



## Cycle 1 2019 Funding Cycle

### Limited PCORI Funding Announcement: Partnerships to Conduct Research (PaCR) within PCORnet

Published March 7, 2019

Updated April 9, 2019

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Letters of Intent for this limited PCORI Funding Announcement (PFA) are due April 15, 2019, by 5 pm (ET). This limited PFA closes on June 24, 2019, at 5 pm (ET). Application Guidelines, templates, and other resources are available at <https://www.pcori.org/funding-opportunities/announcement/limited-pcori-funding-announcement-partnerships-cycle-1-2019>.

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## **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 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 and other stakeholders.

PCORI was authorized by Congress in 2010 as a nonprofit, nongovernmental organization. PCORI's purpose, as defined by our authorizing legislation, is to help patients, caregivers, clinicians, policy makers, and other healthcare system stakeholders 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."

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## Overview

Published	March 7, 2019														
Letter of Intent Due	<p>April 15, 2019, by 5 pm (ET)</p> <p>All applicants must submit a noncompetitive Letter of Intent (LOI) by the April 15, 2019, deadline to be eligible to submit a full application. Please refer to the <a href="#">Application Guidelines</a> on the PCORI Funding Opportunities web page for information on how to submit your LOI in <a href="#">PCORI Online</a>.</p>														
Summary	<p>PCORI-funded Clinical Research Networks (CRNs; formerly known as Clinical Data Research Networks, or CDRNs) to support system-based networks that include hospitals and community-based practices to capture complete, longitudinal healthcare data and to develop their capacity to conduct a range of study designs, including large longitudinal observational studies, large pragmatic clinical trials conducted within delivery systems, and rapid-cycle research in concert with health systems and plans.</p> <p>PCORI also funded Health Plan Research Networks (HPRNs) to partner with PCORnet CRNs to establish the governance framework and subsequent implementation necessary to link longitudinal healthcare claims with CRN healthcare data for use in comparative clinical effectiveness studies.</p> <p>In this limited PCORI Funding Announcement (PFA) for CRNs and HPRNs, PCORI seeks to fund up to two high-quality clinical studies to answer important patient- and stakeholder-prioritized clinical comparative effectiveness research (CER) questions that remain unanswered due to insufficient or inconclusive evidence. An important aim of this PFA is to promote PCORnet sustainability through collaboration and engagement with non-PCORI co-funders in the conduct of CER and to promote greater completeness of PCORnet data through linkages of CRN electronic data with that of health plans, disease registries, and other complementary data sources on individuals within CRN or HPRN databases. These capabilities—attracting external funders and improving PCORnet’s capacity for data linkage—are fundamental steps in building a sustainable national research infrastructure that attracts a diverse set of public and private funders of research.</p> <p>Note that this funding program does not support applications to conduct cost-effectiveness analyses or systematic reviews (with or without meta-analyses). It also does not support applications to develop or conduct an efficacy evaluation of shared decision making. PCORI will not cover costs for clinical interventions (e.g., screening, diagnostic or treatment interventions) that are being compared in the proposed study (see Appendix 2: Allowable and Unallowable Costs in the <a href="#">Application Guidelines</a> for details).</p>														
Applicant Resources	<a href="https://www.pcori.org/funding-opportunities/announcement/limited-pcori-funding-announcement-partnerships-cycle-1-2019">https://www.pcori.org/funding-opportunities/announcement/limited-pcori-funding-announcement-partnerships-cycle-1-2019</a>														
Key Dates	<table> <tr> <td>Online System Opens:</td> <td>March 7, 2019</td> </tr> <tr> <td>Applicant Town Hall Session:</td> <td>April 3, 2019, 12 pm–1:00 pm (ET)</td> </tr> <tr> <td>LOI Deadline:</td> <td>April 15, 2019, by 5 pm (ET)</td> </tr> <tr> <td>Application Deadline:</td> <td>June 24, 2019, by 5 pm (ET)</td> </tr> <tr> <td>Merit Review:</td> <td>August 5, 2019</td> </tr> <tr> <td>Awards Announced:</td> <td>October 2019</td> </tr> <tr> <td>Earliest Project Start Date:</td> <td>January 2020</td> </tr> </table>	Online System Opens:	March 7, 2019	Applicant Town Hall Session:	April 3, 2019, 12 pm–1:00 pm (ET)	LOI Deadline:	April 15, 2019, by 5 pm (ET)	Application Deadline:	June 24, 2019, by 5 pm (ET)	Merit Review:	August 5, 2019	Awards Announced:	October 2019	Earliest Project Start Date:	January 2020
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Maximum Project Budget (Direct Costs)	\$2 million (maximum direct costs from PCORI), with a requirement for additional funding from another funding source (50 percent co-funding of direct costs)														
Maximum Research Project Period	Three years														
Total Funds Available	Up to \$6 million														
Eligibility	For this limited PFA, PCORI is soliciting applications from only CRNs or HPRNs that are currently funded to participate in PCORnet 2.0. Each CRN or HPRN is eligible to submit one LOI as the primary site.														
Review Criteria	1. Potential for the study to fill critical gaps in clinical comparative effectiveness evidence														



	<ol style="list-style-type: none"><li>2. Potential for the study findings to be adopted into clinical practice and improve delivery of care</li><li>3. Scientific merit (research design, analysis, data linkages, and outcomes)</li><li>4. Investigator(s) and environment</li><li>5. Patient-centeredness</li><li>6. Patient and stakeholder engagement</li></ol>
Contact Us	<p><b>Programmatic Inquiries:</b> Please contact the PCORI Helpdesk via email (<a href="mailto:sciencequestions@pcori.org">sciencequestions@pcori.org</a>), phone (202-627-1884), or online (<a href="http://www.pcori.org/PFA/inquiry">http://www.pcori.org/PFA/inquiry</a>). PCORI will respond within two business days; however, we cannot guarantee that we can address all questions in a timely fashion when the inquiry is made two or fewer business days before an LOI or application deadline. In light of the requirements for data linkage and collaborative support from other organizations, PCORI is especially interested in discussing proposals in advance with applicants and prospective collaborating entities.</p> <p><b>Administrative, Financial, or Technical Inquiries:</b> Please contact the PCORI Helpdesk at <a href="mailto:pfa@pcori.org">pfa@pcori.org</a>. PCORI will respond within two business days. Applicants may also call the PCORI Helpdesk at 202-627-1885. Please note that during the week of the application deadline, response times may exceed two business days. We ask that applicants plan accordingly. It is the applicant's responsibility to submit the application on or before the application deadline.</p>
Other	Deadlines are at 5 pm (ET). If deadlines fall on a weekend or a federal holiday, the deadline will be the following Monday or the next business day after the federal holiday.



