PCORI Funding Announcement:
Management of Care Transitions for Emerging Adults with Sickle Cell Disease

Published August 15, 2016
Updated May 19, 2017

This PCORI Funding Announcement (PFA) applies to the funding cycle that closes on December 19, 2016, at 5 p.m. (ET). Application Guidelines, templates, and other resources are available at http://www.pcori.org/Cycle-3-2016-Sickle-Cell-Disease.
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, purchasers, 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.”
Overview

Published: August 15, 2016
Updated: May 19, 2017

Letter of Intent Due: September 14, 2016 by 5 p.m. (ET)

Letters of Intent (LOIs) will be screened for responsiveness to this PCORI Funding Announcement (PFA) and for fit to program goals. Only those applicants selected will be permitted to submit full applications. Notification of denial or approval to submit a full application will occur no later than October 21, 2016.

Summary

**PCORI Research Question:**
What is the comparative effectiveness of established transition coordination models for emerging adults with Sickle Cell Disease transitioning from pediatric to adult care?

PCORI seeks to fund comparative clinical effectiveness research (CER) that evaluates and compares two or more evidence-based or widely used transition coordination models for emerging adults with Sickle Cell Disease (SCD) transitioning from pediatric to adult care. PCORI is interested in randomized controlled trials (RCTs) comparing interventions with proven efficacy with emerging adults with SCD or related populations.

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. 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.

*Note that this funding program does not support applications to conduct cost-effectiveness analysis, systematic reviews, or development and evaluation of shared decision-making or decision-support tools.*

The proposed studies must address the priority research question identified in the main body of the PFA.

Applicant Resources:
See [http://www.pcori.org/Cycle-3-2016-sickle-cell-disease](http://www.pcori.org/Cycle-3-2016-sickle-cell-disease)

Key Dates:
- **Online System Opens:** August 15, 2016
- ** Applicant Town Hall Session:** July 21, 2016
- **LOI Deadline:** September 14, 2016, by 5 p.m. (ET)
- **LOI Status Notification:** October 21, 2016
- **Application Deadline:** December 19, 2016, by 5 p.m. (ET)

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*The intent of the SAC described in the PFA is 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. 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. The SAC provision is not meant to require that a separate governance or advisory entity be established beyond the study governance and advisory structure the awardee has planned, if an applicant already has an approach for including the relevant and required patient and other stakeholder partners. For clarification in your application materials and merit review purposes, please indicate which body or structure is fulfilling the SAC requirements, including the requirements for in-person meetings at least two times per year, and appropriate budgeting.*

PCORI Cycle 3 2016 Funding Announcement: Management of Care Transitions for Emerging Adults with Sickle Cell Disease
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<thead>
<tr>
<th><strong>Merit Review Dates:</strong></th>
<th>March 2017</th>
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<tr>
<td><strong>Awards Announced:</strong></td>
<td>September 12, 2017</td>
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<tr>
<td><strong>Earliest Project Start Date:</strong></td>
<td>November 2017</td>
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<tr>
<th><strong>Maximum Project Budget (Total Costs)</strong></th>
<th>$8.33 million</th>
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<tr>
<td><strong>Maximum Project Period</strong></td>
<td>Five years</td>
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<td><strong>Funds Available Up To</strong></td>
<td>$25 million</td>
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<th><strong>Eligibility</strong></th>
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<tr>
<td>Applications may be submitted by 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 any unit of local, state, or federal government. The Internal Revenue Service must recognize all U.S. applicant organizations. Nondomestic components of organizations based in the United States 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 clearly shown. Organizations may submit multiple applications for funding. Individuals are not permitted to apply.</td>
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<tr>
<th><strong>Review Criteria</strong></th>
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<tbody>
<tr>
<td>1. Potential for the study to fill critical gaps in evidence</td>
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<td>2. Potential for the study findings to be adopted into clinical practice and improve delivery of care</td>
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<td>3. Scientific merit (research design, analysis, and outcomes)</td>
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<td>4. Investigator(s) and environment</td>
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<td>5. Patient-centeredness</td>
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<td>6. Patient and stakeholder engagement</td>
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<th><strong>Contact Us</strong></th>
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<tr>
<td><strong>Programmatic Inquiries:</strong> Contact the PCORI Helpdesk via email (<a href="mailto:sciencequestions@pcori.org">sciencequestions@pcori.org</a>) or phone (202-627-1884), or complete the Research Inquiry Form (<a href="http://www.pcori.org/content/research-inquiry">http://www.pcori.org/content/research-inquiry</a>). PCORI will provide a response within three business days. However, we cannot guarantee that all questions will be addressed in a timely fashion when the inquiry is made three or fewer business days before an LOI or application deadline.</td>
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| **Administrative, Financial, or Technical Inquiries:** Contact the PCORI Helpdesk at pfa@pcori.org. PCORI will provide a response within two business days. Note that during the week of the application deadline, response times may exceed two business days. One week before an application deadline, applicants may also call the PCORI Helpdesk (202-627-1885). Applicants are asked to plan accordingly. It is the applicant’s responsibility to submit the application on or before the application deadline. |

