Cycle 2 2018 Funding Cycle

PCORI Funding Announcement:
Psychosocial Interventions with Office-Based Opioid Treatment (OBOT) for Opioid Use Disorder

Published June 1, 2018

This PCORI Funding Announcement (PFA) applies to the funding cycle that closes October 25, 2018, at 5 p.m. (ET). Application Guidelines, templates, and other resources are available at https://www.pcori.org/funding-opportunities/announcement/psychosocial-interventions-office-based-opioid-treatment-opioid.
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.”

Patient-Centered Outcomes Research Institute
1828 L St., NW, Suite 900
Washington, DC 20036
Phone: 202-827-7700
Fax: 202-355-9558
Email: info@pcori.org

Follow us on Twitter: @PCORI
Overview

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<th>Published</th>
<th>June 1, 2018</th>
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<tr>
<td>Letter of Intent Due</td>
<td>June 28, 2018, by 5 p.m. (ET)</td>
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The Patient-Centered Outcomes Research Institute (PCORI) will screen Letters of Intent (LOIs) for responsiveness to this PCORI Funding Announcement (PFA) and for fit to program goals. Only those applicants with an approved LOI will be permitted to submit a full application. Notification of denial or approval to submit a full application will occur no later than July 23, 2018.

Summary

The Patient-Centered Outcomes Research Institute (PCORI) seeks to fund high-quality comparative effectiveness research on psychosocial interventions for patients with Opioid Use Disorder (OUD) who receive Office-Based Opioid Treatment (OBOT). PCORI is particularly interested in receiving applications that focus on or include urban, low-income, and racial-ethnic minority populations. PCORI is interested in stepped care approaches, with a preference for step-down models. Studies may compare different levels of intensity (e.g., different frequencies, group vs individual format, combinations of interventions), and different durations of efficacious interventions. Each proposed comparator must be clearly defined, evidence-based or in use, and available. Standard medical management includes buprenorphine delivery and brief office visits during which such things as buprenorphine effects, patient concerns, and recent drug use may be discussed. Studies must include a minimum follow-up period of one year from baseline. In addition, all studies funded through this initiative must include robust sample sizes with sufficient power demonstrated to conduct proposed primary analyses and subgroups as appropriate.

For this solicitation, applicants are not required to demonstrate that patients and other stakeholders are already engaged as research team members at the time an application is submitted. However, applicants should outline how patients and other stakeholders will participate as partners in various phases of the proposed research, once awarded. Applicants should describe their plan to form a Study Advisory Committee (SAC), or other appropriate engagement body, to ensure that a broad spectrum of patients and other stakeholders advise and assist the research team with refining the study questions, outcomes, and protocols. These patients and other stakeholders must include national or regional organizations that represent—at a minimum—patients, caregivers, clinicians, policy makers, and other healthcare system stakeholders. PCORI may recommend additional representation in collaboration with the applicant, including individual patients with lived experience and other relevant stakeholders, such as scientific and methodological experts.

Note that this funding program does not support applications in conducting cost-
effectiveness analyses, systematic reviews (with or without meta-analyses), or developing or conducting efficacy evaluation of shared decision making. PCORI will not cover costs for interventions that are being compared in the proposed study. (See Appendix 3: Administrative Actions in the Psychosocial Interventions OBOT Application Guidelines for details.)

### Applicant Resources


### Key Dates

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<thead>
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<th>Event</th>
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<tr>
<td>Online System Opens</td>
<td>June 1, 2018</td>
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<tr>
<td>LOI Town Hall</td>
<td>June 13, 2018 at 12pm (ET)</td>
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<td>LOI Deadline</td>
<td>June 28, 2018, by 5 p.m. (ET)</td>
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<tr>
<td>LOI Status Notification</td>
<td>July 23, 2018</td>
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<td>Applicant Town Hall</td>
<td>August 8, 2018 at 12pm (ET)</td>
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<tr>
<td>Application Deadline</td>
<td>September 25, 2018, by 5 p.m. (ET)</td>
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<tr>
<td>Merit Review</td>
<td>December 2018</td>
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<td>Awards Announced</td>
<td>April 2019</td>
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<tr>
<td>Earliest Project Start Date</td>
<td>June 2019</td>
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### Maximum Project Budget (Direct Costs)

$4 million

### Maximum Research Project Period

Four years

### Funds Available Up to

$25 million

### Eligibility

Any private-sector research organization, including any nonprofit or for-profit organization; any public-sector research organization, including any university or college hospital or healthcare system; any laboratory or manufacturer; or unit of local, state, or federal government may submit applications. The Internal Revenue Service must recognize all U.S. applicant organizations. Nondomestic components of organizations based in the U.S. and foreign organizations may apply, as long as there is demonstrable benefit to the U.S. healthcare system and U.S. efforts in the area of patient-centered research can be shown clearly. Organizations may submit multiple applications for funding. Individuals are not permitted to apply.