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**What Has Changed since Cycle 2 2017 for Cycle 1 2019:**

- New PCORI Methodology Standard categories (Standards for Studies of Complex Interventions, Standards for Qualitative Methods, Standards for Mixed Methods Research, and Standards for Individual Participant-Level Data Meta-Analysis) have been added—see pages 8-9; the Methodology Standards Checklist has been updated to reflect these categories.
- New language has been added on PCORI’s Policy for Data Management and Data Sharing—see page 12.
- The Letter of Intent for this cycle is non-competitive but a requirement in order to submit an application.
- See the Application Guidelines for information about administrative and template changes.

## I. Introduction

### Background

To improve the United States' capacity to conduct clinical research more efficiently and to answer important questions that patients and clinicians face, the Patient-Centered Outcomes Research Institute (PCORI) provided \$105 million in 2014 to begin building the infrastructure for the National Patient-Centered Clinical Research Network (PCORnet). This large clinical research network represents people, patients, clinicians, systems, and health plans across the country and supports research that will improve health care and health outcomes.

Since 2013 PCORI has awarded more than \$417.5 million to create the infrastructure of a national, patient-centered research network. The network (i.e., PCORnet 2.0) currently includes nine clinical research networks (CRNs), two Health Plan Research Networks (HPRNs), a Coordinating Center, and a central headquarters. The PCORnet 2.0 headquarters evolved from a workgroup of researchers within PCORnet to further the network's sustainability model. The following elements are central to the rationale for and the sustainability of this network: preexisting, standardized, curated, and research-ready clinical data on large numbers of persons with specific clinical conditions and illnesses; actively engaged patients who join in governing the research uses of this data; distributed (rather than centralized) data that maximizes the security and local control of all data; a readiness among network members to collaborate and a willingness to share data in pursuit of worthy research aims; the capacity to link data across data sources at the individual patient level to create complete, longitudinal data; and the ability, ultimately, to attract research funding from a variety of funding sources, including federal agencies, industry sponsors, and not-for-profit foundations.

Having made the infrastructure investment, PCORI now intends to test the network's readiness. Specifically, this PCORI Funding Announcement (PFA) aims to test the readiness of the CRNs and HPRNs within PCORnet 2.0 to develop and lead competitive research projects that will depend on capacity in three critical areas: (1) the capacity of PCORnet 2.0's governance policies and study intake process to conceive and propose patient-driven clinical comparative effectiveness research (CER) questions and studies, including collection of relevant preparatory data; (2) the capacity to accomplish data linkages between data held by CRNs and data held by HPRNs and/or other sources that will enhance the proposed project's quality and strengthen PCORnet over the long term; and (3) the capacity to attract participation and support from non-PCORI research funders.

### Developing External Partnerships

PCORnet's value will ultimately depend on its ability to meet the research needs of a range of funders external to PCORnet and PCORI. The goal is a network that attracts a diverse set of public and private funders of research, finds ways to work with other networks and registries, and actively encourages collaboration with researchers not presently affiliated with PCORnet. Before submitting the full application, respondents to this PFA must collaborate with stakeholders external to PCORnet, including federal, industry, and other nonfederal co-funders (e.g., health plans, health systems, advocacy organizations), to secure at least 50 percent of direct project funding (which may include direct financial



contributions and in-kind support). Co-funding partnerships must demonstrate potential for additional future partnerships/collaboration with the network. Among comparably meritorious applicants PCORI will prioritize its funding for those that have obtained greater contributions and support from partner funding. Applications will require detailed Letters of Support from proposed co-funding partners to allow PCORI to evaluate the nature of the co-funding as well as the partners' awareness of and commitment to the study.

### **Advancing Data Integration**

In Phase II of PCORnet, PCORI encouraged networks to increase their access to more complete, accurate, and timely patient and organization data. Greater data completeness enhances the capture of relevant outcomes; in other cases, it may add details of specific procedures or treatments, disease severity, or the presence of comorbid illnesses. Data completeness can be enhanced in many ways, all of which require individual-level linkage of CRN or HPRN data with that from other sources. Examples of rich data sources include claims data from commercial insurers, singly or as already aggregated in several large consortia; Medicare or Medicaid data; comprehensive and timely mortality data; and detailed clinical and self-reported data contained in disease-specific registries, such as those built by specialty clinician organizations. This solicitation, therefore, requires that applicant CRNs and HPRNs describe the linkage(s) that they are including in the proposed study, how linkages will serve study needs, how linkages will be accomplished (e.g., using de-identified or identifiable data linkages), and approaches to Institutional Review Board (IRB) oversight of those linkages. Applicant CRNs and HPRNs are required to use the PCORnet 2.0 Common Data Linkage Method, if available.

### **Funds Available**

PCORI has allotted up to \$6 million in total costs under this PFA to fund up to two high-quality and impactful studies to answer important patient- and stakeholder-prioritized CER questions. The proposed budget for studies under this initiative may be up to \$2 million in direct costs, as appropriate. The maximum project period is three years; however, PCORI will consider exceptions to the research project period of three years (not to exceed four years) with justification during the application phase.

PCORI will not cover costs for usual patient care or for study interventions that constitute the procedures, treatments, interventions, or other standard care (patient care) under study. Provision of the interventional treatment or diagnostic test costs by an external collaborating organization would be considered a highly appropriate contribution and, if these costs are substantial, would satisfy requirements for an external partner. Host healthcare delivery systems, third-party payers, product manufacturers, intervention developers, other interested parties, or any combination of these sources could cover these costs, and by doing so would become partnered collaborators in the study. Covering only the routine ("usual") care costs and excluding the costs of one or both comparators would not be responsive or considered a contribution. In the case of multiple participating organizations (more than one CRN, collaborating HPRNs, or external collaborators), PCORI encourages the use of a single IRB of record for the research study. The Awardee Institution is responsible for the study, including oversight and dispersion of awarded funds to all subcontracts, including institutions from the CRNs, HPRNs, the Coordinating Center, and the People-Centered Research Foundation (PCRF). The [Application Guidelines](#) contain additional details for developing the research plan.