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<th><strong>Other</strong></th>
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<tr>
<td>*Deadlines are at 5 p.m. (ET). If a deadline falls on a weekend or federal holiday, the deadline will be the following Monday or the next day after the federal holiday.</td>
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I. Introduction

Summary

The Patient-Centered Outcomes Research Institute (PCORI) is launching this funding initiative to support patient-centered comparative clinical effectiveness research (CER) comparing transition coordination models that focus on the transition from pediatric to adult care for emerging adults (between the ages of 16–25 years old) with Sickle Cell Disease (SCD). This life-changing active transition can be extremely difficult for individuals living with SCD due to the increase in disease and healthcare-related complications that often arise in this population during this vulnerable time transitioning to adulthood. Through this PCORI Funding Announcement (PFA), PCORI seeks to fund comparative effectiveness trials to improve quality of care and outcomes for individuals with SCD who are transitioning from pediatric care to adult care. Studies should be scientifically sound and demonstrate appropriate engagement of patients, caregivers, clinicians, and other important stakeholders. This PFA’s goal is to fill current evidence gaps and generate valid clinical knowledge for emerging adults with SCD to ensure a healthy and high-quality care transition with the best possible patient outcomes.

Background

SCD is a chronic genetic disorder affecting the body’s red blood cells. It is estimated that between 70,000 and 100,000 Americans—predominantly African Americans—have SCD. These individuals are concentrated primarily in the Southern and Eastern regions of the United States (See Figure 1). This disorder induces a series of disease-related complications, including acute chest syndrome, stroke, and pain crises. Although severe complications may begin at the onset of disease in early infancy (5–6 months of age), advancements in multidisciplinary pediatric care have made it possible for nearly all children with SCD to achieve adulthood. Despite these improvements in care, the average life span of individuals living with SCD remains relatively short compared to the U.S. life expectancy, ranging between 36 and 53 years for men, and 39 and 56 years for women.9

Because more children with SCD are now living into adulthood, the healthcare system has not kept pace, shifting the burden of SCD-related morbidity and mortality to emerging adults. The emerging adult...
population, defined as those between the ages of 16–25 years old, remains particularly vulnerable to poor health outcomes during the time of transition from pediatric to adult care. This transition is a process that includes the purposeful and planned movement of adolescents and young adults from a child-oriented to adult-oriented healthcare setting. Although there is little empirical evidence about the optimal age at which to begin the transition process, a majority of SCD patients transition between the ages of 16–18 years old, and most emerging adults transition by age 26. Additional transition activities may occur beyond the age of 26 years old, closer to the age of 30 years old, as they relate to the transition of insurance coverage.

