Limited PCORI Funding Announcement: Patient-Powered Research Networks (PPRN) Research Demonstration Projects

Published May 26, 2015

This limited PCORI Funding Announcement (PFA), which applies to the PPRN Research Demonstration Projects, closes on September 30, 2015, at 5:00 p.m. (ET). Funding announcements, templates, and other resources are available at http://www.pcori.org/PFA-PPRN-Demo-Projects.
About PCORI

The Patient-Centered Outcomes Research Institute (PCORI) is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI uses a variety of forums and public comment periods to obtain public input in order to enhance its work. PCORI helps people make informed healthcare decisions and improves healthcare delivery and outcomes by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

PCORI was authorized by the Patient Protection and Affordable Care Act of 2010 as a nonprofit, nongovernmental organization. PCORI’s purpose, as defined by the Act, is to help patients, clinicians, purchasers, and policy makers make better-informed health decisions by “advancing the quality and relevance of evidence about how to prevent, diagnose, treat, monitor, and manage diseases, disorders, and other health conditions.”
### Overview

**Published**

May 26, 2015

**Summary**

Patient-powered research networks (PPRNs) were funded by PCORI with the intent of supporting communities or networks of patients motivated to participate in clinical research through the National Patient-Centered Clinical Research Network (PCORnet) and to develop their capacity to govern the research activities of their networks. PCORI will fund up to eight individual research projects under this limited PCORI Funding Announcement (PFA).

PCORI seeks to fund PPRN-initiated research based on questions that have been generated and prioritized by participants within the PPRN community. There are three objectives:

1. **Relevance**: Answer important patient-identified research questions generated by the PPRN community that remain unanswered due to insufficient or inconclusive evidence.
2. **Collaboration**: Use, develop, and contribute to PCORnet’s shared tools and resources (the PCORnet Commons) to accelerate the conduct of research using PCORnet through collaborations with other PPRNs.
3. **Evaluation**: Evaluate the contribution and impact of the project to and on the development of the PCORnet Commons and on PCORnet’s capacity to support an increasing volume of future research.

**Applicant Resources**

http://www.pcori.org/PFA-PPRN-Demo-Projects

**Key Dates**

- **Online System Opens:** May 26, 2015
- **Applicant Town Hall:** June 17, 2015, 2 p.m. – 3 p.m. (ET)
- **Letter of Intent Deadline:** July 31, 2015
- **Application Deadline:** September 30, 2015, by 5 p.m. (ET)
- **Merit Review:** December 2015
- **Awards Announced:** January 2016
- **Earliest Project Start Date:** January 2016

**Maximum Project Budget (Total Costs)**

$2.5 million

**Funds Available up to**

$18 million

**Maximum Project Period**

3 years

**Eligibility**

For this limited PFA, PCORI is soliciting applications only from PCORnet PPRNs successfully funded in Phase II. This includes Phase I PPRNs that receive Phase II awards and new Phase II awardees.

The Internal Revenue Service must recognize all applicant organizations.

**Review Criteria**

1. Potential for study to improve health care and outcomes
2. Technical merit
3. Patient-centeredness
4. Patient and stakeholder engagement
| Contact Us | For programmatic questions, please email (sciencequestions@pcori.org), phone (202-627-1884), or contact us online (http://www.pcori.org/PFA/inquiry). PCORI will provide a response within three business days. However, PCORI cannot guarantee that all questions will be addressed three business days before an application deadline. Please email (pfa@pcori.org) for any administrative, financial, or technical questions. PCORI will provide a response within two business days. Applicants may also call the helpdesk (202-627-1885) for technical or administrative support. Please note that during the week of the application deadline, response times may exceed two business days. Applicants are asked to plan accordingly. It is the applicant’s responsibility to submit the application on or before the application deadline. |
| Other | Deadlines are at 5 p.m. (ET). If deadlines fall on a weekend or a federal holiday, the deadline will be the following Monday or the next day after the federal holiday, respectively. |
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Limited PCORI Funding Announcement: PPRN Research Demonstration Projects
I. Opportunity Snapshot