### Features of Patient-Centered Outcomes Research (PCOR)

PCOR is CER that helps people and their caregivers communicate and make better-informed, personalized healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research does the following:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, and palliative care to inform decision making, highlighting the choices that matter to people
- Focuses on outcomes that people notice and care about (including survival, functioning, symptoms, and health-related quality of life) and is inclusive of an individual's preferences, autonomy, and needs
- 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 or could soon become available in the clinical setting
- Obtains stakeholder perspectives to address the burdens to individuals, availability of services, and requirements for technology and personnel

### Characteristics and Objectives of Clinical Comparative Effectiveness Research

PCORI seeks to support new research that addresses critical clinical and health-related questions faced by patients, their caregivers, and their providers. PCORI seeks to fund stakeholder-driven, investigator-initiated CER that displays the following characteristics:

- The research measures and compares the benefits and harms of different interventions and strategies that are currently delivered in typical clinical and community settings for screening, diagnosis, treatment, or management of illnesses.
- The study demonstrates a clear potential to change practice or to reduce practice variation for the condition(s) under study.
- The research compares at least two alternative clinical approaches. Because PCORI's mission is to develop evidence to inform difficult decisions, we strongly prefer applications that propose to compare well-defined interventions that are already being used in the condition and the population of interest. In the case of newly approved treatments and technologies, acceptable studies may compare the new technology with the currently used technology for the same condition.
- The research examines such interventions as specific drugs, devices, procedures, assistive technologies, behavioral change, communication or dissemination, or complementary treatments; it may compare strategies for screening, diagnosing, treating, or managing illnesses. Studies may also address complex interventions that occur at or pertain to care delivery systems. Please note that "usual care" is not a sufficiently described comparator for CER studies submitted to PCORI. "Usual care" is too often ill defined; difficult to quantify; and subject to considerable geographic and temporal variations that limit interpretability, applicability, and

reproducibility. If the applicant proposes usual care as a rational and important comparator in the proposed study, then the actual care expected in the usual care arm must be described in detail in the proposal, must be coherent as a fully acceptable clinical alternative, and must be justified properly as the best legitimate comparator (e.g., usual care could be current guidelines-based care or current “optimal management”). The applicant must also include an explanation of how the care given in the usual care group will be measured in each patient, to the extent possible, during the study and the nature of inferences that will be appropriately drawn from such a comparative study.

- The research compares health outcomes that include those meaningful to the study’s patient population (e.g., morbidity, mortality, symptoms, functional status, quality of life, absenteeism from work or school). Such outcomes should be measured using validated methods. In select instances, surrogate physiological measurements may be sufficiently linked to final health outcomes to be of interest, but they might not be the sole study outcomes. Outcomes should be supported by careful involvement or consultation in study planning by affected patients—NOT necessarily by study participants.

## II. Guidance for Preparing Applications

### Specific Requirements

The proposed study should meet the following requirements:

- Focus on a clinical comparative effectiveness question that is important to patients and other decision makers and that has been formulated in consultation with patients and other stakeholders.
- Compare strategies for prevention, screening and diagnosis, treatment, or population management.
- Have endorsement from relevant patient organizations, clinician organizations, payer or purchaser consortia, and life sciences industry representatives that the study addresses a critical question—one that, if adequately answered, would substantially improve decision making.
- Submit a detailed letter from the co-funder(s) with the application. This letter must demonstrate a clear understanding of the project and a detailed description of the contributions, in terms of funding and in-kind services, including the estimated value of the contributions with details on how the value was calculated.
- Address an evidence gap in deciding among available options. Optimally, this gap should have been substantiated by an existing (recent or updated), rigorously conducted systematic review or emphasized by an official professional society’s clinical practice guideline.
- Demonstrate consultation with patients and other stakeholders or their representative groups, or reference previously documented decisional dilemmas, to determine if the study is answering a critical question—one that, if adequately answered, would substantially improve decision making.

- Propose a sample size that is sufficiently large to allow for precise estimation of hypothesized effect sizes or for clear demonstration of noninferiority. The sample size must also support testing of a priori hypotheses related to potential differences in effectiveness among relevant patient subgroups (i.e., Heterogeneity of Treatment Effect). Carefully explain in your application your choice for the proposed sample size and the consequences of that sample size.
- Examine diverse populations receiving care in real-world settings. Specify broad and simple eligibility criteria that allow for wide generalization of results while attending appropriately to ethical concerns of excess risk in some patient subgroups. Highly selected patient populations are not of interest.
- For studies aiming to reduce or eliminate health or healthcare disparities, specify one or more of the Addressing Disparities Program target populations (i.e., racial or ethnic minorities; low-income groups; residents of rural areas; individuals with special healthcare needs, including individuals with disabilities; individuals with low health literacy or numeracy, or limited English proficiency; and lesbian, gay, bisexual, transgender, and questioning [LGBTQ] persons) that will be the focus of the study. Studies should test the ability of interventions to improve outcomes (including patient-centered, clinical, and structural outcomes) and reduce disparities for at-risk populations.
- Demonstrate strong interest from and support of host delivery systems and clinical care settings when appropriate (i.e., when the intervention is delivered or data are collected within the system).
- Compare interventions that are known to be efficacious/effective or that are already in common use and that can be implemented in real-world settings.
- Feature near-term outcomes, including patient-reported outcomes (PROs), as primary outcomes, when appropriate.
- Plan to collect patient-centered outcome data efficiently and periodically during follow-up, as appropriate.
- Provide preliminary evidence of the potential for efficient recruitment, high participation rates, and appropriate oversight by local or centralized IRBs, including plans for streamlining or waiving individual informed consent in cases of low-risk interventions (if applicable). PCORI believes that the intensity of oversight and the complexity of informed consent procedures should be closely related to the degree of risk from study participation. Applicants must address this issue and present evidence that the study will not encounter significant barriers to approval or to recruitment and participation plans. The relevant IRBs make the final determination regarding the adequacy of informed-consent procedures and participant protections.
- Adhere to all applicable [PCORI Methodology Standards](#).<sup>1</sup> The full application will require the applicant to identify the standards appropriate to the proposed study and to describe how the

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<sup>1</sup> Available at <http://www.pcori.org/research-results/research-methodology>

study team plans to address each standard.

- In the case of randomized controlled trials (RCTs), adhere to current best practices (standardized inclusion or exclusion criteria; proper randomization; techniques to minimize potential for missing data; and appropriate safety monitoring, including establishing a Data and Safety Monitoring Board [DSMB] or indicating why such a board is unnecessary).

To carry out studies that allow for adoption of the findings in a real-world setting and to maximize the efficient use of resources, take care to prevent these trials from becoming more complex and onerous than necessary. We encourage the applicant to be creative and consider the following innovative strategies, as appropriate and feasible:

- Consult with patients and other stakeholders on their decisional dilemma and evidence needs, or reference previously documented decisional dilemmas in preparation for submitting LOIs and full applications.
- Carefully describe the pertinent evidence gaps and why the project questions represent decisional dilemmas for patients, caregivers, clinicians, policy makers, and other healthcare system stakeholders. Similarly, applicants should document why project outcomes are especially relevant and meaningful end points for patients and their families.
- Minimize disruption to participants' daily routines (e.g., minimize participant visits intended for study assessment purposes and capture PROs during office visits, electronically, or via phone).
- Design the study so that you can conduct it using routine clinic or office operations. Minimize disruption to clinical care when conducting portions of the study within clinical settings.
- Use efficient methods to obtain participant consent (e.g., electronic consent via a website or email) while still meeting ethical and legal requirements.
- Capitalize on existing electronic health records (EHRs) and other computerized information to identify and recruit eligible patients, monitor study conduct and patient safety, and collect study outcomes information.
- Take advantage of the data standardization and interoperability of PCORnet's Common Data Model (CDM) to the full extent possible. Point out any areas where the study could provide opportunities to expand or enhance standardization of electronic data across study sites.
- Identify and engage with major patient and other stakeholder organizations that would help disseminate and implement study findings.