This is a life-changing transition and, in many ways, a continuous rather than a discrete process with a high risk of care discontinuity and disengagement from the healthcare system. It is different from care transition models in traditional settings, such as those from the hospital to home. Many SCD patients lose the usual source of care from their pediatric providers, resulting in fragmented or loss of routine preventive and screening visits to SCD clinics or primary care doctors for treatments such as hydroxyurea, vaccines, and blood transfusions—all of which are imperative to managing their disease. As a result, patients are more likely to seek care for acute medical events in the emergency department. By the time patients seek care for acute events, they are at an increased risk for complications (e.g., infections or stroke) or death. Many factors, such as the high proportion of SCD patients on public insurance; limited access to specialists; and poor coordination between pediatric and adult providers (e.g., poor transfer of disease- and patient-specific knowledge) further exacerbate the challenges related to the transition from pediatric to adult care.

Although mortality rates remain relatively constant through the age of 15 years old, there is an approximate seven-fold increase in mortality rates during the transition period. This can be attributed to a number of factors, including high rates of comorbid conditions (e.g., asthma, restrictive lung disease, and cardiac and renal dysfunction), cumulative disease effects, and changes in utilization of health care. This population also experiences high rates of hospitalization (3.62 per year versus 1.93 in other SCD age groups) and re-hospitalization (41% re-hospitalized within 30 days versus 23% in other SCD age groups). Furthermore, impairments due to disease-related complications (e.g., strokes); high prevalence of depression (26% versus 9.5% in the general population); and high rates of school and work absences (average of 38.4 missed school days per year) are all contributing factors that make this population vulnerable.

Current guidelines for transitions from pediatric to adult care are sparse or are based on weak evidence or consensus-based opinion. In addition, there are no current CER trials on care transitions for individuals living with SCD. A limited number of studies have evaluated transition coordination interventions in patients with diabetes and congenital heart disease, with very few randomized controlled trials (RCTs). Among these studies, existing evidence supports the efficacy of educational programs, co-located pediatric and adult providers, transition coordinators, and young adult clinics.

Research Question

The topic and research question within this PFA were prioritized with input from a diverse group of stakeholders during a workshop on March 7, 2016. The meeting was open to the public via teleconference, with slides and meeting materials posted on the PCORI website. For more information
About the workgroup, please see the event page: http://www.pcori.org/events/2016/prioritizing-comparative-effectiveness-research-questions-management-sickle-cell-disease.

Through this PFA, PCORI is interested in funding strong proposals that focus on the following research question:

**What is the comparative effectiveness of established transition coordination models for emerging adults with Sickle Cell Disease transitioning from pediatric to adult care?**

Proposals submitted in response to this PFA should focus on patient-centered comparative effectiveness trials with direct comparisons of two or more evidence-based interventions, as outlined below. The table below provides specific guidelines in developing proposals.

| Target Population | • Emerging adults (16–25 years old) with SCD  
|                   | o Age of transition should take into account the individual’s developmental maturation, physical abilities, disease severity and control, and environment.  
|                   | o If an intervention proposes to address issues related to insurance factors for emerging adults during transition, an upper age range of 30 years might be considered acceptable with appropriate justification. |
| Interventions/Comparators | • Examples of transition coordination interventions may include, but are not limited to:  
|                   | o Co-located pediatric and adult care providers (e.g., two providers in the same facility)  
|                   | o Virtual consultation (telehealth) with a provider or specialist  
|                   | o Clinic-based transition coordinator (specialized position residing within clinics to provide coordination and expertise for a smooth transition)  
|                   | o Other patient-facing interventions (e.g., mHealth, mobile applications, or text messaging) with strong rationale, shown efficacy, and generalizability  
|                   | • Proposed interventions should have proven efficacy and effectiveness in other childhood-onset chronic diseases (e.g., congenital heart disease and diabetes). Applicability of this evidence to the SCD population must be explained and justified.  
|                   | • “Usual care” or “standard of care” may be an appropriate comparator, but must be justified, well-defined, and sufficiently measured.  
|                   | • Interventions in widespread use also may be an appropriate comparator, with adequate evidence and justification. |
| Outcomes | • Measures of patient-reported and health service utilization outcomes, in addition to relevant clinical outcomes, should be included. Potential outcomes may include, but are not limited to:  
|           | o Health-related quality of life (HRQOL) indicators  
|           | o Satisfaction and experiences of care  
|           | o Social functioning (e.g., missed days from work or school)  
|           | o Number of hospitalizations and number of days hospitalized due to SCD or
SCD complications, such as pain crisis
  - Measures of emergency department use

Additional outcomes may include valid indicators or measures of a successful transition.

