical Effectiveness & Decision Science

Policy for Data Management & Data Sharing

- Approved by PCORI's Board of Governors in September 2018
 - <https://www.pcori.org/about-us/governance/policy-data-management-and-data-sharing>
- Articulates expectations for data management and data sharing for applicants and awardees
 - Specifies data and data documentation to be shared
 - Specifies when data to be made available for third-party requests
 - Describes third-party data request and review process
- Provides funding to support Awardees' time/effort to prepare data (as applicable)
- **Submit questions to: OpenScience@pcori.org**

Data Deposition: Requirements for Awardees

Broad PFAs

- Maintain full data package for 7 years
- PCORI may notify Awardee of its intent to provide funds for the deposition of the full data package in a PCORI-designated repository

Targeted & Pragmatic Clinical Studies PFAs

- Deposit full data package (or required data elements, as applicable) in a PCORI-designated repository

PCORnet PFAs

- Deposit applicable data elements, such as the full protocol, analytic code used to query PCORnet data, and aggregate level datasets in a PCORI-designated repository

4.



Merit Review Overview

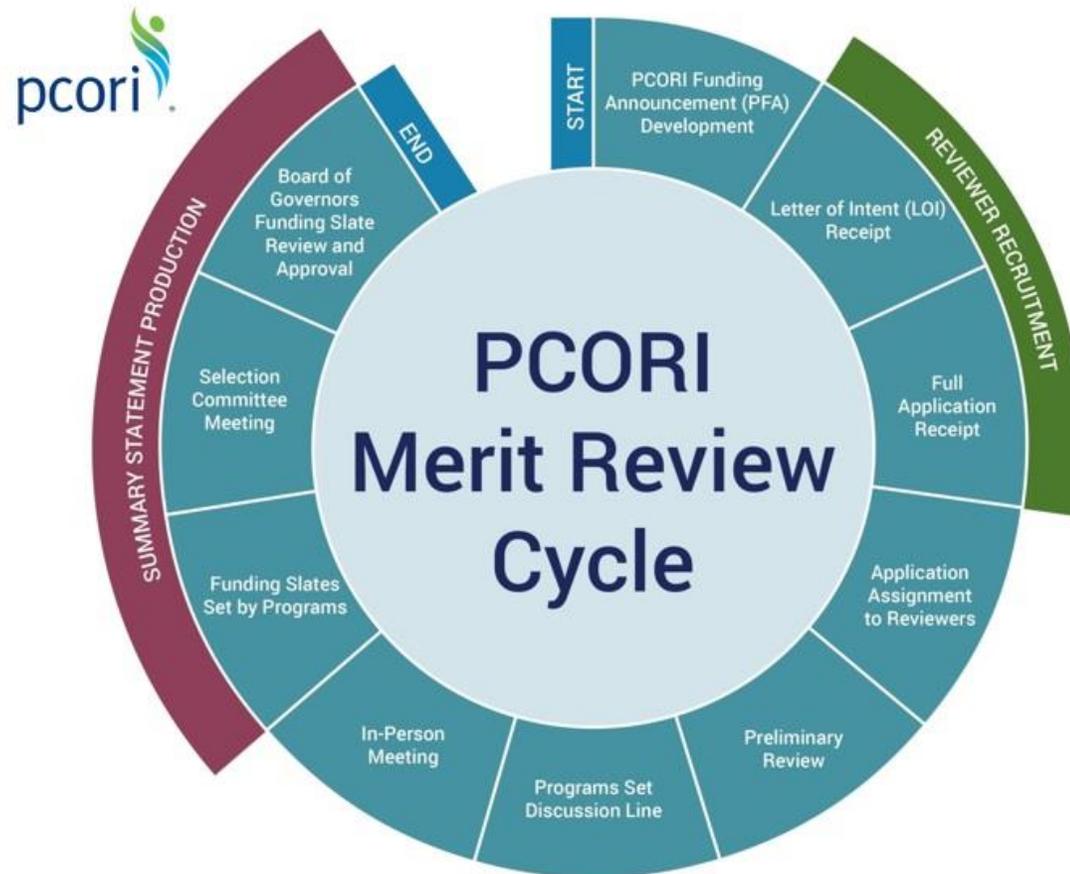
Merit Review Overview



Laura Sheahan, PhD

Merit Review Officer

Merit Review Process



www.pcori.org/content/merit-review-process

Application Review

Applications are reviewed against six criteria:

1. Study identifies critical methodological gap(s) in PCOR/CER
2. Potential for the study to improve PCOR/CER methods
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 stakeholder

PCORI's Board of Governors makes funding decisions based on merit review and staff recommendations.