### Non-responsiveness

Applications will be considered nonresponsive to this PFA if the proposed research does the following:

- Tests initial efficacy (or comparative efficacy) of interventions that are novel or have very limited prior evidence of efficacy. Applications should discuss current evidence on the efficacy and effectiveness of proposed interventions. New combinations of interventions known to be effective singly are acceptable.

- Involves studies conducted within tightly controlled research environments instead of in clinical settings reflective of real-world healthcare delivery
- Conducts a formal cost-effectiveness analysis
- Uses quality-adjusted life-years (QALYs) gained/lost to calculate estimates of value such as costs per QALY
- Conducts studies of the natural history of disease, instrument development, pharmacodynamics, and/or fundamental science or biological mechanisms
- Evaluates validity or efficacy of (rather than the clinical comparative effectiveness of) new or existing decision support tools. This includes the development and efficacy evaluation of decision support or shared decision tools or systems for patients, clinicians, or both.
- Simply develops clinical prediction or prognostication tools without evaluating the impact of their use on clinical outcomes
- Is a pilot study intended to inform larger efforts. This PFA is not soliciting pilot studies.
- Is a descriptive epidemiologic study. This PFA is not soliciting descriptive epidemiologic studies.
- Compares interventions for which the primary focus or the sole intervention is examining the role of compensated or volunteer community health workers, including patient navigators
- Has not received endorsement from relevant patient organizations, clinician organizations, payer or purchaser consortia, and life sciences industry representatives as potentially answering a critical question

Proposals may report use of any health services but may not employ direct measurements of care costs. For further information, please reference PCORI's [cost-effectiveness analysis FAQs](#).

PCORI does have an abiding interest, however, in studies that address questions about conditions leading to high costs to the individual or to society. This is included in our review criterion on the potential for research to fill a critical gap in knowledge or practice. As a result, PCORI is interested in studies that do the following:

- Examine the effect of costs on patients, such as patients' out-of-pocket costs, hardship or lost opportunity, or costs as a determinant of or a barrier to care access.
- Address cost-related issues, such as the resources needed to replicate or disseminate a successful intervention.
- Evaluate interventions to reduce health system waste or increase health system efficiency.

### Methodological Considerations

Regardless of study design, applications must adhere to all relevant [PCORI Methodology Standards](#).<sup>2</sup> These include 65 individual standards that fall into 16 categories. The first five categories are cross-

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<sup>2</sup> Available at <http://www.pcori.org/research-results/about-our-research/research-methodology/pcori-methodology-standards>

cutting and relevant to most PCOR studies. Researchers should refer to all of these standards when planning and conducting their research projects. These cross-cutting categories are:

1. Standards for Formulating Research Questions
2. Standards Associated with Patient-Centeredness
3. Standards on Data Integrity and Rigorous Analyses
4. Standards for Preventing and Handling Missing Data
5. Standards for Heterogeneity of Treatment Effect (HTE)

In addition to these five sets of standards, the first standard of “Standards for Causal Inference Methods”—(CI-1)—is cross-cutting and applicable to all PCOR studies.

The 11 other standards categories will be applicable to particular study designs and methods. Applicants should use the standards in each of these categories as guidance when they are relevant to a study. These categories are:

1. Standards for Data Registries
2. Standards for Data Networks as Research-Facilitating Structures
3. Standards for Causal Inference Methods
4. Standards for Adaptive and Bayesian Trial Designs
5. Standards for Studies of Medical Tests
6. Standards for Systematic Reviews
7. Standards on Research Designs Using Clusters
8. Standards for Studies of Complex Interventions
9. Standards for Qualitative Methods
10. Standards for Mixed Methods Research
11. Standards for Individual Participant-Level Data Meta-Analysis (IPD-MA)

Most of these standards are minimal. The PCORI Methodology Standards reflect practices that applicants should follow in all cases, and applicants must explain and justify all deviations. Applicants should address additional best practices—including relevant guidelines for conducting clinical trials developed by other organizations—in the application for PCORI funding. To help reviewers quickly identify adherence to a particular standard, applicants must cite each relevant [PCORI Methodology Standard](#) within the [PCORI Methodology Standards Checklist](#), following the instruction in the checklist itself and in the [Application Guidelines](#). Program staff use the checklist to evaluate applications.

Applicants should specifically discuss their capacity to measure such factors as differential adherence to chosen treatments (or participation in intervention programs) that could create or explain apparent differences in the effectiveness of the alternative interventions being compared in clinical populations.

## Patient-Centered Outcome Measures

PCORI encourages investigators to design their research using validated outcome measures. Include preliminary data that support using the proposed measures in the study population. We encourage investigators to consider those measures described in the [Patient-Reported Outcomes Measurement Information System](#).<sup>3</sup>

## Leveraging Existing Resources

PCORI encourages applicant CRNs and HPRNs to propose studies that leverage existing data resources within the CRNs, HPRNs, Patient-Powered Research Networks (PPRNs; if appropriate), or data sources external to PCORnet. These may include local or national registries of patients with the disease(s) under study. Registries built and managed by charitable foundations, disease advocacy organizations, physician specialty organizations, the life sciences industry, or the federal government might be of interest, as would Medicare or Medicaid data and vital statistics data of the Social Security Administration or the National Death Index. All linked sources are of interest to the extent that they can be shown to expand the study population's size or diversity or to enhance the capture of needed data on the study population. Examples include claims data from insurers or from the Centers for Medicare and Medicaid that capture outcomes, healthcare utilization, or vital status; EHR data that can add information on disease status and severity, comorbidity, laboratory, pathology, or other clinical information; and registry data that may add additional details of treatments or patient-reported data on outcomes, symptoms, or functional status. A key feature is that data must be linkable at the level of the individual patient or person. You must explain how you will accomplish this using the PCORnet 2.0 Common Data Linkage Method (if available) and provide clear evidence of the data partners' willingness and capacity to affect such linkages. Both direct linkages using identifiable information and anonymous linkages may be feasible, but you must state this distinction and explain how the linkages will meet patient consent requirements for the relevant overseeing IRB(s).

Applicants that propose use of data from CRNs, HPRNs, PPRNs (if appropriate), or data sources external to PCORnet should address the following in the Research Plan (as appropriate), with sufficient specificity:

- Identify and justify all participating research network entities (e.g., health plans, consortia projects, disease registries). For PCORnet, identify the names of participating CRNs, PPRNs, HPRNs, their affiliated study performance sites, and PCORnet Collaborative Research Groups (CRGs) that will be collaborating on the project.
- Demonstrate that the proposed data source(s) can comprehensively capture the study variables needed to assess the interventions, covariates, and outcomes.
- Describe how you will link and manage data across proposed study sites, research consortia, and/or other collaborating organizations and whether you will use any dedicated data-

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<sup>3</sup> Available at <http://www.nihpromis.org/>

coordinating functions or facilities. When feasible and relevant, the PCORnet Coordinating Center should meet data coordination needs.