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<th>Timing</th>
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<td>Maximum allotted time for any individual study is five years.</td>
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<tr>
<td>Studies should allow adequate time for implementation of interventions and a thorough follow-up to determine adherence to, or improvements in, patient outcomes derived from the subject intervention(s).</td>
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<tr>
<th>Setting</th>
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<tr>
<td>Outpatient settings such as (but not limited to) primary care practices, federally qualified health centers, patient-centered medical homes, and specialty clinics</td>
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<tr>
<th>Study Design</th>
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<tr>
<td>Multi-site, cluster RCT</td>
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<td>Based on existing research, evidence, and stakeholder input, PCORI encourages applications using a cluster RCT study design. An individual RCT might be an acceptable study design if adequate rationale, justification, and feasibility information are provided.</td>
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Although not required, applicants are encouraged to leverage existing SCD networks, cohorts, and consortia (e.g., the National Heart, Lung, and Blood Institute [NHLBI] and the National Patient-Centered Clinical Research Network [PCORnet]) to the extent possible. Applicants should describe the participating collaborators, the benefit of the collaboration, and the roles of all collaborators.

**Collaboration Across Funded Trials**

PCORI is expecting to fund up to three clinical trials within this PFA. As such, PCORI will expect collaboration between the three project teams, including but not limited to in-person or teleconference meetings to discuss recruitment challenges and collection of common measures. Thus, PCORI requires a statement from each research team addressing willingness to collaborate.

The nature and scope of any in-person meetings remain to be defined; therefore, applicants are asked not to include travel costs for these meetings within their budget. If awarded, PCORI will establish a separate cost center to cover these expenses. Awardees will submit separate invoices to PCORI for these meetings, which will be reimbursed to the awardee at cost.

**Funds Available**

PCORI has allotted up to $25 million in total costs under this PFA to fund high-impact studies related to the Management of Care Transitions for Emerging Adults with SCD. The proposed budget for any individual study under this initiative may go up to $8.33 million in total costs (including indirect costs), as appropriate. The maximum project period is five years.

For this solicitation, PCORI is not requiring that relevant national patient organizations, professional organizations, or payer or purchaser organizations be formally included as partners and active participants prior to contract award. However, applicants should document that they have consulted with patients and other stakeholders to identify the important decisional dilemmas and evidence needs, or they should refer to previously documented decisional dilemmas. Successful applicants are required
to work in collaboration with PCORI staff upon award of the proposed studies to establish a project Study Advisory Committee (SAC) or other appropriate engagement body, which is comprised of national or regional organizations that represent—at a minimum—patients or families with lived experience, relevant clinicians, payers, and health plans. Additional representation may be recommended in collaboration with PCORI, including individual patients with lived experience and other relevant stakeholders, among them scientific and methodological experts. The SAC advises and assists the research team with further refinement of the study questions, outcomes, and protocol.

Given the significant treatment costs associated with some interventions, the applications must address—in the context of the proposed studies—the support from payers, health plans, industry sponsors, or others in covering the study interventions and non-study protocol-related clinical costs and services rendered in the care processes. Of particular concern would be different levels of co-payment between two arms in a comparative study. Ideally, cost-sharing barriers should be eliminated or equalized in the study arms. If the study design does not allow for either option, the applicant should describe why and should also discuss how differences in co-payment costs will be accounted for in the analysis of the study’s findings.