A group of people is motivated to participate in patient-centered outcomes research in order to inform better healthcare decisions for individuals in their communities. This group is ready and willing to engage with researchers and participate in prospective studies to understand their condition better. This group has even made strides in collecting some of these data through a protected online portal, which allows them to enter self-reported information and to access the aggregated, de-identified information of other participants. However, they are still a small group of very motivated individuals and will need to grow larger and become more representative of all people with the condition if they are to become a legitimate site for conducting valid research studies. In addition, they have not yet been able to obtain individual members’ electronic health record information, which will be an important part of building a research database. Realizing that they do not have the resources or research experience for continued growth or to conduct investigations themselves, they begin to reach out to skilled researchers in relevant clinical areas and to organizations skilled in collecting, linking, protecting, and analyzing data. This process presents some challenges. The community group is struck by how much difficulty researchers seem to have finding patients for their studies, despite their own group’s eagerness to work with them. Even with their eagerness to participate, the community group is dismayed by the complexity of the recruitment and enrollment procedures used by most research organizations. They wonder if there are more direct and efficient ways to find interested patients, obtain their consent for participation, and begin conducting research.

II. Introduction

Summary of Program

To improve our nation’s capacity to conduct clinical research efficiently to answer important questions faced by patients and clinicians, PCORI provided $105 million in 2014 to begin building the infrastructure for PCORnet. This large network represents patients across the country and will support research to improve health outcomes.

In Phase I of PCORnet, PCORI awarded funds for the development of 11 clinical data research networks (CDRNs), which are based in large health systems; 18 PPRNs, which are operated by patient-led groups; and one coordinating center (www.pcornet.org). To create a nimble and efficient national resource, we need collaborative, integrated, and flexible networks that are highly adaptable. The strength and value of PCORnet will ultimately depend on its ability to facilitate relationships between PPRNs, CDRNs, and other stakeholders and on its ability to develop and share tools and resources that have broad utility.

PPRNs were funded to support communities or networks of motivated people willing to participate in clinical research and to develop their capacity to govern the research activities of their networks. The main goals of the PPRNs during Phase I have been to develop a patient-centered governance and to increase the number of participants willing to become actively engaged in research. Such activities include generation of topics for research; collaborations with researchers to plan and carry out surveys, clinical trials, and other studies; and dissemination of results back to communities.

As the development of PCORnet moves into Phase II, anticipated to occur in September 2015, there is
the potential to bring participants’ voices to clinical research nationwide and transform research through true participant partnership. To meet those objectives, Phase II PCORnet activities will include:

- Supporting PPRNs in moving from a focus on the individual network to a focus on a larger community, where individual networks, while continuing to undertake research that is of interest to them, become part of a wider network engaged in bringing patient leadership to research

- Establishing the PCORnet Commons, where networks share tools, knowledge, expertise, and infrastructure—and develop ways to share these resources more efficiently—to accelerate progress in conducting research

- Empowering PPRNs and their communities to participate in key national discussions to accelerate clinical research

- Supporting individual research projects led by PPRNs

PCORI is issuing a limited funding opportunity to support patient-initiated research. PCORI will give awards for up to eight individual PPRN research projects. These projects must be designed to:

- Answer important patient-identified research questions generated by the communities a PPRN represents

- Accelerate the conduct of research within PCORnet by using, developing, and contributing to the PCORnet Commons

- Evaluate the contribution and impact of the project on the development of the PCORnet Commons and on PCORnet’s capacity to support an increasing volume of research

We encourage PPRNs to propose a project that includes collaboration between two or more PPRNs. The collaboration may take many shapes. It is expected that in most cases, the collaborating PPRNs will develop and/or share tools and resources. (Please note that only the patient community of the applicant PPRN must validate the application’s research question; validation is not required from the other participating PPRNs.)

PCORI will initially ask PPRNs to submit a Letter of Intent (LOI; see Section IV below) and will then invite selected PPRNs to prepare a full application. Each PPRN can be the lead on only one submitted LOI and can participate in a maximum of three LOIs.

