Cycle 2 2015 Funding Cycle

PCORI Funding Announcement:
Communication and Dissemination Research

Published June 29, 2015

This PCORI Funding Announcement applies to the funding cycle that closes on November 3, 2015, at 5:00 p.m. (ET). Application guidelines, templates, and other resources are available at http://www.pcori.org/Cycle-2-2015-communication/.
About PCORI

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, caregivers, and the broader healthcare community.

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

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

<table>
<thead>
<tr>
<th>Published</th>
<th>June 29, 2015</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Letter of Intent Due</strong></td>
<td>July 29, 2015, by 5:00 p.m. (ET)</td>
</tr>
<tr>
<td>Letters of Intent (LOIs) will be screened for responsiveness to this PCORI Funding Announcement (PFA) and for fit to program goals. Only those selected will be permitted to submit full applications. Notification of denial or approval to submit full application will occur no later than September 8, 2015.</td>
<td></td>
</tr>
<tr>
<td><strong>Summary</strong></td>
<td>PCORI seeks to fund projects that address critical knowledge gaps in the communication and dissemination process—both the communication and dissemination of research results to patients, their caregivers, and clinicians, and the communication between patients, caregivers, and clinicians—in the service of enabling patients and caregivers to make the best possible decisions in choosing among available options for care and treatment.</td>
</tr>
<tr>
<td><strong>Key Dates</strong></td>
<td></td>
</tr>
<tr>
<td>Online System Opens:</td>
<td>June 29, 2015</td>
</tr>
<tr>
<td>Letter of Intent (LOI) Deadline:</td>
<td>July 29, 2015, by 5:00 p.m. (ET)</td>
</tr>
<tr>
<td>Applicant Town Hall Sessions:</td>
<td>July 7, 2015, 2:30 p.m. – 4:00 p.m. (ET)</td>
</tr>
<tr>
<td></td>
<td>July 13, 2015, 11:00 a.m. – 12:30 p.m. (ET)</td>
</tr>
<tr>
<td>LOI Status Notification:</td>
<td>September 8, 2015</td>
</tr>
<tr>
<td>Application Deadline:</td>
<td>November 3, 2015, by 5:00 p.m. (ET)</td>
</tr>
<tr>
<td>Merit Review:</td>
<td>February 2016</td>
</tr>
<tr>
<td>Awards Announced:</td>
<td>April 2016</td>
</tr>
<tr>
<td>Earliest Project Start Date:</td>
<td>June 2016</td>
</tr>
<tr>
<td><strong>Maximum Project Budget (Direct Costs)</strong></td>
<td>$1.5 million</td>
</tr>
<tr>
<td><strong>Maximum Project Period</strong></td>
<td>Three years</td>
</tr>
<tr>
<td><strong>Budget/Time Limits</strong></td>
<td>Applicants must submit a Greater Than Time/Budget Request with their LOI if the proposed project’s budget or duration exceeds limits specified in this announcement.</td>
</tr>
<tr>
<td><strong>Funds Available up to</strong></td>
<td>$8 million</td>
</tr>
<tr>
<td><strong>Eligibility</strong></td>
<td>Applications may be submitted by any private-sector research organization, including any nonprofit or for-profit organization, and any public-sector research organization, including any university or college hospital or healthcare system, laboratory or manufacturer, or 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>
</tr>
</tbody>
</table>
### Review Criteria

1. Impact of the condition on the health of individuals and populations
2. Potential for the study to improve health care and outcomes
3. Technical merit
4. Patient-centeredness
5. Patient and stakeholder engagement

### Contact Us

**Programmatic Inquires:** Please contact the PCORI Helpdesk via email (sciencequestions@pcori.org), phone (202-627-1884), or online (http://www.pcori.org/PFA/inquiry). PCORI will provide a response within three business days. However, we cannot guarantee that all questions will be addressed three business days prior to a Letter of Intent or application deadline.

**Administrative, Financial, or Technical Inquiries:** Please contact the PCORI Helpdesk at pfa@pcori.org. PCORI will provide a response within two business days. Please note that during the week of a deadline, response times may exceed two business days. 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.