### Review Criteria

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

### Contact Us

**Programmatic Inquiries:** Please contact the PCORI Helpdesk via email (sciencequestions@pcori.org), phone (202-627-1884), or online (http://www.pcori.org/PFA/inquiry). 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 three or fewer business days before an LOI or application deadline.
| Administrative, Financial, or Technical Inquiries: | Please contact the PCORI Helpdesk at pfa@pcori.org. 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. |
| 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. |

New or Revised for the Cycle 2 2018 Funding Cycle:
- Added a new Methodology Standard category (Standards for Studies of Complex Interventions)—see pages 9-10
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I. Introduction

Summary of Program

The Patient-Centered Outcomes Research Institute (PCORI) is issuing this funding initiative to support patient-centered comparative effectiveness research (CER) on psychosocial interventions for patients with opioid use disorder (OUD) who receive office-based opioid treatment (OBOT). Due to ongoing concern about the opioid epidemic, PCORI leadership remains committed to supporting high-quality research in this area. Through this PCORI Funding Announcement (PFA), PCORI seeks to fund studies with sufficient sample sizes to address the priority research question:

- **What is the comparative effectiveness of psychosocial interventions versus standard medical management for patients who receive office-based opioid treatment with buprenorphine?**

The goal of the PFA is to generate valid evidence that is readily generalizable to the broader population of patients with OUD. Study designs to be considered include large randomized controlled trials (RCTs) or well-justified observational studies. PCORI is particularly interested in receiving applications that focus on or include urban, low-income, and racial/ethnic minority populations. PCORI is also especially interested in large studies that can investigate potential heterogeneity of treatment effect (HTE) regarding important sociodemographic and clinical characteristics (e.g., addiction severity, socioeconomic status, racial or ethnic status, comorbid mental health conditions, or other clinical or patient/demographic characteristics proposed by investigators with an accompanying strong rationale).

Applicants should follow the *Diagnostic and Statistical Manual of Mental Disorders* definition of opioid use disorder: “a problematic pattern of opioid use leading to clinically significant impairment or distress.”

There is concern that the term “medication-assisted treatment” (MAT) suggests that medication is merely an ancillary part of the treatment rather than a central component. In this PFA, the term MAT is used because it is well defined and broadly used and recognized, and to distinguish it from approaches that focus on abstinence. MAT is essential to the standard management of patients with opioid use disorder. Studies will be considered nonresponsive if they do not include maintenance medication.

Applications must address the priority research question described in this PFA. Proposed studies should compare two or more approaches that include interventions that are documented to be efficacious or in common use, and that are well characterized to facilitate replication and dissemination efforts.

Proposed interventions must be evidence based or in current use. If applicants propose interventions that are in common use but without clear evidence of efficacy, they must document the extensiveness of their use. Proposed comparators must be adequately defined and described as to how they will be measured. Studies should include interventions that are or can be made be available to most patients.

Standard medical management consists of buprenorphine and brief office visits during which buprenorphine effects and side effects, recent drug use, and patient concerns may be discussed.

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Standard medical management should be clearly described and adequately justified. Outcomes should include treatment adherence/retention, patient function, illicit opioid use, other drug use, emergency department visits, overdose, provider satisfaction, and provider stress. Studies should conduct periodic outcome assessments and include at least one year of patient follow-up.

Background

Illicit and prescription opioid abuse and addiction have increased dramatically in the past decade. In 2016, 11.8 million people ages 12 or older in the United States misused opioids, and 2.1 million people had an opioid use disorder. Heroin use, partly due to reduced access to prescription opioids for patients with OUD, has increased in populations with traditionally low rates of heroin use, including women, people with private insurance, and high-income individuals.