Research of Interest

PCORI seeks to fund participant-driven research that answers questions generated and prioritized by participants within the PPRN community. PCORI is particularly interested in studies that:

- Propose novel methods, treatments, or other interventions, or strategies for conducting patient-centered research

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Propose a participant-generated question that has been identified as a critical gap in current knowledge from, for example, systematic reviews, guideline development efforts, or previous research prioritization

Collect outcomes that are important to community participants, such as overall health, ability to function, health-related quality of life, stress, severity of symptoms, and survival

Partner with patient leaders and community participants throughout the entire research process, from idea generation, through study development and conduct, to dissemination and implementation of findings

Foster and leverage partnerships with other PPRN networks and key stakeholders to develop methods, tools, or processes that benefit PCORnet as a whole

To enable PPRNs to address the most pressing needs of their participant communities and encourage innovation, we will consider a range of study designs. Comparative clinical effectiveness research (CER) conducted as observational studies or randomized clinical trials is preferred and will be prioritized during the review process. However, we will consider studies, including methods studies, that are required to conduct CER.

Applicants are strongly encouraged to review the funded research on PCORI’s website to ensure that their proposed research does not duplicate projects already funded.

**PPRN Research Opportunity**

For this limited PFA, PCORI is soliciting applications only from PCORnet PPRNs successfully funded in Phase II. This includes Phase I PPRNs that receive Phase II awards and new Phase II awardees.

We encourage collaboration between at least two PPRNs. Each PPRN can lead only one Letter of Intent (LOI) and participate in a maximum of three LOIs.

PCORI is requiring the use of a single Institutional Review Board (IRB) of record for each study.

**PPRN Research Demonstration Project Objectives**

The PPRN Research Demonstration Project has three main objectives:

1. **Relevance**: Provide high-quality evidence to answer important patient-identified research questions generated by the PPRN community

2. **Collaboration**: Use, develop, and contribute to the PCORnet Commons to accelerate the conduct of research

3. **Evaluation**: Assess the contribution of the project to the development of the PCORnet Commons and on PCORnet’s capacity to support an increasing volume of future research

**Continuation of Project**

Funding for years two and three will be contingent upon successful progress on and completion of deliverables (which will be determined during the contract negotiation phase) and an interim report. The interim report will identify challenges encountered, solutions identified, and enhancements made to the research study during the first year. The deliverables and interim report will need to demonstrate
the feasibility of the research plan and redesign, where appropriate.

III. Guidance for Proposing Research

The application must include a detailed research plan with a clear collaboration component, an engagement plan, an evaluation plan, a staffing plan, and a budget. Please see the Research Plan Template and Application Guidelines, which can be found in the Funding Center, for more detailed instructions on these elements.

The awardee institution(s) is responsible for the study, including oversight and dispersion of funds to any and all necessary subcontracts, including institutions within the PPRNs, CDRNs, and the PCORnet Coordinating Center.

Guidance on the Research Plan

This section relates to the PFA aims of relevance and collaboration.

All research plans must include a justification for the proposed research question and study design that includes:

- The importance of the research question to the patient/participant community of the applicant PPRN (other PPRNs involved in the study do not need to participate in the generation of the research question)
- The evidence gap that is addressed
- The choice of study design to answer the research question
- The anticipated impact of the study results on patient health outcomes
- Tools and resources available from other PPRNs that will be useful for this project
- Tools and resources, not available within PCORnet, that will be developed or adapted as part of this project
- Other tools and resources needed, including but not limited to:
  - Data governance policies and procedures
  - Network policies
  - Procedures and protocols
  - Data-use agreements
  - Business associate agreements
  - Contracting processes
  - Processes related to IRB submissions
  - Development of computable phenotypes
  - Measures of and procedures for using patient-reported outcomes
  - Training for patients and academic/clinical stakeholders
  - Sharing staff across PPRNs with specific expertise (e.g., biostatistics)
- Any proposed collaboration with one or more PPRNs to develop tools and resources
- A timeline that includes clear and specific scientific and engagement milestones
- A plan and timeline for when and how the tools and resources developed will be made available to the PCORnet community, facilitated by the PPRN Coordinating Center
- Key aspects of the collaborative activities, including how the activities will be managed and led by the PPRN collaborators, identify key responsibilities for each collaborator, highlight how
coordination will be enhanced and conflict will be mitigated, and describe relevant training or resources that may be necessary to facilitate the effectiveness of the collaboration and budget allocation (IOM, 2015\(^2\))

Additionally, where relevant, the plan should:

- Describe the data sources, means of data collection, follow-up period, study endpoints (including method by which events will be ascertained), statistical analyses, and anticipated limitations
- Describe how data will be captured for the proposed study, including the extent to which data already collected during Phase I will be leveraged for the research project and the additional data collection efforts that will be required during Phase II
- Confirm the number of network participants potentially willing and eligible to participate in the proposed study and provide evidence to support anticipated enrollee numbers if additional partnerships are being considered
- Identify how data elements from the PCORnet Common Data Model (CDM) Version 2.0 may be used, if at all (use of data elements in the CDM is optional, but encouraged where appropriate)
- List data elements not currently captured by CDM Version 2.0 that will be necessary for the research study and describe how these data elements could be incorporated into future versions of the CDM
- Address confounding issues associated with the observational or intervention design, where appropriate, and discuss the appropriateness of the analytical models proposed
- Discuss potential limitations to the study and how they will be addressed
- Provide a detailed plan for data sharing, where appropriate (see below)
- Indicate whether you have an IRB Master Reliance Agreement

When applicable, the research plan should indicate how the project will:

- Compare at least two alternative clinical interventions or approaches
  - Optimally, the study will compare two or more defined strategies. In general, “usual care” is not an appropriate comparator for CER studies submitted to PCORI for funding consideration. “Usual care” is too often ill-defined, difficult to quantify, and subject to considerable geographic and temporal variations, limiting its interpretability, applicability, and reproducibility. If the applicant proposes “usual care” as a rational and important comparator in the proposed study, then it must be described in detail, be coherent as a clinical alternative, and be properly justified as a legitimate comparator (e.g., usual care is guidelines-based). In addition, it must be accompanied by an

explanation of how the care given in the usual care group will be measured in each individual patient and how appropriate inferences will be drawn from its inclusion.

- Evaluate the benefits and harms of each intervention as delivered in typical clinical and community settings
- Ensure that the health outcomes studied are meaningful to the patient population under study; in selected instances, surrogate physiological measurements may be sufficiently linked to final health outcomes of interest, but they may not be the sole study outcome

**Guidance on the Engagement Plan**

PCORI requires all applicants to describe clearly the patient and stakeholder engagement planned for their proposed project. PCORI is not seeking one particular type or method of engagement. In addition, studies are expected to adhere to PCORI’s Methodology Standards Associated with Patient-Centeredness and to the PCOR Engagement Principles found within the [PCORI Engagement Rubric](https://www.pcori.org/priorities). These and additional resources are available in [PCORI’s Funding Center](https://www.pcori.org/funding-center).

The PCORI Engagement Rubric also provides guidance on promising practices to consider when developing an engagement plan.

The engagement plan for this application must:

- Describe how the applicant PPRN’s participant communities identified and prioritized the research question proposed and the methods used
- Provide a detailed plan for how stakeholders will partner throughout the research process; the Engagement Rubric provides guidance on promising practices for engagement in the planning, conduct, and dissemination of research
- Describe the mechanisms proposed to disseminate information to the PPRN participant community throughout the period of study, including data use and sharing, relevant interim results, changes to the initial plan, and final outcomes of the study, and it must consider their efficacy, including accessibility, appropriateness, and adequacy

Applicants are encouraged to budget for engagement activities appropriately, including the cost of meetings, travel, and other necessary expenses. In recognition of the value of their contributions, PCORI strongly encourages the financial compensation of patient and stakeholder partners serving on research teams.

**Guidance on the Evaluation Plan**

This section relates to the PFA aim of evaluation.

A significant aim of the PPRN demonstration projects is to assess and develop key aspects of the PCORnet infrastructure. The applicant PPRN should develop a plan to evaluate the contribution of the proposed project on the development of the PCORnet Commons, as well as its capacity to support an increasing volume of research in Phase II and beyond.

The application should include a list of activities and a timeline describing the evaluation process.
Results from the evaluation will be described in an interim report to PCORI at the end of the project’s first year and summarized at the end of the project in a final report that will be made available to the PCORnet community.