It is expected that project budgets and duration will vary substantially, depending on the topic and approach selected, needs for recruitment or primary data collection, length of follow-up, and analytic complexity. PCORI seeks efficient studies, such as those that take advantage of large populations already under observation, registries, and the supportive involvement of delivery systems or health plans to enhance recruitment, data collection, and coverage of treatment-related costs. A prolonged recruitment period is not an acceptable rationale for longer studies. Funding requests to develop or build on initial collaboration between researchers and patient/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 effectiveness question that is important to patients and other decision makers.
- Conduct direct comparisons of efficacious transition coordination interventions inclusive of patients, caregivers, and clinicians.
  - Because there is limited evidence available regarding effective transition interventions for emerging adults with SCD, proposed interventions with proven efficacy or effectiveness in other childhood-onset chronic diseases (e.g., congenital heart disease and diabetes) may be proposed with justification.\(^{20-23}\) If proposing a multi-component intervention, applicants should describe the existing evidence of efficacy for individual components, if it does not exist for the grouping of the components. Applicants should provide a convincing explanation for the relevance of the interventions compared in the proposed study, citing evidence gaps that are justified on the basis of up-to-date
literature reviews. PCORI is interested in interventions with established efficacy; thus, interventions that develop, test, and validate new interventions will be considered out of scope.

- If proposing to compare to “usual care” or “standard of care,” it must be described in detail, coherent as a clinical alternative, and properly justified as a legitimate comparator (e.g., “usual care” is guidelines-based). It must also be accompanied by an explanation of how the care given in the “usual care” or “standard of care” group will be measured in each individual patient, and how appropriate inferences will be drawn from its inclusion.

- Demonstrate consultation with patients and other stakeholders or their representative groups.
- Receive endorsement by relevant patient organizations, clinician organizations, payer or purchaser consortia, and life-sciences industry representatives as potentially answering a critical question—one that, if adequately answered, would substantially improve decision making.
- Propose the number of clusters and/or a sample size that is sufficiently large to allow for precise estimation of hypothesized effect sizes. The sample size must also support testing of *a priori* hypotheses related to potential differences in effectiveness among relevant patient subgroups (Heterogeneity of Treatment Effect, or 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 wide generalization of results, while attending appropriately to ethical concerns of excess risk in some patient subgroups.
- As applicable, compare interventions that are known to be efficacious, effective, or commonly used and that can be implemented in real-world settings.
- Include patient-reported outcomes (PROs) as primary outcomes, when appropriate.
- 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 barriers to recruitment or participation. The relevant IRBs make the final determination of the adequacy of informed consent procedures and participant protections. Applicants must address this issue and should present evidence that the study will not encounter significant barriers to recruitment or participation. Applicants should also carefully consider and provide details supporting how the target sample size will be met across all study sites (e.g., expected eligible patient panels, recruitment capacity, integration of research and clinical workflow, etc.).
• Adhere to all applicable PCORI Methodology Standards The full application will require the applicant to identify the standards appropriate to the proposed study and 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 clinical studies, readily adopt the findings in a real-world setting, and maximize the efficient use of resources, applicants must prevent these trials from becoming more complex and onerous than necessary. PCORI encourages the applicant to be creative and consider the following innovative strategies, as appropriate and feasible:

• Be prepared to identify and engage with major patient and stakeholder organizations that would implement study findings, as well as with existing local communities of patients and care providers to refine the research questions and study protocol, help monitor progress, and disseminate the findings.

• Consult with patients and other stakeholders on their decisional dilemma and evidence needs, or reference previously documented decisional dilemmas in preparation for submitting Letters of Intent (LOIs) and the full applications.

• Describe carefully 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 endpoints for patients and other stakeholders. Minimize disruption to participants’ daily routines (e.g., minimize participant visits intended for study-assessment purposes; capture PROs during office visits, electronically, or by phone).

• Design the study so that the conduct can integrate with routine clinic or office operations as seamlessly as possible.