OUD is associated with increased morbidity and mortality, decreased quality of life, and multiple other negative outcomes for the individual, his or her family, communities, and society at large. The rate of overdose deaths involving opioids has increased dramatically between 1999 and 2016, doubling in the past 10 years and quadrupling in the past 16, with 42,249 reported opioid overdose deaths in 2016. Along with increases in opioid addiction and overdose deaths, there has been a rise in the incidence of neonatal abstinence syndrome, and in the prevalence of infectious diseases including HIV and hepatitis C. The Council of Economic Advisers estimates that the economic cost of the US opioid crisis amounted to $504 billion in 2015, nearly 3 percent of GDP that year.

Medication-assisted treatment, in which maintenance medication (methadone or buprenorphine) is combined with psychosocial services, is an evidence-based, clinically effective treatment for patients with OUD. Psychosocial services may consist of addiction counseling, contingency management, cognitive behavioral therapy, 12-step programs, community reinforcement approach, or other approaches.

Until 2000, MAT was offered only in federally regulated Opioid Treatment Programs (OTPs). OTPs (“methadone clinics”) are typically located in urban areas, which, combined with the daily attendance requirement to allow for observed ingestion of the medication, creates significant access challenges for many patients. Buprenorphine is a partial agonist with a lower risk of overdose due to its ceiling effect on respiration. In an effort to increase access to MAT, the Drug Abuse Treatment Act of 2000 allowed buprenorphine to be prescribed by physicians outside of OTPs following training (OBOT). More recently, physicians’ assistants and nurse practitioners are also eligible to prescribe buprenorphine.

OTPs are required by federal law to provide substance abuse counseling by licensed counselors to patients receiving MAT, and the use of 12-step or other mutual-help groups is encouraged. Clinicians offering OBOT are required to offer or refer patients to evidence-based psychosocial services. However, evidence regarding psychosocial services, including counseling, as part of MAT is mixed, with some studies and systematic reviews suggesting improvements in various outcomes, and others suggesting no benefits. Consequently, guidelines recommend evidence-based interventions, but do not specify which interventions.

Psychosocial interventions that have demonstrated efficacy or are in use with either methadone or buprenorphine include Contingency Management (CM), Cognitive Behavioral Therapy (CBT), Community Reinforcement Approach (CRA), and other approaches.
Reinforcement Approach, family therapy, Motivational Interviewing, relapse prevention, self-help groups, telephonic patient support, and substance abuse counseling. However, current evidence regarding their effectiveness and their comparative effectiveness is conflicting. Given the size of the opioid epidemic and the substantial nationwide efforts to increase patient access to OBOT, it is imperative to assess which interventions at which intensity are best for different patients.

Evidence Gaps

The current evidence regarding psychosocial services as part of OBOT with buprenorphine is mixed. Overall, systematic reviews and meta-analyses offer conflicting evidence regarding the benefits of psychosocial services for patients in buprenorphine maintenance treatment. However, many individual studies show positive effects for at least one outcome.

- A meta-analysis by Dutra et al.\(^2\) reported that across 34 controlled trials, psychosocial treatments do appear to provide benefit in the treatment of substance use disorders. The two interventions found to have the strongest evidence were CM and CBT.

- Two Cochrane reviews produced conflicting results for psychosocial interventions with methadone or buprenorphine. The first review included 11 studies that assessed four psychosocial interventions (CM, community reinforcement, counseling, and family therapy), and concluded that psychosocial treatments increase treatment retention and reduce illicit opioid use, but that the evidence did not support one psychosocial approach over others (Amato et al.\(^3\)). However, the second review, which included 35 studies and tested 13 psychosocial interventions, concluded that psychosocial treatments do not affect treatment retention, illicit opioid use, or psychiatric or depressive symptoms. Individual studies evaluated five behavioral interventions, five psychotherapy interventions, and three counseling interventions (Amato et al.\(^4\)). However, the authors noted that control conditions frequently included counseling sessions as part of standard medical management, which may have obscured any effects of the tested interventions.

- Carroll and Weiss\(^5\) reviewed eight randomized controlled trials that tested the efficacy of psychosocial interventions for patients in buprenorphine maintenance treatment specifically. All studies included relatively large sample sizes, manualized interventions, and biological indicators of drug use outcomes. Results were mixed, with four trials reporting no effect of manualized drug counseling, CM, or CBT, and four trials providing evidence that counseling, community-reinforcement approaches, CM, and CBT increased opioid-free urine samples and treatment. These authors also suggest that the relatively intensive medical management in the control conditions in the negative studies may have obscured effects of the psychosocial interventions.