The evaluation plan should:

- List the potential resources that will be made available to the applicant PPRN by other PPRNs
- List the proposed resources that will be developed by the applicant PPRN and made available to the PCORnet Commons
- Describe how the project will assess the impact of the collaborative aspects of the study (between PPRNs and other stakeholder partnerships) on meeting the technical assistance needs of the PPRN community, enhancing the PCORnet Commons, and strengthening the capacity of PCORnet to conduct research
- Describe how the project will provide evidence of the scalability of the resources developed for the research project to other PPRNs and the larger PCORnet community; scalability refers to the adaptability and utility of the tool or resource for other PPRNs within the larger PCORnet community
- Describe how the project will provide evidence that the infrastructure developed by the PPRN will benefit future research conducted in PCORnet
- Evaluate whether the location of the PCORnet Commons is readily accessible for the PPRNs

Selection of the Principal Investigators

Individual research projects must be led by two co-Principal Investigators (PIs), one of whom is designated as lead PI and has the requisite qualifications and training for conducting the proposed research consistent with any applicable human subject research laws or other laws and any requirements of the applicable IRB. The lead PI must come from the applicant PPRN. The PIs for this research demonstration proposal can be different from the PPRN PIs. The two PIs should follow the designations listed below.

(1) PI representing patients or caregivers or the community

At least one PI should have the expertise or experience described below:

- Meaningfully representing participant perspectives as a patient, caregiver, or community member
- Working collaboratively with researchers

Given the challenges for patients or caregivers with full-time jobs in committing significant time to a research project of this nature, PCORI will be flexible with the time commitment from the patient PI.

(2) PI representing researchers or clinicians

At least one PI should have the expertise or experience described below:

- Research expertise in the content area of the proposal
Research expertise in epidemiology and/or health services
Expertise in working with data from his/her host institution
Leading research studies collaboratively with patient partners

Nonresponsiveness

Applications will be considered nonresponsive to this PFA if the proposed research:
• Duplicates questions already in the research topic database
• Exceeds the limits on budget and project duration (see below)
• Tests efficacy (the ability to produce the intended result) or comparative efficacy within a tightly protocol-controlled research setting (as opposed to a more real-world, clinical setting)
• Conducts a formal cost-effectiveness analysis in the form of dollar-cost per quality-adjusted life year to compare two or more alternatives
• Directly compares the costs of care between two or more alternative approaches

PCORI does have an interest, however, in studies of conditions that lead to high costs to the individual or to society. PCORI is also interested in studies that examine healthcare resources or costs as a determinant of—or barrier to—good outcomes. Examples include ways in which out-of-pocket costs may constitute barriers to care.

Further, PCORI considers it important for applicants to discuss cost-related issues, such as the resources needed to implement, replicate, or disseminate a successful intervention. PCORI is interested in the evaluation of interventions intended to reduce health-system waste or increase health-system efficiency. Proposals that include studies of these issues without using a formal cost-effectiveness analysis or comparing the costs of alternatives are considered responsive.

Features of Patient-Centered Outcomes Research

Patient-centered outcomes research (PCOR) helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research:
• Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health-delivery-system features to inform decision-making, highlighting the choices that matter to people
• Includes an individual’s preferences, autonomy, and needs, focusing on outcomes that people notice and care about, such as survival, functioning, symptoms, and health-related quality of life
• Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination
• Directly compares clinical interventions that are generally available in settings where people access health care

Preliminary Data and Use of Accepted Measures

PCORI encourages investigators to design their research using valid patient-centered outcome (PCO)
measures and to include preliminary data that support the proposed measures. Investigators are encouraged to consider those measures described in the Patient-Reported Outcomes Measurement Information System.

**Justification of Assumptions**

PCORI specifically seeks studies that are large enough to detect clinically meaningful effects. To that end, applicants must justify the proposed sample sizes by explaining the assumptions used to decide on those numbers. The application should clearly state all the necessary assumptions (i.e., the primary outcome measure, estimated difference in the mean value of this measure between study arms, standard deviation of the measure, type I error rate, and any other assumptions). All such estimates must be justified by referring to prior published research or preliminary data.

**Adherence to Methodology Standards**

Regardless of study design, proposals must adhere to all relevant PCORI Methodology Standards. These 47 individual standards fall into 11 categories. The first five categories are cross-cutting and relevant to most PCOR studies. Researchers should refer to the standards in these categories when planning and conducting their research projects. The categories are:

- Standards for Formulating Research Questions
- Standards Associated with Patient-Centeredness
- Standards on Data Integrity and Rigorous Analyses
- Standards for Preventing and Handling Missing Data
- Standards for Heterogeneity of Treatment Effects

Five other categories of standards will be applicable to particular study designs and methods and should be used for guidance when relevant. These categories are:

- Standards for Data Registries
- Standards for Data Networks as Research-Facilitating Infrastructures
- Standards for Causal Inference Methods
- Standards for Adaptive and Bayesian Trial Designs
- Standards for Studies of Diagnostic Tests

These should be considered minimal standards. Relevant additional best practices—including guidelines for the conduct of clinical trials developed by other organizations—should be addressed in the application.