### Other

*Deadlines are at 5:00 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 2015 Funding Cycle:

- New Budget and Project Duration Section; Greater Than Time/Budget Requests considered at time of Letter of Intent submission
- New Letter of Intent Template
**Contents**

I. Introduction .......................................................................................................................... 7
   - About PCORI ..................................................................................................................... 7
   - Summary of Program ......................................................................................................... 7
   - Background ...................................................................................................................... 8
   - Research of Interest ......................................................................................................... 11
   - Sample Research Questions .......................................................................................... 11
   - Evidence to Action Networks .......................................................................................... 12

II. Requirements for PCORI Research .................................................................................. 12
   - Research Priorities ........................................................................................................... 12
   - Leveraging Existing Resources ....................................................................................... 13
   - Patient-Centered Outcome Measures ............................................................................ 13
   - Studies in Rare Diseases ................................................................................................. 14
   - Studies of Cost-Effectiveness .......................................................................................... 14
   - Categories of Nonresponsiveness .................................................................................... 14
   - Avoiding Redundancy ....................................................................................................... 15
   - Methodological Considerations ...................................................................................... 15
   - Patient and Stakeholder Engagement ............................................................................. 16
   - Populations Studied ......................................................................................................... 16
   - Protection of Human Subjects ......................................................................................... 17
   - Required Education of Key Personnel on the Protection of Human Subject Participants .................................................................................................................. 18
   - Replication and Reproducibility of Research and Data-Sharing Plan ......................... 18
   - Recruitment .................................................................................................................... 18
   - Peer Review and Release of Research Findings ............................................................... 18

III. How to Submit a Proposal ............................................................................................... 19
   - Letter of Intent ................................................................................................................ 19
   - Letter of Intent Review .................................................................................................... 20
   - Budget and Project Duration ............................................................................................ 20
   - Submission Dates ............................................................................................................. 20
   - PCORI Online System ...................................................................................................... 21
   - Applicant Resources ........................................................................................................ 21
IV. Merit Review ........................................................................................................................................21

- Preliminary Review ..............................................................................................................................21
- In-Person Review .................................................................................................................................23
- Post-Panel Review ...............................................................................................................................23
- Summary Statements and Funding Recommendations .........................................................................24
I. Introduction

About PCORI

PCORI funds Patient-Centered Outcomes Research (PCOR), a type of comparative effectiveness research (CER) that focuses on outcomes that matter to patients, their caregivers, and their families. The studies PCORI funds must include the perspectives of patients and other healthcare stakeholders.

To be considered responsive, applications must describe research that compares at least two alternative approaches. Approaches may address diagnostic methods or options, screening, interventions for prevention or treatment, or strategies to improve the healthcare system. “Usual care” (or no specific intervention) may be an appropriate comparator if this is a realistic choice faced by patients and other stakeholders, but the clinical characteristics must be specified. In general, through its various funding announcements, PCORI is interested in studies of interventions that may include:

- Specific drugs, devices, and procedures
- Other types of alternatives, such as medical and assistive devices and technologies
- Behavior change, including the use of behavioral or financial incentives (the latter for patients only)
- Organizational models and policies within and across healthcare systems (e.g., patient-centered medical homes, clinical protocols, and pathways)
- Communication and/or dissemination strategies

Summary of Program

Knowledge about how to communicate optimally and facilitate the effective use of PCOR and CER findings by patients, caregivers, and healthcare professionals needs to be strengthened. Well-documented barriers exist to the rapid transfer of evidence. Informed healthcare decisions require innovative and effective strategies to make existing PCOR/CER evidence available to patients and providers in real-world settings. Moreover, the information needs to be understandable in order to improve decision making.

The Communication and Dissemination Research Program at PCORI invites applications that study the comparative effectiveness of communication and dissemination strategies. We are looking for strategies aimed at informing and empowering patients, caregivers, and other healthcare decision makers so that they know what questions to ask and have the information needed to provide support in shared decision making.

This announcement is designed to solicit applications that include the direct comparison of two or more health communication and dissemination interventions or strategies that have established efficacy and engage patients, caregivers, and providers in the context of real-world clinical-care settings and situations. Interventions without efficacy data will not be considered in scope, unless they are already in wide use in clinical practice despite lack of these data.
Background

Making an informed healthcare choice requires critical assessment of the potential benefits and harms of the options within the context of the patient’s personal characteristics, conditions, and preferences.\(^1\),\(^2\) The environment where patients, caregivers, and their providers communicate is also evolving rapidly to include a wide array of available health information and communication applications. These tools can help fill critical information gaps, but are oftentimes confusing and difficult to use. The type of healthcare decisions being made is an important determinant of the appropriate information needed and the best vehicle for providing it. (For example, the information needs of a patient weighing options for treating high blood pressure will differ from those of a patient facing a terminal cancer diagnosis with complicated treatment options.) Furthermore, patients and caregivers desire information that does not necessarily deliver decisions or tell them what to do, but rather informs them of the relevant trade-offs and facilitates improved decision making in collaboration with their healthcare team.