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Dugosh et al.\textsuperscript{6} included eight studies focused on buprenorphine in a review of psychosocial services offered in conjunction with medications for the treatment of OUD. The interventions included community reinforcement and family training, CBT, intensive role induction, CM, telephonic patient support system, general drug counseling, and level of care (intensive outpatient treatment versus outpatient treatment). Five of the eight studies reported significant effects for community reinforcement and family training, CBT, intensive role induction, and a telephonic patient support system on outcomes including treatment attendance and drug use. The authors concluded that, in general, psychosocial services improve outcomes for patients in maintenance treatment, although the evidence is less robust for buprenorphine than for methadone. These authors also suggest that medication management may have been more intensive than it would be in a real-world setting and may have obscured any effects.

Schwartz\textsuperscript{7} offers a criticism of the Dugosh et al.\textsuperscript{5} review, arguing that the authors’ broad inclusion of all psychosocial treatments and outcomes obfuscated the review’s findings, and that the most appropriate conclusion based on existing evidence is that there is no added benefit of psychosocial treatment offered in conjunction with agonist medications. Furthermore, he asserts that providing agonist medications only when they can be offered with psychosocial treatment limits the use of this effective intervention.

However, Dugosh et al.,\textsuperscript{8} in their response to this commentary, emphasized that the majority of individual studies demonstrate efficacy of psychosocial interventions for at least one outcome. The authors conclude that there is a clear need for additional research on which combinations of medications and psychosocial treatments work best for which patients.

In summary, it is not clear for which patients buprenorphine with standard medical management is sufficient, and which patients should be offered enhanced medical management and/or additional psychosocial interventions, and which additional interventions these patients should be offered. Evidence regarding psychosocial interventions as part of OBOT with buprenorphine is mixed, and several authors of systematic reviews have suggested that conflicting findings may be the result of “enhanced” medical management in control groups. Large comparative effectiveness studies can answer the important questions regarding which patients should be referred to which psychosocial interventions at which intensities in order to optimize their chance of success.

**Priority Research Question**

PCORI seeks to fund high-quality studies that address this PFA’s research question:

- What is the comparative effectiveness of psychosocial interventions versus standard medical management for patients who receive office-based opioid treatment with buprenorphine?


Studies should propose interventions that are or can be made be available to most patients. PCORI is especially interested in stepped-care and needs-based approaches rather than straightforward comparisons of interventions. Studies may include comparisons of different levels of intensity (e.g., different frequencies or durations, combinations of interventions) and format (group versus individual format) of efficacious interventions. Studies may include comparisons of teledelivery or web-based delivery and in-person delivery for interventions that have evidence of efficacy with remote delivery, but this comparison should not be the sole focus of the proposal.

**Important Safety Considerations**

Awardees must plan to have specific and adequate human subjects protection measures in place (e.g., inclusion of a data safety monitoring board, a risk monitoring plan, and discussion of potential risk and how it will be monitored in the consent process).

**Funds Available**

PCORI has allotted up to $25 million in total costs under this PFA to fund up to four high-quality and high-impact studies related to psychosocial interventions for patients receiving buprenorphine for OUD. The proposed budget for studies under this initiative may be up to $4 million in direct costs as appropriate. Requested budgets should be appropriately scaled to the actual size and scope of the proposed project. The maximum project period is four years.

PCORI will not cover costs for the procedures, treatments, interventions, or other standard clinical care (“patient care”) that an applicant is proposing for comparison in the research project (“patient care costs”). The host healthcare delivery system, third-party payer, product manufacturer, intervention developer, or other interested party must cover the patient care costs.

PCORI seeks efficient studies, such as those that take advantage of large populations already under observation, natural experiments, and research cooperatives, and the supportive involvement of delivery systems or health plans to enhance recruitment, data collection, and coverage of intervention-related costs. A prolonged recruitment period is not an acceptable rationale for longer studies. Funding requests with the primary purpose of developing or building on initial collaboration between researchers and patient or stakeholder groups are also not appropriate for this PFA.