- Provide a study management structure that identifies roles, responsibilities, and decision-making authority across the proposed research consortia.
- As applicable, provide a timeline for establishing data use agreements and linkages.
- Describe how the project will comply with PCORnet 2.0 governance policies.
- Describe all PCORnet and PCRF infrastructure resources used to conduct the study (e.g., Coordinating Center, streamlined IRBs, contracting, engagement and consenting processes, standardized data resources training).
- Indicate the experience of participating sites in the use of centralized versus localized IRBs.
- As applicable, document the involvement of partnered networks or entities in the study with detailed Letters of Support, clear budgets, and budget justifications that cover the costs of each network or entity's described contributions.
- Use the CDM to the full extent possible.

### Studies in Rare Diseases

PCORI is interested in the investigation of strategies that address care for patients with rare diseases. These conditions are defined as “life-threatening” or “chronically debilitating.” They are of such low prevalence (affecting fewer than 200,000 in the United States [i.e., less than one in 1,500 persons]) that special efforts—such as combining data across large populations—might be needed to address them.

### Patient and Stakeholder Engagement

PCORI encourages all applicants to outline how patients, non-PCORI funding partner(s), and other stakeholders (e.g., caregivers, clinicians, delivery systems, health plans) have participated in the planning of the application and will participate as partners in various phases of the proposed research. Before completing this section of the Research Strategy, we encourage you to review [PCORI's Engagement Rubric](#),<sup>4</sup> which can be found in the PCORI Funding Opportunities web page. Applicants should also review the PCORI Methodology Standards Associated with Patient-Centeredness. The rubric is not comprehensive or prescriptive; instead, it provides a variety of examples to incorporate engagement, where relevant, into the research process.

PCORI expects applicants to consult with patients and other stakeholders to identify and clarify the decisional dilemma and evidence needs that will be addressed in the proposed study. Alternatively, if decisional dilemmas have previously been identified, they may be referenced in preparation of the LOI and application. To describe the decisional dilemma, state the specific clinical decision(s), screening and diagnostic, or treatment choice(s) the decision makers face, and explain how the findings from the proposed research will inform those choices. State why this decision—such as choosing a specific medication, surgical approach, or care delivery strategy to treat a condition or manage a specific

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<sup>4</sup> Available at <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>



population—is important to patients in terms of potential benefits and risks, out-of-pocket costs, and preferences. Document the uncertainty patients and other stakeholders face when making this decision. Identify the patients and other stakeholders you consulted in planning the study, or reference the source of information that helped you identify the decisional dilemma. Applicants should document how the project outcomes chosen are especially relevant and meaningful for patients and other stakeholders.

### **Populations Studied**

PCORI seeks to fund research that includes populations diverse in age, gender, race, ethnicity, geography, and clinical status. PCORI recognizes that some proposed studies might represent important PCOR opportunities, even in the absence of a broadly diverse study population; however, the burden is on the applicant in such cases to justify the study’s importance in the absence of diversity. You should present and discuss preliminary data that show aspects of the diversity of the planned study population.

PCORI has an ongoing interest in including previously understudied populations for whom effectiveness information is needed, such as hard-to-reach populations or patients with multiple conditions. PCORI is also particularly interested in studying the possibly different impact of strategies in various subpopulations, with attention to the possibility that the strategy’s effects might differ across subgroups. PCORI has developed the following list of populations of interest to guide our research and engagement efforts:

- Racial and ethnic minority groups
- Low-income groups
- Women
- Children (age 0–17 years)
- Older adults (age 65 years and older)
- Residents of rural areas
- Individuals with special healthcare needs, including individuals with disabilities
- Individuals with multiple chronic diseases
- Individuals with rare diseases
- Individuals whose genetic makeup affects their medical outcomes
- Individuals with low health literacy, numeracy, or limited English proficiency
- LGBTQ persons
- Veterans and members of the Armed Forces and their families

### **Protection of Human Subjects**

This component (up to five pages) is in the Research Plan Template. Describe the protection of human subjects involved in your proposed research. 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 Section 5, titled “Human Subjects Research Policy,” in the [Supplemental Grant Application Instructions for All Competing Applications and Progress Reports](#),<sup>5</sup> which is issued by the US Department of Health and Human Services. PCORI does not require that applicants comply with sections of this policy that refer to requirements for federal wide assurance or that refer to standards for including women, minorities, and children. Awardees must also comply with appropriate state, local, and institutional regulations and guidelines pertaining to the use of human subjects in research.

PCORI requires awardees to ensure that there is a Data and Safety Monitoring Plan, which may include the need to appoint a DSMB, as provided in the [PCORI Policy on Data and Safety Monitoring Plans for PCORI-Funded Research](#).<sup>6</sup>

PCORI Merit Reviewers will examine plans for protection of human subjects in all applications and may provide comments regarding the plans (see [How to Evaluate Human Subjects Protections](#)<sup>7</sup>). Reviewers’ comments on human subject research are not reflected in the overall application score, but PCORI staff might use them during funding negotiations. Final determinations about the adequacy of human subject protections rest with the IRB or international equivalent that has jurisdiction for the study.

The Awardee Institution, whether domestic or foreign, 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 Research Participants**

PCORI requires that all applicants adhere to the National Institutes of Health (NIH) policy on education in the protection of human research participants in the conduct of research. This applies to all individuals listed as key personnel in the application. The policy and FAQs are available on the [NIH website](#).<sup>8</sup>

#### **Data Management and Data-Sharing Plan**

PCORI encourages openness in research and making research data available for purposes of replication and reproducibility. As such, if an award is made through this targeted PFA, then the awardee will be required to adhere to [PCORI’s Policy for Data Management and Data Sharing](#). The policy articulates PCORI’s requirement that certain awardees—specifically those funded through the Pragmatic Clinical Studies and all targeted PFAs, and PCORnet Research Funding Announcements—make the underlying data and/or specific data elements from their PCORI-funded research projects available to third-party requestors.