• Use efficient methods to obtain participant consent 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.

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

**Nonresponsiveness**

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

• Tests efficacy (or comparative efficacy) within a tight, protocol-controlled research setting (as
opposed to more real-world and pragmatic CER)

- Conducts a cost-effectiveness analysis in the form of dollar-cost per quality-adjusted life-year to compare two or more alternatives
- Directly compares the care costs between two or more alternative approaches
- Measures the relative care costs of two or more alternative approaches as the primary criteria for choosing the preferred alternative
- Conducts studies of the natural history of disease, instrument development, pharmacodynamics, and fundamental science of 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 patients and clinicians
- Develops clinical prediction or prognostication tools

Applications that include studies of these issues may measure and report utilization of any or all health services, but may not employ direct measurements of care costs.

PCORI does have an interest, however, in studying conditions that lead to high costs to the individual or to society. Thus, PCORI is also interested in studies that:

- 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

Applications that include studies of these issues without using cost-effectiveness analyses or comparing the costs of alternatives are considered responsive.

Furthermore, PCORI discourages applications in the following categories and is likely to deem them nonresponsive:

- Study of the natural history of disease
- Instrument development
- Pharmacodynamics
- Fundamental science or study of biological mechanisms
- Establishing efficacy for a new clinical strategy
- Pilot studies intended to inform larger efforts
- Comparisons of patient characteristics rather than clinical strategy options

**Features of Patient-Centered Outcomes Research**

Patient-centered outcomes research (PCOR) helps people and their caregivers communicate and make
informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, or palliative care to inform decision making, highlighting the choices that matter to people
- Is inclusive of an individual’s preferences, autonomy and needs, focusing on outcomes that people notice and care about (including survival, functioning, symptoms, and HRQOL)
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination
- Directly compares clinical interventions that are generally available in the clinical settings
- Obtains stakeholder perspectives to address the burdens to individuals, availability of services, and requirements technology and personnel requirements

**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, relevant information that may be used to answer important CER questions.

**Preliminary Data and Use of Accepted Measures**

PCORI encourages investigators to design their research using valid patient-centered outcomes measures. Include preliminary data that support using the proposed measures. We encourage investigators to consider those measures described in the *Patient-Reported Outcomes Measurement Information System (PROMIS)*.

**Methodological Considerations**

Regardless of study design, applications must adhere to all relevant PCORI Methodology Standards. These include 47 individual standards that fall into 11 categories. The first five categories are cross-cutting and are 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)

Six other standards categories will be applicable to particular study designs and methods. The standards in each of these categories should be used as guidance when they are relevant to a particular 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 Diagnostic Tests
6. Standards for Systematic Reviews

Most of these standards are minimal. Additional best practices—including relevant guidelines for conducting clinical trials developed by other organizations—should be addressed in the application for PCORI funding. To help reviewers quickly identify the adherence to a particular standard, applicants must cite each relevant PCORI Methodology Standard within their applications as the standard is being addressed. For example, when applicants describe the need for their proposed study within the Background section, they should indicate the particular standard to identify evidence gaps in parentheses, such as “(RQ-1).”

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 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, applicants are encouraged to review PCORI’s Engagement Rubric, which can be found in the PCORI Funding Center. Applicants should also review the PCORI Methodology Standards Associated with Patient-Centeredness, and PCORI’s Sample Engagement Plans. The rubric and Sample Engagement Plans are not intended to be comprehensive or prescriptive; instead, they provide a variety of examples to incorporate engagement, where relevant, into the research process.

Applicants are expected to consult with patients and other stakeholders on their decisional dilemma 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) confronted by the decision makers 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 in making this decision. Identify the patients and other stakeholders you consulted in determining that the proposed study addresses their evidentiary needs for decision making, and indicate your commitment to continue engaging them actively in the conduct of the study. Similarly, applicants should document how the project outcomes are especially relevant and meaningful endpoints to patients and other stakeholders.