Clear communication approaches and active dissemination of PCOR/CER research findings to all audiences (in easy-to-understand formats) are critical to increasing the awareness, consideration, adoption, and use of these data by patients, caregivers, and healthcare providers.\(^3\) This funding announcement focuses on three key areas: 1) communication strategies, 2) dissemination strategies, and 3) explaining uncertainty.

Communication strategies to promote the use of health and healthcare CER evidence by patients and clinicians

Translating existing scientific research into accessible and usable formats that clearly outline the risks and benefits of various healthcare options for patients, caregivers, and healthcare providers is an important area of research in both clinical and community-based settings. In clinical care, shared decision making and decision support interventions are two of the primary ways in which medical evidence is translated into a usable format for patients, families, and caregivers. Understanding the best ways to communicate, while addressing numeracy and health literacy, is fundamental to communicating PCOR/CER effectively via shared decision making.\(^4\),\(^5\),\(^6\) For example, the integration of

---

6 Sorensen, K., Van den Broucke, S., Fullam, J., Doyle, G., Pelikan, J., Slonska, Z., and Brand, H., for Consortium
patient decision support, electronic health records (EHRs), and associated patient systems holds considerable promise, but little evidence is available to guide best practices.

Research gaps identified in a systematic review included the need for more head-to-head comparisons of communication strategies. For example, more CER is needed to determine how shared decision making and decision support interventions perform using different media, what level of information and detail they require, how they perform in different patient subpopulations, and how they can reflect new evidence and remain current. Also, most of the CER research to date consists of comparisons of communication strategies relative to usual care. In most cases, it is difficult to determine what “usual care” is or how it differs from “standard of care.” Therefore, proposed research that includes usual care as the control condition or one arm of an intervention trial should clearly explain how the term “usual care” is defined, assessed, and justified as an appropriate comparator.

There are a number of areas where research is needed to determine the impact of CER on healthcare providers’ attitudes and actions. Changes in practice by providers in response to the availability of PCOR/CER findings have been limited. As such, it is unclear which methods for translating CER results into clinical care will prove to be most effective in terms of reaching the greatest number of patients and improving patient outcomes.

Effective communication skills of both patients and healthcare providers are important for the optimal use of CER results. Research on doctor-patient communication has focused primarily on the doctor-patient dyad, but little is known about other health professionals who communicate with patients and play a critical role in the patient care experience. Moreover, additional information is needed on how family involvement and family dynamics affect communication and the decision-making process. Applicants should consider broadening their focus beyond the patient-clinician dyad by recognizing that patient-centered care and communication are characterized by a complex web of communication between patients, caregivers, and a variety of healthcare professionals with whom they interact during different stages of the care continuum (pre-diagnosis to end-of-life).

**Dissemination strategies to promote the use of health and healthcare CER evidence by patients and clinicians**

The dissemination of CER information to patients, caregivers, and providers (in both clinical and community-based settings) is an area that has not received sufficient research attention. Dissemination is defined as the active and targeted approach of spreading evidence-based interventions to potential adopters and the target audience through determined channels using planned strategies. The goals of dissemination research are to increase the reach of information, motivation, and ability of patients, caregivers, and providers to use and apply evidence. Little is known about the comparative

---


effectiveness of dissemination methods and approaches for achieving these goals.

More research is needed to identify the most effective approaches for disseminating CER results to healthcare providers, with the goals of sustained changes in clinical practice and effective dissemination to patients of results that enable behavior changes (e.g., adherence and self-care). Research is also needed to identify trusted intermediaries and channels of communication most often turned to by patients, caregivers, and clinicians. Additionally, further investigation is needed to explore how strategies used in public health communication and social marketing can be adapted to disseminate the results of CER and to identify creative ways of combining multiple channels of communication and dissemination to increase exposure to CER. Research is also needed to examine the reliability of any CER data currently available through social media sites and to understand how individuals evaluate and use the information in their prevention, screening, diagnosis, and treatment decision-making processes. More specifically, there is a lack of information on how these media may influence patient self-care and adherence to treatment recommendations. In order to ensure that the “e-health revolution” does not widen existing health-related knowledge gaps among low-income and racial and ethnic minority populations, further exploration is needed to understand the disparities that may remain regarding access to social media resources.