PCORI’s Policy for Data Management and Data Sharing recognizes that the distributed nature of PCORnet data may prohibit the deposition of the underlying data into a PCORI-designated repository. It does, however, state that awardees must deposit the following data elements into a PCORI-designated repository:

- Full protocol for specific research project

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<sup>5</sup> Available at <http://grants.nih.gov/sites/default/files/supplementalinstructions.docx>

<sup>6</sup> See <http://www.pcori.org/sites/default/files/PCORI-Policy-Data-Safety-Monitoring-Plans.pdf>

<sup>7</sup> See <http://www.pcori.org/sites/default/files/PCORI-Checklist-for-Evaluating-Human-Subjects-Protections.pdf/>

<sup>8</sup> Available at <http://grants.nih.gov/grants/guide/notice-files/NOT-OD-08-054.html>

- Description of the PCORnet CDM tables, including ancillary or ad hoc tables (if applicable)
- All codes used to query PCORnet data
- Aggregate level data set(s)
- Aggregate results of any new or research project–specific data quality investigations
- Results from research project–specific analytical queries

A full data management and data sharing plan is not required at the time of application. If an award is made, the awardee will be required to develop and maintain such a plan, which is described in detail in the [PCORI Methodology Standards for Data Integrity and Rigorous Analyses](#), specifically Standard IR-7. This plan must be appropriate for the nature of the research project and the types of research project data, and consistent with applicable privacy, confidentiality, and other legal requirements. Awardees are also strongly encouraged to include, as appropriate, language in the research project’s informed consent forms that allows for the de-identification and sharing of study data for secondary research purposes.

As part of the policy, PCORI intends to cover reasonable costs associated with the time and effort needed for preparing, depositing, and maintaining the data elements in the repository for a period of at least seven years following acceptance by PCORI of the Final Research Report. PCORI will negotiate with awardees on the specific budget needs associated with this policy requirement at the time of award, in addition to the requested research project budget.

The information here is meant for informational purposes only and does not attempt to be an exhaustive representation of the PCORI Policy for Data Management and Data Sharing. Please refer to the policy in its entirety for additional information.

### Recruitment

For studies that require recruitment of subjects, applications should include information about the size and representativeness of the potential recruitment pool of patients (across all contributing sites) and the means by which this size estimate was determined (e.g., EHRs, claims records, clinic logs, other administrative systems). Likewise, applications should provide evidence-based estimates of how many participants are ultimately expected in the study, based on expected recruitment applying the study’s inclusion and exclusion criteria; anticipated acceptance (or refusal) rates; other factors, such as loss to follow-up; and any preliminary data. Such estimates must be discussed in the application, specified in the milestones, reviewed by PCORI Merit Review Officers (MROs) and PCORI staff, and monitored by PCORI in the funded research.

### Peer Review and Release of Research Findings

PCORI has a legislative mandate to ensure the scientific integrity of the primary research it supports and to make study findings widely available and useful to patients, clinicians, and the general public within a specific timeframe. Accordingly, the PCORI Board of Governors (Board) adopted the [Process for Peer Review of Primary Research and Public Release of Research Findings](#).<sup>9</sup>

In summary, Awardee Institutions must submit to PCORI for peer review a draft final research report

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<sup>9</sup> See <http://www.pcori.org/sites/default/files/PCORI-Peer-Review-and-Release-of-Findings-Process.pdf>

that provides the methodological details, describes the main study results, and interprets the findings in clinical or other decisional contexts. Subject matter experts (SMEs); individuals with expertise in research methodology or biostatistics; and patients, caregivers, and other healthcare stakeholders will review the draft final research report. After Awardee Institutions have responded to reviewers' comments to PCORI's satisfaction, the report will be accepted and considered final. PCORI will then prepare a 500-word abstract summarizing the study results for patients and the general public, which the Awardee Institution will review and approve.

PCORI will post the following materials on its website no later than 90 days after it accepts the draft final research report: (1) a 500-word abstract for medical professionals; (2) a 500-word standardized abstract summarizing the study results for patients and the general public; (3) a link to the study record on [ClinicalTrials.gov](https://clinicaltrials.gov) (as applicable); and (4) ancillary information, including conflict-of-interest disclosures. The final research report, along with anonymized reviewer comments, will be made publicly available on the PCORI website no later than 12 months after its acceptance, except by prior mutual agreement with the Awardee Institution.

### III. How to Submit an Application

#### Noncompetitive Letter of Intent (LOI)

Applicants should download the [Cycle 1 2019 PaCR LOI Template](#) from the [PCORI Funding Opportunities](#) web page. They must complete the document and convert it to a PDF with a three-page limit, excluding references. PCORI suggests including all references as in-text citations using American Medical Association citation style, but we do accept other citation styles. A Letter of Support from the PCORnet 2.0 Steering Committee is also due with the LOI. Do not upload additional documents, such as Letters of Endorsement or Support, as part of your LOI because they are not requested at this stage. Please visit the [PCORI Funding Opportunities](#) web page for additional applicant resources, including FAQs and required templates.

Please answer all of the questions in the LOI template. This includes the question that asks for a brief justification for the study's proposed cost. Providing the answer "costs not to exceed \$2 million" is not sufficient. Upload your document to [PCORI Online](#). The deadline for LOI submission is April 15, 2019, by 5 pm (ET).

All applicants must submit an LOI by the April 15, 2019, deadline to be eligible to submit a full application. Please refer to the [Application Guidelines](#) on the [PCORI Funding Opportunities](#) web page for due dates and information on how to submit your LOI in [PCORI Online](#).

Because of the complex requirements for partnered funding and data linkage in this solicitation, PCORI staff are willing and available to discuss proposal ideas with potential applicants both before and after the LOI submission deadline. The appropriate staff person may be reached by emailing [sciencequestions@pcori.org](mailto:sciencequestions@pcori.org).

**Note:** A Principal Investigator (PI) can submit only one LOI per PFA; however, an individual listed as a PI on one LOI can be listed as and serve in another non-PI role (e.g., co-investigator or consultant) on other LOIs submitted under this limited PFA that do not address similar research topics and projects. This

applies to single- and dual-PI submissions. Similarly, each CRN or HPRN may be the lead PCORnet affiliation for one LOI; however, a CRN or HPRN can be listed and serve as a collaborating entity on additional LOIs.

### **Project Budget and Duration**

Applicants may request up to \$2 million in total direct costs for a research project period not to exceed three years (not including peer review). At the time of contract execution, PCORI sets aside all of the funds associated with an awarded project to be made available throughout the contract's period of performance. The maximum budget includes all research-related costs as well as costs associated with PCORI's peer-review process. (Please refer to the [Application Guidelines](#) for further details.) In general, PCORI will not cover the costs for interventions that are being compared in the proposed study. (See Appendix 2: Allowable and Unallowable Costs in the [Application Guidelines](#) for details.) Applicants should submit a realistic budget and timeline that reflects the proposed study's scope and requirements. PCORI will consider exceptions to the research project period of three years (not to exceed four years) with justification when submitting the application. PCORI will not consider exceptions to the budget limit of \$2 million in direct costs. However, PCORI will respond to an applicant's inquiry regarding budget-related issues during the period leading up to the submission deadline. Note that, although subcontractor indirect costs are included in the prime applicant's direct cost budget, PCORI does not factor in subcontractor indirect costs when determining adherence to the PFA's direct cost limit.