For this funding announcement, applicants are not required to demonstrate that patients and other

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b Available at http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf
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 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 or clinical status, so that possible differences in CER may be examined (otherwise known as HTE). 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. The applicant must also discuss which subgroups are most important and how they will be analyzed, including whether there will be power to examine the question of effectiveness in subgroups. PCORI is particularly interested in including previously understudied populations for whom effectiveness information is especially needed, such as hard-to-reach populations or patients with multiple conditions. Thus, comparisons should examine the impact of the strategies in various subpopulations, with attention to the possibility that the strategy’s effects might differ across subpopulations. PCORI has developed the following list of priority populations 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
• Patients with low health literacy, numeracy, or limited English proficiency
• Lesbian, gay, bisexual, and transgender (LGBT) persons
• Veterans and members of the Armed Forces and their families

**Project Budget and Duration**

Applicants may request up to $8.33 million in total costs for a project period not to exceed five years. 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. Note that, in general, PCORI will not cover costs for interventions that are being compared in the proposed study. (See Appendix 2 in the Application Guidelines for details.) Applicants should submit realistic budgets and timelines. For those rare circumstances in which the estimated total costs exceed $8.33 million, provide a detailed justification in your LOI that ties the extra expense to the project’s success. Not all requests for additional funds will be approved. Any request for a project period longer than five years will be denied. For further information regarding PCORI’s policies about allowable and unallowable costs, refer to Appendix 2 of the Application Guidelines. 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.

The funding mechanism for this program is a contract. Total project funding is contingent upon successful programmatic and budget performance (e.g., meeting recruitment targets). Milestones and targets, as well as possible pilot phases for the sole purpose of assessing recruitment feasibility, should be included in the budget and will be negotiated at the time of the award. Awardees will be expected to provide corroborating evidence to receive continual funding support. Some of the 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 implementing the project
• Forming an SAC or other appropriate engagement body
• Obtaining clearances from all institutional and community partners, including IRB approvals
• Establishing a DSMB, or providing a clear description of why a DSMB is not necessary
• 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
• Demonstrating successful recruitment during a pilot phase (if indicated)
Refer to the Application Guidelines for a list of additional PFA-specific project milestones.

Collaboration

PCORI is particularly interested in applications that involve 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.

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, which is issued by the U.S. Department of Health and Human Services. PCORI does not require that applicants comply with sections of this policy that refer to requirements for 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 (DSMP), 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’ 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 have 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 Subject Participants

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

Data Management and Data-Sharing Plan

PCORI encourages openness in research and making research data available for purposes of replication.

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and reproducibility. Although not required to be submitted as a component of the research application, if an award is made, the awardee is required to develop and maintain a plan that addresses data management and data sharing of research project data in a manner that is appropriate for the nature of the research project and the types of research project data, and that is consistent with applicable privacy, confidentiality, and other legal requirements.

**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](http://www.pcori.org/sites/default/files/PCORI-Peer-Review-and-Release-of-Findings-Process.pdf).

In summary, Awardee Institutions are required to 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; 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 the draft final research report is accepted: (1) a 500-word abstract for medical professionals; (2) a standardized summary of 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

**Letter of Intent**

Applicants should download the [Cycle 3 2016 Sickle Cell Disease LOI Template](http://www.pcori.org/sites/default/files/PCORI-Peer-Review-and-Release-of-Findings-Process.pdf) from the PCORI Funding Center. They must complete the document and convert it to a PDF with a four-page limit. PCORI suggests including all references as in-text citations using American Medical Association citation style, but other citation styles are accepted. 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 Center for additional applicant resources, including the PFA 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 $8.33 million” is not
sufficient. Upload your document to PCORI Online. The deadline for LOI submission is September 14, 2016, by 5 p.m. (ET).