Criterion 1

Study identifies critical methodological gap(s) in PCOR/CER



- Does the application identify and make a persuasive argument for addressing critical gaps in current PCOR/CER methods and provide sufficient support from the published scientific literature?

Criterion 2

Potential for the study to improve PCOR/CER methods



- Does the application articulate clearly how the development, refinement, comparison of methods, and/or the novel application of methods to PCOR/CER improves the validity, trustworthiness, and usefulness of PCOR/CER findings?
- Are the PCOR/CER methods generated from this study likely to inform best practices or standards for PCOR/CER?

Criterion 3

Scientific merit (research design, analysis, and outcomes)



- Does the application provide a clear conceptual framework or theoretical model and empirical evidence that inform the study design, key variables or constructs, analytical approach, and relationships being tested or explored?
- Does the application demonstrate adherence to the relevant PCORI Methodology Standards and describe methods that reflect state-of-the-art thinking and practice in the relevant methodological area?
- Are the study scope and timeline realistic, including the completion of specific scientific and engagement milestones?

Criterion 4

Investigator(s) and Environment

- How well-qualified are the PIs, collaborators, and other researchers to conduct the proposed activities? Is there evidence of sufficient clinical or statistical expertise?
- Does the investigator or co-investigator have demonstrated experience conducting projects of a similar size, scope, and complexity?
- If the project is collaborative or dual-PI, do the investigators have complementary and integrated expertise? Are the leadership, governance, and organizational structures appropriate for the project?
- Is the level of effort for each team member appropriate for successfully conducting the proposed work?
- Does the application describe adequate availability of and access to facilities and resources (including patient populations, samples, and collaborative arrangements) to carry out the proposed research?
- Is the institutional support appropriate for the proposed research?

Criterion 5

Patient Centeredness



- Does the application articulate clearly how the study will improve PCOR/CER methods that address outcomes of interest to patients and their caregivers?
- *Note:* A study can be patient-centered even if the end-user is not the patient, as long as patients will benefit from the study findings (e.g., methods to produce more valid, trustworthy, and useful PCOR/CER findings).

Criterion 6

Patient & Stakeholder Engagement

- Are patients and/or other relevant stakeholders meaningfully engaged in appropriate phases of the research?
- Does the proposal demonstrate the principles of reciprocal relationships; co-learning; partnership; and trust, transparency, and honesty?
- If engagement is deemed inappropriate in some or all aspects of the proposed research, does the application justify why it is not appropriate?

Submission and Key Dates



| What | When |
|----------------------|---------------------------------|
| LOI Deadline | November 6, 2018 by 5:00 pm ET |
| Application Deadline | February 12, 2019 by 5:00 pm ET |
| Merit Review Dates | May 2019 |
| Awards Announced | August 2019 |
| Earliest Start Date | November 2019 |

Refer to the **funding opportunities** page in our **Funding Center** for the following resources:

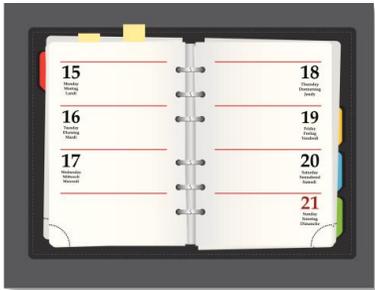
<http://www.pcori.org/funding/opportunities>

- PFA and Application Guidelines
- General Applicant FAQs: <https://help.pcori.org/Applicant-Resources>
- PCORI Methodology Standards: <https://www.pcori.org/research-results/about-our-research/research-methodology/pcori-methodology-standards>
- PCORI Online: <https://pcori.force.com/engagement/>
- PCORI Online User Manuals: <https://www.pcori.org/sites/default/files/PCORI-Online-User-Access-Cheat-Sheet.pdf>

Where can I find help?



- **Visit pcori.org/apply**
 - Application Guidelines
 - FAQs
 - PCORI Online User Manuals



- **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)



Ask 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 ****

Thank You!