A contract is the funding mechanism for this program. A milestones and deliverables schedule, as well as specified recruitment targets, should be linked directly to and included in the proposed budget that will be subject to negotiation at the time of award. Some of the other activities that PCORI will consider during negotiations include the following:

- Developing a study protocol and procedure manual for the intervention
- Assigning roles and responsibilities to study team members for project implementation
- Forming an appropriate engagement body
- Providing a detailed task-based budget with level of effort for project staff, specified by task
- Obtaining clearances from all institutional and community partners, including IRB approvals
- Establishing a DSMB or providing a clear description of why one is unnecessary
- Executing all subcontractor agreements
- Agreeing on eligible patient populations for study recruitment
- Identifying barriers to patient recruitment in the study and addressing these barriers effectively
- Structuring a feasibility phase to demonstrate the potential for successful recruitment

Total project funding is contingent on successful programmatic and budget performance (e.g., meeting recruitment targets). Awardees must provide corroborating evidence to receive continuous funding support. Specifically, after 12 months, but no more than 18 months, of study performance, PCORI will

use information from the awardee to conduct a formal programmatic assessment of the study's progress and specified recruitment targets to determine its viability and sustainability. Only studies that PCORI deems satisfactory in this assessment will receive continuous funding support.

Refer to the [Application Guidelines](#)<sup>10</sup> for a list of additional project milestones specific to this PFA.

### Submission Dates

You must submit LOIs and applications in accordance with the published dates and times listed in the Overview section of this document and on the [PCORI Funding Opportunities web page](#).<sup>11</sup>

### PCORI Online System

To submit an application, you must register in [PCORI Online](#)<sup>12</sup> and submit an LOI and an application for each cycle to which you are applying.

### Applicant Resources

<b>PCORI Funding Opportunities</b>	<a href="https://www.pcori.org/funding-opportunities/announcement/limited-pcori-funding-announcement-partnerships-cycle-1-2019">https://www.pcori.org/funding-opportunities/announcement/limited-pcori-funding-announcement-partnerships-cycle-1-2019</a>
<b>PCORI Online</b>	<a href="https://pcori.force.com/engagement">https://pcori.force.com/engagement</a>
<b>PCORI Funding Awards</b>	<a href="http://www.pcori.org/research-results-home">http://www.pcori.org/research-results-home</a>

## IV. Merit Review

PCORI's merit review process is designed to support the following goals:

- Identify applications that have the strongest potential to help patients, caregivers, clinicians, policy makers, and other healthcare system stakeholders make informed decisions to improve patient outcomes.
- Implement a transparent, fair, objective, and consistent process to identify these applications.
- Elicit high-quality feedback that reflects a diversity of perspectives to ensure that the PCORI-funded research reflects the interests and views of patients and other stakeholders and those who care for them, and that it meets the criteria for scientific rigor.
- Fund projects that fill important evidence gaps and have strong implementation potential.
- Regularly evaluate and continually improve the merit review process and policies in support of PCORI's mission.

PCORI merit review is a multiphase process that includes PFA development; staff evaluation of LOIs; the review panel's preliminary review of full applications; an in-person panel discussion of a subset of full applications (identified by PCORI's Research Priority Area Program staff and based on the preliminary

<sup>10</sup> Available at <http://www.pcori.org/sites/default/files/PCORI-PFA-2017-Cycle-2-PaCR-Application-Guidelines.pdf>

<sup>11</sup> Available at <http://www.pcori.org/funding-opportunities>

<sup>12</sup> Available at <https://pcori.force.com/engagement>



review and program priorities); the PCORI Selection Committee’s recommendation of applications for funding; and, finally, Board award approval.

**Preliminary Review**

PCORI conducts rigorous merit review of the full applications it receives. Note that PCORI may eliminate applications from the review process for administrative or scientific reasons (e.g., non-responsiveness). PCORI may administratively withdraw an application if it is incomplete; submitted past the stated due date and time; or does not meet the formatting criteria outlined in the [Application Guidelines](#), in the PCORI templates, and in [PCORI Online](#). Applications that do not include partner funding (may include in-kind) from industry or organization collaborators will also be considered nonresponsive and administratively withdrawn. An application can be scientifically withdrawn if it is not responsive to the guidelines described in this PFA, describes research that is not comparative, includes a cost-effectiveness analysis, or otherwise does not meet PCORI programmatic requirements.

PCORI MROs recruit each panel based on the number and type of topic areas represented by submitted LOIs. MROs recruit the panel chair, scientist reviewers who are SMEs, patient representatives, and representatives of other stakeholder groups. All panel members receive training during the review cycle to ensure that they understand the programmatic and organizational goals of review.

We designed the table below to help applicants understand how the PCORI merit review criteria align with criteria from other funding organizations with which applicants might be familiar (e.g., NIH). Though PCORI’s criteria do map to most NIH criteria, there are areas where we ask for different information (i.e., PCORI does not include a criterion that tracks to NIH’s innovation criterion, but PCORI does include criteria evaluating patient-centeredness and engagement), reflecting PCORI’s unique approach.

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	
PATIENT-CENTEREDNESS/ENGAGEMENT	5. Patient-centeredness 6. Patient and stakeholder engagement

Below are PCORI’s merit review criteria. PCORI’s merit review panels use these criteria during the preliminary and in-person review phases to evaluate and score all submitted applications and to ensure consistency and fairness in application evaluation.

**Criterion 1. Potential for the study to fill critical gaps in evidence**

The application should address the following questions:

- Does the application convincingly describe the clinical burden?

- Does the application identify a critical gap in current knowledge as noted in systematic reviews, guideline development efforts, or previous research prioritizations?
- Does the application identify a critical gap in current knowledge, evidenced by inconsistency in clinical practice and decision making?
- Would research findings from the study have the potential to fill these evidence gaps?

**Criterion 2. Potential for the study findings to be adopted into clinical practice and improve delivery of care**

The application should describe how evidence generated from this study could be adopted into clinical practice and delivery of care by others. The application should also address the following questions:

- Does the application identify who will make the decision (i.e., the decision maker) or use (i.e., the end-user) the study findings (not the intervention) that this study produces, such as local and national stakeholders?
- Does the application identify potential end-users of study findings—such as local and national stakeholders—and describe strategies to engage these end-users?
- Does the application provide information that supports a demand for this kind of a study from end-users?
- Would this study’s research findings have the potential to inform decision making for key stakeholders? If so, provide an example. How likely is it that others could reproduce positive findings, resulting in improvements in practice and patient outcomes? Identify the potential barriers that could hinder others from adopting the intervention.
- Does the application describe a plan for how to disseminate study findings beyond publication in peer-reviewed journals and at national conferences?