**II. Guidance for Preparing Applications**

**Specific Requirements**

The proposed study should strive to meet the following requirements:

- Focus on a comparative clinical effectiveness question that is important to patients and other decision makers.
- Address an evidence gap in deciding among available options; 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 non-inferiority. The sample size must also support testing of *a priori* hypotheses related to potential differences in effectiveness among relevant patient subgroups (HTE).
- Examine diverse populations receiving care in real-world settings.
- Have strong interest from and support of host delivery systems and clinical care settings.
- Specify broad and simple eligibility criteria that will allow for wide generalization of results while attending appropriately to ethical concerns of excess risk in some patient subgroups.
- Compare interventions that are known to be efficacious, effective, or commonly used, and that can be implemented in real-world settings.
- Feature near-term outcomes and PROs as primary outcomes, when appropriate.
- Plan to collect patient-centered outcome data efficiently and periodically during follow-up.
- Provide preliminary evidence of the potential for efficient recruitment, high participation rates, and appropriate oversight by local or centralized Institutional Review Boards (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 recruitment or participation barriers. The relevant IRBs make the final determination of the adequacy of informed-consent procedures and participant protections.
- Adhere to all applicable PCORI Methodology Standards. The full application will require the applicant to identify the standards appropriate to the proposed study and to describe how the study team plans to address each standard.
- In the case of RCTs, also 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.
• 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.

• Use efficient methods to obtain participant consent while still meeting ethical and legal requirements.

• Capitalize on the 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. PCORI specifically encourages applications that use the National Patient-Centered Clinical Research Network (PCORnet) infrastructure.

• If data standardization and interoperability across study sites have not already been accomplished, develop methods that will enhance the standardization of data that are accessed from different EHR systems.

### Project Budget and Duration

Applicants may request up to $4 million in total direct costs for a research project period not to exceed 4 years (not including peer review). At the time of contract execution, PCORI sets aside all funds associated with an awarded project to be made available throughout the contract’s period of performance. The maximum budget includes all research and peer-review-related costs. (Please refer to the Application Guidelines for further details.) In general, PCORI will not cover costs for interventions that are being compared in the proposed study. (Appendix 2: Allowable and Unallowable Costs in the Application Guidelines for details.) Applicants should submit a realistic budget and timeline reflecting the proposed study’s scope and requirements. In some rare circumstances, the estimated budget may exceed $4 million total direct costs, depending on the nature of the research question, the design and analytical requirements of the proposed study, the expected size of the patient enrollment, or the complexity and frequency of the outcomes assessment. PCORI expects these to be selective cases, which include high-priority topics that are of greatest interest to us. Applicants who intend to propose such studies must provide evidence of prior approval by PCORI scientific staff to exceed the budgetary limit and succinct justifications in their LOI, documenting the budget requirements with respect to the scope of the proposed research and the data-collection and analysis efforts. Please note that this justification counts toward the LOI three-page limit. PCORI will deny any request for a project period longer than 4 years. Note that although subcontractor indirect costs are included in the prime applicant’s direct-cost budget, subcontractor indirect costs are not factored 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 will be considered during negotiations include:

• Developing a study protocol and procedure manual for the intervention

• Assigning roles and responsibilities to study team members for project implementation
• Forming a SAC or other 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 upon successful programmatic and budget performance (e.g., meeting recruitment targets). Awardees must provide corroborating evidence to receive continuous funding support. Specifically, after 12 months of study performance, but no later than 18 months, PCORI will use information from the awardee to conduct a formal programmatic assessment of the study’s progress and specified recruitment targets to determine the study’s viability and sustainability. Only studies that are deemed satisfactory in this assessment will receive continuous funding support.

Refer to the Application Guidelines for a list of additional project milestones specific to this PFA.

**Non-responsiveness**

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

• Proposes a study with a sample size of less than 300 patients.
• Tests efficacy (or comparative efficacy) of interventions.
• 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.
• Directly compares the costs of care between two or more alternative approaches to providing care.
• Conducts studies of the natural history of disease, instrument development, pharmacodynamics, and fundamental science or biological mechanisms.
• Evaluates validity or efficacy of (rather than the 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.
• Develops clinical prediction or prognostication tools.
• Establishes efficacy for a new clinical strategy.
• Proposes a pilot study intended to inform larger efforts.
• Compares patient characteristics rather than clinical strategy options.
• 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. PCORI will consider interventions focused on CHWs and patient or peer navigators if they are part of a larger multicomponent intervention, are integrated with multidisciplinary primary care teams, or are compared with other non-personnel-based efficacious interventions.

Proposals may report use of any or all health services, but may not employ direct measurements of care costs. For further information, please reference our cost-effectiveness analysis FAQs.

PCORI does have an interest, however, in studies addressing 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:
• 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 barrier to access to care.
• 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.