Finally, the majority of work to date lacks a theoretical or conceptual justification for the dissemination strategy tested and/or the associated hypothesis for why the strategy was expected to be more effective than another. With no underlying framework, published results are difficult to interpret.3

Explaining uncertain health and healthcare CER evidence to patients and clinicians

Risk and uncertainty are ubiquitous in health care. Like most decisions, many healthcare decisions have consequences and involve uncertainties and trade-offs. A significant gap exists in the limited research on risk communication, in general, and with underserved individuals and those with limited health literacy and numeracy, in particular. In addition, research is lacking on effective methods for communicating risk to healthcare providers and enabling them to use the information effectively. A seminal publication on patient-centered communication from the National Cancer Institute identified managing uncertainty as a core function of patient-clinician communication.7 Uncertainty creates many challenges, including difficulties such as: a) determining whether preventive services and treatments should be implemented in clinical practice; b) determining for whom and in what settings services and treatments should be implemented; and c) communicating evidence so that consumers can make informed decisions.3 A systematic review identified research gaps that revealed a need for: analyses that identify and prioritize uncertainties that should be communicated; methods that measure and provide a better understanding of uncertainties, as they pertain to risks, practice recommendations, and other types of evidence; and standardized language used to communicate uncertainties in clinical evidence. The systematic review also revealed a need for formal systems used to rate uncertainty from clinical evidence that incorporates the patient perspective to ensure comprehensibility, meaningfulness, and appropriate use.9

Research of Interest

The Communication and Dissemination Research Program seeks to fund investigator-initiated CER that:

- Compares strategies that increase knowledge on how to communicate complex information to patients and caregivers
- Compares and identifies best practices of dissemination and translation techniques to facilitate shared decision making in everyday practice
- Identifies and compares practices that increase understanding of the tension between strongly held beliefs and contrary evidence, and of their impact on the shared decision-making process
- Compares strategies meant to generate conversations between patients and providers about what is appropriate and necessary treatment (e.g., Choosing Wisely\textsuperscript{10}) based on patients’ preferences and conditions
- Compares strategies for conveying uncertainty associated with health and healthcare evidence that increase the likelihood that patients and caregivers will understand the information, incorporate it into decision making, and evaluate personal trade-offs
- Identifies and compares promising practices that address contextual factors and their impact on patient-centered communication
- Compares the effectiveness of health literacy- and numeracy-sensitive health communication strategies that relay risks and benefits of health decisions so that individuals can make sound healthcare decisions
- Compares strategies and methods that optimize communication between the patient, family/caregiver, and the healthcare team (e.g., role of family member/caregiver in patient–provider, patient–caregiver, and healthcare team interactions)
- Compares innovative approaches for using existing electronic clinical data and other electronic modalities (e.g., EHRs) from the healthcare system or from a network of systems to enhance clinical decision making by patients and providers

PCORI is interested in understanding the role of shared decision making and established, effective decision aids in communicating and implementing PCOR/CER. The development, testing (establishing efficacy), and validation of individual decision aids/tools will be considered nonresponsive to this PFA.

Research studies may focus on patient populations with a single condition or involve patients with a range of conditions. Studies addressing care for patients with rare conditions are also of special interest. Rare diseases are defined as life-threatening or chronically debilitating diseases that are of such low prevalence that special efforts—such as combining data across large populations—may be needed to address them. Low prevalence is defined as a condition that affects fewer than 200,000 individuals in the United States or that has a prevalence of less than one in 1,500 persons.

Sample Research Questions

The following are meant as examples of the types of questions that your research may help answer. This list is by no means exhaustive, nor is it structured in priority order. \textit{All research questions must have a}

\textsuperscript{10} Available at \url{http://www.choosingwisely.org/}.
comparative component.

- How do designs for decision-support interventions compare in their ability to assist patients and/or caregivers with lower levels of literacy/numeracy, and how do strategies for communicating risk information to vulnerable populations compare?
- Which methods of dissemination are most effective in imparting useful information to patients and their caregivers in order to increase adoption of practices, patient outcomes, and involvement in care decisions?
- How do methods for distributing CER findings to patients, caregivers, or healthcare providers compare in their ability to improve patients’ health outcomes?
- To whom are clinicians most likely to turn for trustworthy information about the effectiveness, relative effectiveness, benefits, and harms of different treatment options for a given condition, and how do they access that information?
- How do strategies learned from public health communication and social marketing compare in their ability to promote the distribution of CER to patients and/or their caregivers and their clinicians?
- How do strategies in community-based settings compare with those in clinical-based settings in their ability to promote the distribution of CER to patients and/or their caregivers?

To be competitive for a PCORI contract, an application must make the case that its proposed research question(s) and outcomes will matter to patients and/or healthcare stakeholders.