**Recruitment**

Proposals should include information about the size of the potential pool of patients from which recruitment will occur and the means by which this size estimate was determined. Likewise, proposals should provide evidence-based estimates of how many participants are ultimately expected in the study based on expected recruitment, application of the study’s inclusion and exclusion criteria, anticipated
acceptance (or refusal) rates, and other factors such as loss to follow-up. Such estimates must be discussed in the applications, must be specified in the milestones, will be reviewed by merit reviewers and PCORI staff, and will be monitored by PCORI in the funded research.

**Protection of Human Subjects**

In the Research Plan Template, describe the protection of human subjects involved in your research. Use up to five pages. PCORI follows the Federal Policy for the Protection of Human Subjects (45 CFR part 46), including the Common Rule. For more detailed information, please see the Section 5 “Human Subjects Research Policy” from the Supplemental Grant Application Instructions for All Competing Applications and Progress Reports\(^3\) issued by the U.S. Department of Health and Human Services. PCORI does not require that applicants comply with sections of this policy that refer to requirements for federalwide assurance, or that refer to standards for inclusion of women, minorities, and children.

PCORI does require applicants proposing clinical trials to consider including a data and safety monitoring plan. Awardees must also comply with appropriate state, local, and institutional regulations and guidelines pertaining to the use of human subjects in research.

PCORI merit reviewers will examine plans for the protection of human subjects in all applications and may provide comments regarding the plans (see How to Evaluate Human Subjects Protections\(^4\)). Reviewers’ comments on human-subjects research are not reflected in the overall application score but may be used by PCORI staff during any potential funding negotiations. Final determinations about adequacy of human subject protections rest with the IRB or IRBs that have jurisdiction for the study. The awardee institution or organization bears ultimate responsibility for safeguarding the rights and welfare of human subjects in PCORI-supported activities.

**Required Education of Key Personnel on the Protection of Human Subject Participants**

PCORI requires that all applicants adhere to the National Institutes of Health (NIH) policy on education in the protection of human subject participants in the conduct of research. This applies to all personnel listed in the application as key personnel. The policy and FAQs are available from the NIH website.\(^5\)

**Replication and Reproducibility of Research and Data-Sharing Plan**

PCORI is committed to maximizing the utility and usability of data collected in our funded projects. This is essential to building confidence in the accuracy of these findings. PCORI supports policies to promote sharing of study documentation (e.g., study protocol, programming code, and data definitions) so that other researchers may replicate the findings in other populations. Please propose a method for sharing data and appropriate documentation upon request.

**Budget and Project Duration**

The maximum budget for each study in this limited PFA is $2.5 million in total costs. Up to $18 million in total costs will be awarded under this PFA for up to eight awards. The maximum period of performance

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is three years. PCORI will not consider exceptions to the budget and period of performance limits. If you submit an application that exceeds $2.5 million in total costs or three years in period of performance, your application will be removed for noncompliance. In the budget justification, the budget for the research activities should be clearly delineated from the budget for the evaluation activities.

IV. How to Submit a Proposal

Letter of Intent

Applicants are required to submit a Letter of Intent (LOI) for the individual project. Each PPRN can submit only one LOI as the sole or lead network. PPRNs can participate in a maximum of three LOIs.

While PCORI is soliciting LOIs from Phase I PPRNs and new PPRN Phase II applicants, please note that only Phase I PPRNs that receive Phase II awards and new Phase II awardees will be eligible for the award.

Applicants should download the Letter of Intent Template from the PCORI Funding Center. They must complete the document and convert it to a PDF with a length limit of four pages. All references must be listed at the end of the LOI. LOIs that exceed the page limit (excluding references) will not be reviewed.

Do not upload additional documents as part of your LOI. Letters of endorsement or support are not required at this stage. Their inclusion will result in rejection of the LOI without review.

Please visit the PCORI Funding Center for additional applicant resources, including required templates.