Avoiding Redundancy
PCORI encourages potential applicants to review funded research at pcori.org. We intend to balance our funded portfolio to achieve synergy and avoid redundancy where possible.

Methodological Considerations
Regardless of study design, applications must adhere to all relevant PCORI Methodology Standards. These include 54 individual standards that fall into 13 categories. The first five categories are cross-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 eight 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 Infrastructures
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

Most of these standards are minimal. The PCORI Methodology Standards reflect practices that applicants should follow in all cases, and all deviations need to be explained and justified. 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 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 valid patient-centered outcome measures. Include preliminary data that support using the proposed measures in the study population. We also encourage investigators to consider those measures described in the Patient-Reported Outcomes Measurement Information System (PROMIS). 9

Leveraging Existing Resources

PCORI encourages investigators to propose studies that leverage existing resources, such as adding PCOR to an existing large clinical trial or analyzing existing large databases that contain valuable and relevant information that can be used to answer important CER questions. PCORI is interested in studies that leverage existing research networks or consortia that would facilitate the conduct of large, multi-site studies called for in this PFA.

Applicants proposing use of an existing research network infrastructure (e.g., PCORnet); research consortia; or related data resources (e.g., patient outcomes registries and/or electronic medical record [EMR] data from healthcare delivery systems or administrative claims data from public or commercial insurers) 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 and members, etc.) or, in the case of PCORnet, identify the names of the participating Clinical Data Research Networks (CDRNs), Patient-Powered Research Networks (PPRNs), and

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9 Available at http://www.nihpromis.org/.
PCORnet Collaborative Research Groups) that will be collaborating on the project. Also, identify the affiliated study performance sites.

- Demonstrate that the data source can comprehensively capture the study variables needed to assess the interventions, covariates, and outcomes.

- Describe how you will manage data across study sites within the research network or the proposed research consortia, and whether you will use any dedicated data-coordinating functions or facilities.

- As applicable, demonstrate familiarity with the existing network governance policies or data-use restrictions. 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.

- As applicable, describe the network infrastructure resource(s) used to conduct the study (i.e., core research-support facilities, streamlined IRBs, contracting, engagement and consenting processes, standardized data resources training, etc.). Indicate the percentage of sites that have previously used centralized versus localized IRBs.

- As applicable, provide documentation supporting the involvement of network leadership throughout the study (e.g., detailed Letters of Support, budgets, and Budget Justifications that cover the costs of the network’s efforts). You can obtain Letters of Support from PCORnet from the Coordinating Center by submitting a request via the PCORnet Front Door.

Collaboration

PCORI is particularly interested in applications involving community and commercial organizations that can help researchers design, implement, disseminate, and sustain effective interventions. We encourage applications that include novel collaborations with accreditation organizations, credentialing bodies, educational enterprises, patient advocacy groups, industry, professional societies, and subspecialty societies.

Patient and Stakeholder Engagement

PCORI encourages all applicants to outline how patients and other stakeholders will participate as partners in various phases of the proposed research. Before completing this section of the Research Strategy, we encourage to review PCORI’s Engagement Rubric, which can be found in the PCORI Funding Opportunities. Applicants should also review the PCORI Methodology Standards Associated with Patient-Centerededness, and PCORI’s Sample Engagement Plans. Additionally, the Engagement in Health Research Literature Explorer10, a searchable, catalogued resource for peer-reviewed literature, can help identify publications about engagement that are specifically relevant to your work. These resources are not intended to be comprehensive or prescriptive; instead, they provide a variety of examples to incorporate engagement, where relevant, into the research process.

PCORI expects applicants to consult with patients and other stakeholders on their decisional dilemma

10 Available at http://www.pcori.org/literature/engagement-literature
and evidence needs, or to reference previously documented decisional dilemmas in preparation for the submission of LOIs and applications. To describe the decisional dilemma, state the specific clinical decision(s) or treatment choice(s) the decision makers confront and explain how the findings from the proposed research will inform those decisions. State why this decision—such as choosing a specific medication, surgical approach, or care delivery strategy to treat a condition or manage a specific population—is important to patients. Document the uncertainty patients and other stakeholders face when making this decision. Identify the patients and other stakeholders you consulted when determining that the proposed study addresses their evidentiary needs for decision making, and indicate your commitment to continue engaging them actively in the study. Similarly, applicants should document how the project outcomes are especially relevant and meaningful endpoints for patients and other stakeholders.