Evidence to Action Networks

PCORI is interested in ensuring communication and engagement between awardees with similar needs and interests and end-users to help refine and improve the research and to facilitate dissemination of research findings that will help patients and the public make better-informed healthcare decisions. To meet this goal, PCORI has set up Evidence to Action Networks, whereby PCORI facilitates engagement among awardees and cross-learning between projects and teams comprising researchers, patients, caregivers, and other stakeholders. In addition, PCORI facilitates exchanges between awardees and end-users (e.g., patients, caregivers, and other stakeholders, such as payers, employers and purchasers, clinicians, professional societies, policy makers, and training institutions) for dissemination and implementation of important research findings.

Awardees are encouraged to participate in such Evidence to Action Networks if they become available on a specific topic relevant to their research.

II. Requirements for PCORI Research

This section includes language that is specific to PCORI’s requirements for applications for funding. Applicants should use this section as guidance when preparing their applications.

Research Priorities

Regardless of the approach being studied, all proposed research projects must compare at least two
alternatives. Optimally, the study will compare two or more defined strategies. In general, “usual care” is not an appropriate comparator for CER studies submitted to PCORI for funding consideration. “Usual care” is too often ill-defined, difficult to quantify, and subject to considerable geographic and temporal variations, thus limiting interpretability, applicability, and reproducibility. If the applicant proposes “usual care” as a rational and important comparator in the proposed study, then it must be described in detail, coherent as a clinical alternative, and properly justified as a legitimate comparator (e.g., usual care is guidelines-based). Additionally, it must be accompanied by an explanation of how the care given in the usual-care group will be measured in each individual patient and how appropriate inferences will be drawn from its inclusion. To be considered responsive, applications must:

- **Describe research that compares two or more strategies that each have established efficacy.** PCORI expects the efficacy or effectiveness of each intervention to be known. If the efficacy/evidence base is insufficient, then data need to be provided to document that the intervention is widely used. The application must provide information about efficacy of the interventions and/or dissemination strategies that will be compared; pilot data may be appropriate. Projects that aim to develop new or novel interventions, which lack evidence of efficacy or effectiveness, will be considered out of scope.

- **Describe research that studies the benefits and harms of interventions and strategies delivered in real-world settings.** PCORI is interested in studies that provide practical information that can help patients and other stakeholders make informed decisions about their health care and health outcomes.

- **Describe research that is based on health outcomes that are meaningful to the patient population, their caregivers, and family members under study and that are likely to guide their decisions.** These outcomes must be demonstrated to matter to patients, including measures of quality of life, symptoms of disease, relevant physiological measurements, treatment-related symptoms (side effects), healthcare utilization, and/or clinical outcomes.

### Leveraging Existing Resources

Investigators are encouraged 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 clinical comparative effectiveness research questions.

### Patient-Centered Outcome Measures

PCORI encourages investigators to design their research using valid outcome measures. Include preliminary data that support the proposed measures. Investigators are encouraged to consider those measures described in the Patient Reported Outcomes Measurement Information System\(^\text{11}\) (PROMIS).

\(^{11}\) Available at http://www.nihpromis.org/.

PCORI Funding Announcement: Communication and Dissemination Research 13
Studies in Rare Diseases

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

Studies of Cost-Effectiveness

Applications will be considered nonresponsive if the proposed research:

- Conducts a formal cost-effectiveness analysis in the form of dollar-cost per quality-adjusted life-year to compare two or more alternatives
- Measures the relative costs of care of two or more alternative approaches

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

PCORI does have an interest, however, in studies that address questions about conditions that lead to high costs to the individual or to society. This is included in our review criterion on impact of the condition on the health of individuals and populations. Thus, 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

Categories of Nonresponsiveness

PCORI discourages proposals in the following categories and will likely deem them nonresponsive:

- Instrument development
- Developing, testing, and validating new decision aids/tools or clinical prognostication tools
- Pilot studies intended to inform larger efforts
- Comparisons of patient characteristics rather than clinical strategy options
- Studies comparing interventions for which the primary focus is the role of community health workers or patient navigators

Consistent with PCORI’s authorizing law, PCORI does not fund research whose findings will include:

\[\text{Available at http://www.pcori.org/sites/default/files/PCORI_Authorizing_Legislation.pdf.}\]
• Practice guidelines
• Coverage recommendations
• Payment or policy recommendations
• Creation of clinical practice guidelines or care pathways
• Establishing efficacy for a new clinical strategy
• Pharmacodynamics
• Study of the natural history of disease
• Fundamental science or study of biological mechanisms

Avoiding Redundancy

PCORI encourages potential applicants to review funded research at pcori.org, because PCORI intends to balance its funded portfolio to achieve synergy where possible and to avoid redundancy.