Please answer all of the questions in the LOI Template and then upload your document into the PCORI Online System. The deadline for LOI submission is July 31, 2015 by 5 p.m. (ET).

Letter of Intent Review

LOIs will be reviewed by PCORI staff for programmatic fit and responsiveness to the PFA and Application Guidelines. LOIs are screened against the following criteria:

- Strength of the patient-driven process to generate and prioritize research questions
- Question’s importance to the patient/participant community and demonstration that the research will fill an evidence gap
- Suitability of the research design
- Exclusion of any cost-effectiveness analysis
- Strength of the engagement approach
- Potential to contribute to the PCORnet Commons through the development of shared tools and resources
- Strength of the proposed collaborations with other PPRNs
- Appropriateness of proposed team
- Programmatic fit and balance

Applicants will be notified no later than August 12, 2015 as to whether they have been selected to
submit a full application.

**Note:** LOIs that show with scientific overlap or that appear to be duplicate submissions will be removed during the LOI screening process.

**Submission Dates**

Applications must be submitted in accordance with the published dates and times listed in the Overview of this PFA and in the PCORI Funding Center.

**PCORI Online System**

To submit an application, you must register with PCORI Online.

**Applicant Resources**

- **PCORI Funding Center**  
  [http://www.pcori.org/PFA-PPRN-Demo-Projects](http://www.pcori.org/PFA-PPRN-Demo-Projects)
- **PCORI Online System**  
  [https://pcori.fluxx.io](https://pcori.fluxx.io)
- **PCORI Funding Awards**  
  [pcori.org/pfaawards](pcori.org/pfaawards)

**IV. Merit Review**

PCORI’s merit review of applications is a multiphase process that includes:

- Preliminary review of full applications by review panels
- In-person panel discussion of a subset of full applications (identified by PCORI’s Research Priority Area Program staff and based on the preliminary review and program priorities)
- PCORI Selection Committee recommendation of applications for funding
- Board of Governors award approval (expected to be no later than January 2016)

**Preliminary Review**

PCORI conducts a rigorous merit review of the full applications it receives. Applications may be eliminated from the review process for administrative or scientific reasons. An application may be administratively withdrawn if it is incomplete or submitted late, or if it does not meet the administrative or formatting criteria outlined in the Application Guidelines, PCORI templates, and PCORI Online System. An application may be scientifically withdrawn if it is not responsive to the guidelines as described in this PFA, includes a cost-effectiveness analysis, or otherwise does not meet PCORI programmatic requirements.

A single merit review panel recruited by PCORI Merit Review Officers will review administratively and scientifically responsive applications. The review panel is composed of a chairperson, scientific experts, patient representatives trained in the review of scientific proposals, and representatives of other stakeholder groups. The scientific experts may include clinical trial specialists, methodologists, and statisticians.
The following are PCORI’s merit review criteria for this announcement. These four criteria will be used by the review panel during the preliminary and in-person phases to evaluate and score all submitted applications.

**Criterion 1. Potential for the study to improve health care and outcomes**

The proposal has the potential to lead to meaningful improvement in the quality and efficiency of care and to improvements in outcomes important to the PPRN participant community. This criterion is assessed through the following questions:

- Does the research question address a critical gap in current knowledge as noted in systematic reviews, guideline development efforts, and/or PPRN research prioritization exercises, and was it generated by the patient or community of the PPRN?
- Has the research question been identified as important by patient, caregiver, or clinician groups?
- Is the research novel or innovative in its methods or approach, in the population being studied, or in the intervention being evaluated, in ways that make it likely to improve care?
- Does the applicant provide evidence, either from previous studies or pilot work, that indicates the potential for a sizable benefit of the intervention (or method) relative to current practice?
- Do the applicants present a credible plan by which findings could be disseminated and implemented quickly within PCORnet (including at the local hospital or clinic level), resulting in improvements in practice and patient outcomes?

**Criterion 2. Technical merit**

The proposal has sufficient technical merit in the research design to ensure that the study goals will be met. This criterion is assessed through the following questions:

- Is there a clear research plan with rigorous methods that adhere to PCORI’s Methodology Standards and prevailing accepted best practices?
- Is there a clear and adequate justification for the study design choices in the proposed study?
- If the study is comparative in nature, are the comparison interventions realistic options that exist in current practice?
- Is the plan for recruitment (if proposed) realistic?
- Are the sample sizes and power estimates presented based on realistic and careful evaluations of the anticipated effect size, where appropriate?
- Is there a plan to recruit a representative study population with respect to age, gender, race, ethnicity, and clinical status (where appropriate), and has the applicant provided evidence that the recruitment of this population is feasible?
- Does the project include a realistic timeline that includes clear and specific scientific and
engagement milestones?