For this PFA, applicants are not required to demonstrate that patients and other stakeholders are already engaged as research team members at the time an application is submitted. However, the Engagement Plan should outline how patients and other stakeholders will participate as partners in various phases of the proposed research, once awarded. Applicants should describe their plan to form a Study Advisory Committee (SAC), or other appropriate engagement body, to ensure that a broad spectrum of patients and other stakeholders advise and assist the research team with refining the study questions, outcomes, and protocols. These patients and other stakeholders must include national or regional organizations that represent—at a minimum—patients, caregivers, clinicians, policy makers, and other healthcare system stakeholders. Additional representation may be recommended in collaboration with PCORI, including individual patients with lived experience and other relevant stakeholders, such as scientific and methodological experts. The SAC or other appropriate engagement body should meet in person at least two times per year, and the budget should account for these engagement costs.

PCORI understands that engagement structures and approaches vary widely. Other engagement approaches, such as forming stakeholder groups, panels, task forces, working groups and other bodies, or involving individual patient and other stakeholder partners in various ways, are also permissible to employ—either in addition to or instead of—the formation of the SAC. For clarification in your application materials and for merit review purposes, please indicate which body or structure is filling the SAC requirements, including the requirements for in-person meetings at least two times per year and appropriate budgeting.

**Populations Studied**

PCORI seeks to fund research that includes diverse populations with respect to 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.

Alternatively, PCORI is particularly interested in including previously understudied populations for whom effectiveness information is needed, such as hard-to-reach populations or patients with multiple conditions. Thus, comparisons should examine the impact of strategies in various subpopulations, with attention to the possibility that the strategy’s effects might differ across subgroups. Populations of
interest include those that are less frequently studied. 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
- Lesbian, gay, bisexual, transgender, and questioning (LGBTQ) persons
- Veterans and members of the Armed Forces and their families

Protection of Human Subjects

This component (up to five pages) is included 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,11 which is issued by the U.S. Department of Health and Human Services (HHS). In referencing the HHS Supplemental Grant Application Instructions, note that PCORI does not require that applicants comply with sections of that policy that refer to requirements for federal-wide assurance and the inclusion of women, minorities, and children in the proposed studies. Instead PCORI expects applicants to address diversity in study participants in the research plan, through a focus on subpopulations, as described in the above section on ‘Populations Studied.’ 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.

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). Reviewers’

11 See http://grants.nih.gov/sites/default/files-supplementalinstructions.docx
comments on human subject research are not reflected in the overall application score, but PCORI staff might use them during potential 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.12

**Data Management and Data-Sharing Plan**

PCORI encourages openness in research and making research data available for purposes of replication and reproducibility. Although not required to be submitted as a component of the research application, if an award is made, the awardee must develop and maintain a plan addressing data management and data sharing of research project data. This must be done in a manner that is appropriate for the research project and the types of research project data, and in a manner consistent with applicable privacy, confidentiality, and other legal requirements.

**Recruitment**

Applications should include information about the size and representativeness of the potential recruitment pool of patients and the means by which this size estimate was determined (e.g., EMRs, claims records, clinic logs, or 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; and other factors, such as loss to follow-up. Such estimates must be discussed in the application, specified in the milestones, reviewed by 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 time frame. Accordingly, the Board adopted the Process for Peer Review of Primary Research and Public Release of Research Findings.

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

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prepare a 500-word standardized 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 the earlier of 13 months after the Final Progress Report OR 90 days from the date the Final Research Report is approved by PCORI: (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 (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

Letter of Intent (LOI)

Applicants should download the Psychosocial Services OBOT LOI Template from the PCORI Funding Opportunities. 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. Do not upload additional documents as part of your LOI, such as Letters of Endorsement or Support, because they are not requested at this stage. Their inclusion will result in LOI rejection without review. Please visit the PCORI Funding Opportunities for additional applicant resources, including FAQs and required templates.

Please answer all of the questions in the LOI Template. This includes the question on brief justification for the proposed cost of the study. Providing the answer, “costs not to exceed $4 million in direct costs” is not sufficient. Upload your document to PCORI Online. The deadline for LOI submission is June 28, 2018, by 5 p.m. (ET).