Methodological Considerations

Regardless of study design, proposals 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 relevant to most PCOR studies. Researchers should refer to all of these standards when planning and conducting their research projects. These categories are:

• Standards for Formulating Research Questions
• Standards Associated with Patient-Centeredness
• Standards on Data Integrity and Rigorous Analyses
• Standards for Preventing and Handling Missing Data
• Standards for Heterogeneity of Treatment Effect (HTE)

Six other categories of standards will be applicable to certain types of study designs and methods. The standards in each of these categories should be used for guidance when they are relevant to a particular study. These categories are:

• Standards for Data Registries
• Standards for Data Networks as Research-facilitating Infrastructures
• Standards for Causal Inference Methods
• Standards for Adaptive and Bayesian Trial Designs
• Standards for Studies of Diagnostic Tests
• Standards for Systematic Reviews
Most of these standards should be considered “minimal.” The methodology standards\textsuperscript{13} reflect practices that should be followed in all cases, and all deviations need to be explained and well justified. Additional best practices, including accepted guidelines for the conduct of clinical trials or observational studies, should be addressed, if applicable, in the application for PCORI funding.

Applicants should discuss specifically how the planned study design will measure and adjust for potential confounding factors that may obscure or artificially create differences attributable to the alternatives being compared. Examples include, but are not limited to, baseline differences in disease severity or other risk factors within the study population or differences in participation, adherence, or follow-up that may affect outcomes independently of the interventions being compared.

**Patient and Stakeholder Engagement**

PCORI encourages all applicants to describe clearly patient and stakeholder engagement in their research proposals. PCORI understands that patient and stakeholder engagement in research can take many forms; it is not seeking one particular type or method of engagement. Rather, applicants should communicate how patients (those with lived experience), family members, caregivers, and the organizations that represent them, as well as any other relevant stakeholders, will be involved in study activities. Because this type of engagement in research is a relatively new concept, PCORI has developed the Engagement Rubric to guide both applicants and merit reviewers. This rubric is intended to provide examples of engagement and is not intended to be prescriptive. As noted above, studies are expected to adhere to PCORI’s Methodology Standards Associated with Patient-Centeredness as well as to the PCOR Engagement Principles found within the rubric. These and additional resources are available in PCORI’s Engagement in Research page.

**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 outcomes may be examined in defined subpopulations, otherwise known as heterogeneity of treatment (HTE). PCORI recognizes that some proposed studies may 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 importance of the study in the absence of diversity and to 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 the inclusion of 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 possibilities that the effects of the strategy might differ across subpopulations. PCORI has developed a list of priority populations to guide our efforts in research and engagement, which includes:

- Racial and ethnic minority groups

\textsuperscript{13} Available at http://www.pcori.org/content/pcori-methodology-report.
• 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 and/or limited English proficiency
• LGBT persons

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 “Human Subjects Research Policy” from the Supplemental Grant Application Instructions for All Competing Applications and Progress Reports,\(^\text{14}\) issued by the U.S. Department of Health and Human Services (DHHS). PCORI does not require that applicants comply with sections of this policy that refer to requirements for federal-wide assurance (FWA) or that refer to standards for inclusion of women, minorities, and children. PCORI requires applicants proposing clinical trials to include a data-and safety-monitoring plan. Awardees must also comply with appropriate state, local, and institutional regulations and guidelines pertaining to the use of human subjects in research.

PCORI merit reviewers will examine plans for protection of human subjects in all applications and may provide comments regarding the plans (see How To Evaluate Human Subjects Protections\(^\text{15}\)). Reviewers’ comments on human subjects research are not reflected in the overall application score but may be used by PCORI staff during any potential funding negotiations. Final determinations about adequacy of human subject protections rest with the Institutional Review Board (IRB) or IRBs that has or have jurisdiction for the study.

The awardee institution or organization, 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 personnel listed as Key Personnel in the application. The policy and FAQs are available from the NIH website.16

Replication and Reproducibility of Research and Data-Sharing Plan

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

Recruitment

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

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. The PCORI Board of Governors adopted the following process for peer review and public release of the results of all funded studies.