- Does the research team have the necessary expertise to complete the research study successfully, including the collaborative aspects and the evaluation? If not, has the applicant partnered with appropriate organizations or experts to assist with their technical assistance needs?

- Does the applicant provide a clear rationale for the collaborative tools, training, and resources being adapted or developed, as well as compelling evidence that the PPRN partnerships selected will optimize the use of existing resources or enhance the efficiency of the co-design processes proposed?

- Do the collaborative aspects of the research project proposed provide an opportunity to transform PCORnet processes or enhance aspects of the PCORnet infrastructure?

- Does the applicant provide a robust approach to evaluating the contribution and impact of the project on the development of the PCORnet Commons and on PCORnet’s capacity to support an increasing volume of research in Phase II and beyond?

**Criterion 3. Patient-centeredness**

The proposal demonstrates patient-centeredness at every stage of the research. This criterion is assessed through the following questions:

- Was the research question generated by patients and/or communities from the PPRN?

- Do the research question and proposed comparisons reflect a choice or choices faced by patients, their caregivers, or clinicians?

- Does the study protocol include health outcomes, including validated Patient Reported Outcomes (PROs) (if appropriate), that are relevant to patients?

- Is there an adequate plan to protect human subjects participating in this study?
  - Does the project use efficient and novel methods to obtain participant consent while still meeting ethical and legal requirements?
  - Does the application adequately describe how potential risks to participants appear reasonable in relation to anticipated benefits?

**Criterion 4. Patient and stakeholder engagement**

The proposal demonstrates that people representing the population of interest and other relevant stakeholders are leading in ways that are appropriate and partnering with other relevant stakeholders where necessary in a given research context. This criterion is assessed through the following questions:

- Are patients and other stakeholders (including professional and patient organizations) leading or partnering in:
  - Identifying the research question?
Designing the study?

- Defining essential characteristics of study participants, comparators, and outcomes?
- Identifying and selecting outcomes that the population of interest cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform decision-making relevant to the research topic?
- Monitoring study conduct and progress?
- Designing plans for dissemination and implementation activities?

- Are the roles and the decision-making authority of all research partners clearly stated?
- Does the proposal demonstrate the principles of reciprocal relationships: co-learning, partnership, trust, transparency, and honesty?

Panel Discussion

After the preliminary review is complete, reviewers meet in person for a panel discussion and further review. During the panel discussion, panelists clarify the merits of the proposed research and study protocol and identify areas for improvement. Applications are then rescored based on the discussion. The Chair and a PCORI Merit Review Officer (MRO) lead the in-person panel meeting and ensure that all applications receive a fair and thorough review informed by the standards outlined in the PFA.

Post-Panel Review

After the panel discussion, PCORI program staff review the scores and comments for the meritorious applications and consider the fit of applications within the programmatic vision. Up to eight applications will then be recommended to a selection committee that includes members of PCORI’s Board of Governors. The selection committee will work with staff to approve or reject a recommendation of funding, taking into account PCORI’s strategic priorities. No more than eight individual research awards will be proposed to PCORI’s Board of Governors for its consideration and final approval.

Funding Recommendations

It is expected that applicants will receive notification of their application’s funding status no later than January 2016. The awards will be for three years, although funding for Years 2 and 3 is contingent on the one-year interim report, which must demonstrate the project’s feasibility and the ability of its staff to redesign, where necessary.

Contract Execution and Activation

PCORI will issue a contract to the selected awardee institution for each study once it conducts a thorough programmatic and administrative review. The awardee accepts PCORI’s contract terms and conditions, which will be based on PCORI’s research funding contract terms and conditions with additional provisions appropriate for the use of the PCORnet infrastructure and the specific research project. Among the expected contractual terms is a fully agreed-upon study plan as evaluated by PCORI. The study will commence only after PCORI and the awardee institution execute the applicable contract and agree on the final protocol content.