LOI Review

PCORI evaluates LOIs based on the following criteria:

- Whether the topics are related to those on PCORI’s own priority list, versus the IOM/AHRQ lists, versus other topics initiated by investigators
- Importance to current clinical decision making, as evidenced by critical gaps identified by clinical guidelines developers or recent relevant systematic reviews
- A size or scope sufficient to have a significant impact on patient outcomes or healthcare practices
- Clarity and credibility of applicants’ responses to the LOI questions, as well as their justification of the need for a large pragmatic study—including the rationale for the estimated sample size—citing published estimates, including effect sizes, standard deviations, and the need for rigorous comparative analysis of important subgroups
- Prior relevant experience
- Programmatic fit and balance, considering whether the application significantly overlaps with
concurrent applications or previously funded studies or, conversely, whether the application fills a gap in PCORI’s portfolio, considering such characteristics as disease category, topics, priority population, and methodologies

- Adherence to the administrative and formatting requirements listed in the Application Guidelines, especially the three-page limit for the LOI

LOIs are reviewed qualitatively; they are not scored. Only applicants whose LOIs are deemed most responsive to this PFA will be invited to submit a full application. At least two PCORI staff review LOIs, and they are not scored during review. Notification of denial or approval to submit a full application will occur no later than July 23, 2018. Please refer to the Application Guidelines in the PCORI Funding Opportunities for due dates and information on how to submit your LOI in PCORI Online.

All applicants are required to submit an LOI for PCORI staff to review. This allows PCORI to determine whether proposed revisions and changes made to specific aims or methodological approaches from the original applications align with our evolving strategic priorities.

If you are invited to submit an application, do not make significant changes to your proposed project without consulting a program officer. For example, you should not revise your major aims and study design. Any significant changes are grounds for removal from the review process.

**Note:** A Principal Investigator (PI) can only submit 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 within the same PFA during the same cycle. A PI may submit multiple LOIs to different program PFAs in a cycle, but the PI must ensure that the research topics and projects are not similar. If a PI submits an LOI to multiple program PFAs, LOIs that exhibit scientific overlap or that appear to be duplicate submissions will be disqualified. PCORI will contact the PI and provide him or her with an opportunity to choose the PFA to which he or she would like to apply. This applies to single and dual-PI submissions.

**Submission Dates**

LOIs and applications must be submitted in accordance with the published dates and times listed in the Overview section of this document and in the PCORI Funding Opportunities.

**PCORI Online System**

To submit an application, you must register in PCORI Online and submit an LOI and an application for each cycle to which you are applying.

**Applicant Resources**


- **PCORI Online System** [https://pcori.force.com/engagement](https://pcori.force.com/engagement)

- **PCORI Funding Awards** [http://www.pcori.org/research-results-home](http://www.pcori.org/research-results-home)
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 review and program priorities); the 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). An application may be administratively withdrawn 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. 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 Merit Review Officers (MROs) recruit each review panel based on the number of invited LOIs and topic areas represented by the invited 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 does include criteria evaluating patient-centeredness and engagement) reflecting PCORI’s unique approach.

Crosswalk of PCORI Merit Review Criteria with NIH Criteria

PCORI Cycle 2 2018 Psychosocial Services OBOT PFA
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) 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, 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, which 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 comparators (e.g., active intervention arm and comparator arm) described 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?

**Criterion 4. Investigator(s) and environment**
This criterion should assess the appropriateness (e.g., qualifications and experience) of the investigator(s)/team and the environment’s capacity (e.g., resources, facilities, and 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 provide information that indicates 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, and training institutions) 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 and other healthcare system stakeholders) to ensure that the projects will be carried out successfully?
- 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, and implementing)? Is the frequency and level of patient and stakeholder involvement sufficient to support the study goals?
- 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, all administratively and scientifically compliant applications are evaluated and scored by panels of external reviewers based on PCORI’s merit review criteria, including evaluation of adherence to the PCORI Methodology Standards. After 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.

In-Person Applicant Presentation

Based on the results of the merit review and PCORI’s programmatic priorities, PCORI invites a selective subset of applicants whose proposed studies are deemed to be highly meritorious or aligned with PCORI’s strategic priorities to participate in follow-up discussions with PCORI on study methodological and execution issues. We expect applicants to address concerns and critiques identified in the merit review in this presentation. We will notify the selected applicants of the logistics for this presentation (including travel arrangements) in separate communications.

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, and 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 funding decisions are
announced. If an application progresses to in-person discussion, the applicant will receive a summary statement that includes:

- In-person panel discussion notes
- Final average overall score
- Preliminary reviewer critiques
- Application quartile, to help applicants understand how they did relative to other discussed applications, as appropriate

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 application no later than April 2019.