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 properly interprets the findings in clinical or other decisional contexts. Subject matter experts, individuals with expertise on research methodology or biostatistics, as well as patients, caregivers, and other healthcare stakeholders, will review the draft final research report. After awardees 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: a 500-word abstract for medical professionals, a standardized summary of the study’s results for patients and the general public, and a link to the study record on ClinicalTrials.gov (as applicable). 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 a Proposal

Letter of Intent

IMPORTANT TO NOTE: The Communication and Dissemination Research Program will be using an LOI screening process. LOIs with the primary focus of tool development, testing, and validation will be screened out and not considered. You may submit a full application only if invited to do so based on your LOI. Research questions proposed in the LOI cannot change if you are invited to submit a full application.

Applicants should download the Letter of Intent Template for the Communication and Dissemination Research PFA from the Funding Center. The LOI has a three-page limit. References should be numbered in the text and full citation provided on a separate page following the LOI. Complete the document and convert it to a PDF file. Letters of Intent that exceed the page limit (excluding references) will not be reviewed. Do not upload additional documents as part of your LOI, including letters of endorsement or support, as they are not requested at this stage. Inclusion of additional documents will result in LOI rejection without review. Please visit the Funding Center for additional applicant resources, including the PFA and required templates.

The LOI for the proposed study should contain the following information:

- Title of proposed study
- Specific aims: clearly stated
- Communication and dissemination issue being addressed by the proposal and how it will improve the quality and relevance of evidence available to help patients and stakeholders make informed health decisions
- Knowledge gap being addressed by research question(s)
- Study design: a concise description
- Study population: clear description; representative of community practice; inclusion of PCORI priority populations
- Outcomes: primary outcomes identified; description of why they are important to patients
- Sample size stated
- Comparators clearly described with demonstrated efficacy specified for each and details on how the interventions/strategies will be delivered in real-world settings
- Patient and stakeholder engagement: involvement throughout planning, implementation of the project, and dissemination of findings discussed

Additional consideration will be given to programmatic fit and balance, taking into consideration whether the proposal significantly overlaps with previously funded studies or concurrent proposals, or, conversely, whether the proposal fills a gap in the portfolio of proposals with certain characteristics, including disease category, topics, priority population, methodologies, and other variables.
Please address all categories in the LOI Template, then upload the document into the PCORI Online System. The deadline for LOI submission is July 29, 2015, by 5:00 p.m. (ET).

Letter of Intent Review

LOIs are evaluated on the following criteria (note that PCORI does not score the LOI):

- Importance and relevance of the topics to PCORI priorities, as evidenced by critical gaps identified by clinical guidelines developers and/or recent relevant systematic reviews
- Clarity and credibility of applicants’ responses to the LOI questions
- Prior relevant experience
- Programmatic fit and balance, taking into consideration whether the proposals significantly overlap with previously funded studies or concurrent proposals, or, conversely, whether the proposal fills a gap in the portfolio of proposals with certain characteristics, including disease category, topics, priority population, methodologies, and other variables

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 full application will occur no later than September 8, 2015.

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)
- Institution

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

Note: A PI may submit multiple LOIs in a cycle but the research topics/projects should not be similar. If a PI submits an LOI to multiple PFAs, LOIs that show scientific overlap or that appear to be duplicate submissions will be disqualified. PCORI will contact the PI and give them an opportunity to choose which PFA they would like to apply to. An individual listed as a PI on one LOI may be listed and serve in another role (e.g., co-investigator, co-PI) on other LOIs within the same PFA during the same cycle.

Budget and Project Duration

The maximum budget for this PFA is $1.5 million total direct costs. The maximum period of performance is three years. This program does consider exceptions to the budget or to period of performance limits. If you submit an application that exceeds the $1.5 million total direct cost cap and/or the three-year period of performance, you must also complete and submit with your LOI a Greater Than Time/Budget Request form.

Submission Dates

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

**PCORI Online System**

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

**Applicant Resources**

<table>
<thead>
<tr>
<th>PCORI Funding Center</th>
<th><a href="http://www.pcori.org/Cycle-2-2015-communication/">http://www.pcori.org/Cycle-2-2015-communication/</a></th>
</tr>
</thead>
<tbody>
<tr>
<td>PCORI Online System</td>
<td><a href="https://pcori.fluxx.io">https://pcori.fluxx.io</a></td>
</tr>
<tr>
<td>PCORI Funding Awards</td>
<td>pcori.org/pfaawards</td>
</tr>
</tbody>
</table>

**IV. Merit Review**

PCORI Merit Review is a multiphase process that includes: PFA development; staff evaluation of LOIs; preliminary review of full applications by review panels; in-person panel discussion of a subset of full applications (identified by PCORI’s Research Priority Area Program staff, based on the preliminary review and program priorities); Selection Committee recommendation of applications for funding; and, finally, Board of Governors (Board) award approval (no later than April 2016).