**Criterion 3. Scientific merit (research design, analysis, data linkages, and outcomes)**

The application should show sufficient technical merit in the research design to ensure that the study goals will be met. The application should also address the following questions:

- Does the application describe a clear conceptual framework, anchored in background literature, that informs the design, key variables, and relationship between interventions and outcomes being tested?
- Does the Research Plan describe rigorous methods that demonstrate adherence to the PCORI Methodology Standards?
- Is the overall study design justified?
- Are the patient population and study setting appropriate for the proposed research question?
- Does the application provide justification that the outcome measures are validated and appropriate for the population?
- Are each of the described comparators (e.g., active intervention arm and comparator arm) clearly justified? If usual care is one of the arms, is it adequately justified and will it be sufficiently measured?



- Are the sample sizes and power estimates appropriate? Is the study design (e.g., cluster randomized design, RCT, or observational study) accounted for, and is the anticipated effect size adequately justified?
- Is the study plan feasible? Is the project timeline realistic, including specific scientific and engagement milestones? Is the strategy for recruiting participants feasible? Are assumptions about participant attrition realistic, and are plans to address patient or site attrition adequate? As applicable, does the application provide a justification for the research project period to extend beyond three years (not to exceed four years)?
- Does the application clearly describe data linkages using the PCORnet 2.0 Common Data Linkage Method (if available) between the required data sources (i.e., patient data, EHR data, claims data, and disease-specific registry data) to facilitate the conduct of the proposed study? Will the proposed data linkage work contribute to PCORnet methodologies?
- Does the proposed project provide an opportunity to utilize and enhance aspects of the PCORnet 2.0 infrastructure? Does the proposed project adhere to PCORnet 2.0 governance? Does the application describe the use of PCORnet and PCRF infrastructure resources (e.g., Coordinating Center, streamlined IRBs, contracting, engagement and consenting processes, standardized data resources training)?

#### **Criterion 4. Investigator(s) and environment**

This criterion should assess the appropriateness (i.e., qualifications and experience) of the investigator(s)/team and the environment's capacity (e.g., resources, facilities, equipment) to support the proposed project. It should not be an assessment of the institution's quality. The application should also address the following questions:

- How qualified are the PIs, collaborators, and other researchers to conduct the proposed activities? Is there evidence of sufficient clinical or statistical expertise (if applicable)?
- 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?
  - (Dual-PI option only) Does the Leadership Plan adequately describe and justify PI roles and areas of responsibility?
- 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**

The application should demonstrate that the study focuses on improving patient-centered outcomes and employs a patient-centered research design (i.e., a design informed or endorsed by patients). *(Note: The study can be patient centered even if the end-user is not the patient, as long as patients will benefit from the information.)* The application should also address the following questions:

- Does the application include a thorough description about which outcomes (both benefits and harms) are important to patients, and are those outcomes included in the study plan?
- Does the application show that closing the evidence gap is important to patients and other stakeholders?
- Are the interventions being compared in the study available to patients now, and are they the best options for comparison (including whether patients and their healthcare providers would choose them for managing the condition being studied)?

### **Criterion 6. Patient and stakeholder engagement**

The application should demonstrate the engagement of relevant patients and other stakeholders (e.g., patients, caregivers, clinicians, policy makers, hospitals and health systems, payers [insurance], purchasers [business], industry, researchers, training institutions, non-PCORI funding partner[s]) in the conduct of the study. Quality of engagement should be evaluated based on scope, form, and frequency of patient and stakeholder involvement throughout the research process. The application should also address the following questions:

- Does the application provide a well-justified description of how the research team incorporates stakeholder involvement? Does the study include the right individuals (e.g., researchers, patients, caregivers, clinicians, policy makers, other healthcare system stakeholders) to ensure that the project will be carried out successfully? Has the study received support from the PCORnet 2.0 Steering Committee?
- Does the application show evidence of active engagement among scientists, patients, and other stakeholders throughout the research process (e.g., formulating questions, identifying outcomes, monitoring the study, disseminating, implementing)? Is the frequency and level of patient and stakeholder involvement sufficient to support the study goals?
- Does the application demonstrate the potential for future partnerships/collaboration with the co-funder?
- Is the proposed Engagement Plan appropriate and tailored to the study?
- Are the roles and the decision-making authority of all study partners described clearly?
- Are the organizational structure and resources appropriate to engage patients and stakeholders throughout the project?

### **In-Person Review**

During preliminary review, PCORI evaluates and scores all administratively and scientifically compliant applications based on PCORI's merit review criteria, including evaluating adherence to the PCORI

Methodology Standards. After PCORI completes the preliminary review, PCORI program staff members evaluate panel scores and critiques to identify a subset of applications for merit reviewers to discuss at the in-person review meeting. Not all submitted applications move forward to in-person review.

During the in-person review, merit reviewers meet to discuss applications and to clarify the merits of the proposed research. They also identify areas for improvement. Each application is re-scored based on the content of discussion. The Panel Chair and PCORI MRO lead the in-person panel meeting and ensure that all applications receive a fair and thorough review according to the standards outlined in the PFA.

### Post-Panel Review

After the in-person meeting, PCORI program staff evaluate final merit review panel scores and comments, identify duplication or synergy among funded projects, and consider the fit of applications within the programmatic vision. Program staff members then recommend projects to a Selection Committee, which includes members of the Board. The Selection Committee considers recommendations and works with staff to identify a slate of applications for possible funding based on merit review scores, programmatic balance and fit, and PCORI's strategic priorities. This slate then goes to the Board for consideration and approval.

In addition, PCORI evaluates applicant risk before issuing an award. Factors considered include financial stability, quality of management systems, audit findings, and past performance on PCORI awards (e.g., compliance with PCORI reporting requirements, conformance to PCORI terms and conditions on previous awards, timely achievement of milestones). Based on the risk assessment, PCORI may impose special terms and conditions on awardees or withhold contract issuance until such business risks are mitigated. **PCORI will not award new contracts to current awardees with overdue reports (progress, interim, final, etc.) until the awardees have submitted the overdue reports.**

### Summary Statements and Funding Recommendations

Applicants receive summary statements approximately two weeks before PCORI announces the funding decisions. **If an application progresses to in-person discussion**, the applicant will receive a summary statement that includes the following:

- In-person panel discussion notes
- Final average overall score
- Preliminary reviewer critiques

Summary statements for applications that do not progress to in-person discussion include only the preliminary reviewer critiques.

PCORI makes funding recommendations by identifying meritorious applications that fit the programmatic needs and that satisfactorily address the merit review criteria while adhering to the PCORI Methodology Standards. PCORI also considers the funds allotted for the current PFA when deciding which applications to recommend to the Board for approval. Applicants to this current cycle's PFA will receive summary statements and notification of the funding status of their applications no later than November 2019.



### **Contract Execution and Activation**

PCORI will issue a contract to the selected Awardee Institutions for the study once it conducts a thorough programmatic and administrative review. Awardees must accept 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-on 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 research project plan.