**Letter of Intent Review**

LOIs are evaluated based on the following criteria:

- Whether the proposed topic addresses the priority research question identified in this funding announcement
- Importance of the specific research question (comparison), as evidenced by critical gaps identified by clinical guidelines developers or recent relevant systematic reviews
- A size or scope sufficient enough to have a significant impact on patient outcomes or healthcare practices
- Clarity and credibility of the applicants’ responses to the LOI questions, as well as their justification of the proposed study 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 research study question and study design are compliant with requirements in this funding announcement
- Adherence to the administrative and formatting requirements listed in the Application Guidelines, specifically the four-page limit for the LOI

Only applicants whose LOIs are deemed most responsive to this PFA will be invited to submit a full application. Notification of denial or approval to submit an application will occur no later than October 21, 2016. Please refer to the Application Guidelines for information on how to submit your LOI via PCORI Online.

You are invited to submit an application based on the information provided in the LOI. Any changes to the following require PCORI’s approval:

- Research question(s)
- Specific aims
- Study design
- Comparators
- Principal Investigator (PI) (Contact PI and PI #2)
- Institution

If you need to change any of this information or have any questions, please email pfa@pcori.org.

**Note:** A PI can only submit one LOI per PFA. However, an individual listed as a PI on one LOI may be listed as and serve in another non-PI role (e.g., co-PI, 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 which PFA he or she would like to apply to. 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 Center.

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

Applicant Resources

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<tr>
<th>PCORI Funding Center</th>
<th><a href="http://www.pcori.org/Cycle-3-2016-sickle-cell-disease">http://www.pcori.org/Cycle-3-2016-sickle-cell-disease</a></th>
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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, policymakers 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., nonresponsiveness). 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 panel based on the number of and topic areas represented by invited LOIs. MROs recruit the panel chair, scientist reviewers who are subject matter experts, 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.

The table below is designed 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.

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<tr>
<th>Crosswalk of PCORI Merit Review Criteria with NIH Criteria</th>
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<tr>
<td>SIGNIFICANCE</td>
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<td>1. Potential for the study to fill critical gaps in evidence</td>
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<td>2. Potential for the study findings to be adopted into clinical practice and improve delivery of care</td>
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<td>APPROACH</td>
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<td>3. Scientific merit (research design, analysis, and outcomes)</td>
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<td>NEW 4. Investigator(s) and environment</td>
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<td>PCORI-only Merit Review Criteria</td>
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<td>PATIENT-CENTEREDNESS/ENGAGEMENT</td>
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<td>5. Patient-centeredness</td>
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<td>6. Patient and stakeholder engagement</td>
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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 how applications are evaluated.

**Criterion 1. Potential for the study to fill critical gaps in evidence:**
The application should address the following questions:

- Does the application convincing 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 positive findings could be reproduced by others, resulting in improvements in practice and patient outcomes? Identify the potential barriers that could hinder adoption of the intervention by others.
- Does the application describe a plan for how study findings will be disseminated beyond publication in peer-review 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 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 and well-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, observational study) accounted for and 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?

**NEW 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 well-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?
  
  o (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 they would be chosen by patients and their healthcare providers 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 based on PCORI’s merit review criteria, including evaluation of 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 further 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 is then proposed to the Board for consideration and approval.

In addition, PCORI evaluates applicant risk before issuing a PCORI 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 overdue reports have been submitted to PCORI.**

**Summary Statements and Funding Recommendations**

Summary statements are provided to applicants approximately two weeks before funding decisions are announced. **If an application progresses to in-person discussion,** the applicant will receive a summary statement inclusive of:

- In-person panel discussion notes
- Final average overall score
- Preliminary reviewer critiques
- Application quartile, which provides information for applicants to understand how they did relative to other discussed applications

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

Funding recommendations are made by identifying meritorious applications that fit the programmatic needs and that satisfactorily address the merit review criteria while adhering to the PCORI Methodology Standards. Programs also consider the funds allotted for the current funding announcement when deciding which applications to recommend to the Board for approval. Applicants to this current cycle’s PFA will receive summary statements in August 2017 and notification of the funding status of their application no later than September 2017.
References