**Preliminary Review**

PCORI conducts rigorous merit review of the full applications it receives. Note that applications may be eliminated 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 may be scientifically withdrawn if it is not responsive to the guidelines as described in this PFA, describes research that is not comparative, includes cost-effectiveness analysis, or otherwise does not meet PCORI programmatic requirements.

PCORI Merit Review Officers (MROs) recruit each panel based on the number 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 all understand the programmatic and organizational goals of review.

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:

**Criterion 1. Impact of the condition on the health of individuals and populations**

The proposal addresses the following questions:

- Is the condition or disease associated with a significant burden in the U.S. population, in terms
of prevalence, mortality, morbidity, individual suffering, or loss of productivity?

- Alternately, does the condition or disease impose a significant burden on a smaller number of people who have a rare disease?
- Does the proposal include a particular emphasis on patients with one or more chronic condition(s)?

**Criterion 2. Potential for the study to improve healthcare and outcomes**

The proposal has the potential to lead to meaningful improvement in the quality and efficiency of care and to improvements in outcomes important to patients. It addresses the following questions:

- Does the research question address a critical gap in current knowledge as noted in systematic reviews, guideline development efforts, or previous research prioritizations?
- Has it been identified as important by patient, caregiver, or clinician groups?
- Do wide variations in practice patterns suggest current clinical uncertainty?
- Is the research novel or innovative in its methods or approach, in the population being studied, or in the intervention being evaluated, in ways that make it likely to improve care?
- Do preliminary studies indicate potential for a sizable benefit of the intervention relative to current practice? How likely is it that positive findings could be disseminated and implemented quickly, resulting in improvements in practice and patient outcomes?

**Criterion 3. Technical merit**

The proposal has sufficient technical merit in the research design to ensure that the study goals will be met. It addresses the following questions:

- Does the proposal delineate a clear conceptual framework/theory/model that anchors the background literature and informs the design, key variables, and relationships being tested?
- Does the research plan describe rigorous methods that demonstrate adherence to PCORI’s Methodology Standards?
- Are the comparison interventions realistic options that exist in current practice?
- Are the sample sizes and power estimates presented based on realistic and careful evaluations of the anticipated effect size?
- Is the project timeline realistic, including specific scientific and engagement milestones?
- Does the research team have the necessary expertise to conduct the project?
- Is the organizational structure and the described resources appropriate to carry out the project?
- Is there a diverse study population with respect to age, gender, race, ethnicity, and clinical status, appropriate for the proposed research?

**Criterion 4. Patient-centeredness**

The proposal demonstrates patient-centeredness at every stage of the research. It addresses the questions:

- Is the research focused on questions that affect outcomes of interest to patients and their
Does the research address one or more of the key questions mentioned in PCORI’s definition of PCOR?

Criterion 5. Patient and stakeholder engagement

The proposal demonstrates that people representing the population of interest and other relevant stakeholders are engaged in ways that are appropriate and necessary in a given research context. It addresses the questions:

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

- Are the roles and the decision-making authority of all research partners clearly stated?

- Does the proposal demonstrate the principles of reciprocal relationships, co-learning, partnership, trust, transparency, and honesty?

In-Person Review

After the preliminary review is completed, PCORI program staff members evaluate panel scores and critiques to identify a subset of applications to be discussed at the in-person review meeting. Not all submitted applications move forward to in-person review, but all applications are evaluated and scored based on PCORI’s merit review criteria, which include evaluation of adherence to PCORI’s Methodology Standards.

During the in-person review, panels meet to discuss applications and to clarify further the merits of the proposed research as well as to identify areas for improvement. Additionally, each application is re-scored based on the content of discussion. The Chair and PCORI MRO lead the in-person panel meeting and ensure that all applications receive a fair and thorough review informed by the standards outlined in the PFA.

Post-Panel Review

After the in-person panel review, PCORI program staff review meritorious applications’ merit review 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 PCORI’s Board. The 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 PCORI’s Board for its consideration and approval.
Summary Statements and Funding Recommendations

Summary statements and funding decision notifications are provided to applicants contemporaneously. If an application progresses to in-person discussion, the applicant will receive a summary statement inclusive of the panel discussion notes, the final average overall score, and preliminary reviewer critiques. 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 PCORI’s Methodology Standards. Programs also consider the funds allotted for the current funding announcement when deciding which applications to recommend to PCORI’s Board for approval. Applicants will receive summary statements and notification of the funding status of their application no later than April 2016.