



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# Patient-Centered Outcomes Research Institute

Funding Announcement:

***Improving Healthcare Systems***

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## Opportunity Snapshot

*A small health plan faces an important challenge to improve the quality and patient-centeredness of care for its enrollees. Resources are limited and the leaders want to make wise decisions that will lead to interventions that improve the effectiveness, safety, timeliness, equity, and efficiency of care—and certainly to more patient-centered care. They have heard and read many reports of successful interventions in other systems, but most of these have not been rigorously evaluated, and they have not been compared with each another. They are interested in the impact of programs on a variety of patient-centered outcomes, and they wonder whether particular interventions could work for organizations and patient populations similar to theirs.*

## Purpose

Every day, healthcare organizations are faced with crucial decisions about improving their systems of care and a lack of critical information to guide them. The research they need should be designed to help them provide better care to the patients in their organization, effectively and efficiently.

We at the Patient-Centered Outcomes Research Institute (PCORI) are entrusted by the public to fund research that will matter to patients and their caregivers, and we now turn to you to help us. We have identified five national priorities and a research agenda that guide the projects we will fund. The agenda is focused on producing knowledge that is useful to patients, their caregivers and clinicians, and others who can affect their care. We believe that the important gaps in knowledge are widespread. Rather than dictate which conditions and questions are more important than others, we have chosen to seek wisdom from around the country in the form of applications for funding in the five priority areas. We also have identified some areas, such as rare conditions, that are often neglected and that we want to be sure to cover among our funded projects.

In this PCORI Funding Announcement (PFA), we seek to fund projects that address critical decisions that face healthcare system leaders and policymakers, clinicians, and the patients and caregivers who rely on them. These decisions must be consequential, be occurring now without key evidence about the comparative effectiveness of two or more approaches, and there must be substantial potential that patients/caregivers will benefit from the new knowledge in ways that are important. The premise of this research is that new knowledge will support critical choices by patients and other key stakeholders in health care, not that it will deliver a verdict that leads us to dictate a choice. This knowledge will provide insight about the comparative benefits and harms of the options and provide information about outcomes that are experienced by patients and important to patients.

We are seeking to change a paradigm, to replace the traditional research model—which places the researcher at the center of the process—with one that involves strong teams with varying perspectives, finding ways to work together to understand and address patient needs. In the end, PCORI will judge itself and those it funded by whether this model succeeds in producing knowledge that patients need—and does so more efficiently and rapidly. We hope that you—patients, caregivers, clinicians, health plans, product manufacturers, policy makers, and researchers from around the country—will join us in producing an unprecedented portfolio of truly patient-centered outcomes research that will transform the ability of

patients, their caregivers, and clinicians to seek, find, and use practical information in the decision-making process.

## Funds Available

We anticipate that approximately 27 contracts totaling up to \$24 million in total costs may be funded under this PFA in this funding cycle, assuming receipt of a sufficient number of high quality applications. PCORI anticipates additional funding cycles related to this announcement. However, funds available may vary, and PCORI reserves the right to modify or terminate this announcement at any time.

## Budget and Project Periods

Direct project costs are limited to a maximum of \$500,000 per year.

## Organizational Eligibility

Applications may be submitted by:

- Any private sector research organization, including any:
  - Non-profit organization
  - For-profit organization
- Any public sector research organization, including any:
  - University or college
  - Hospital or healthcare system
  - Laboratory
  - Unit of state or local government

All US applicant organizations must be recognized by the Internal Revenue Service. Foreign organizations and nondomestic components of organizations based in the United States may apply, as long as there is demonstrable benefit to the US healthcare system and US efforts in the area of patient-centered research can be clearly shown. Individuals may not apply. Foreign organizations should consult the PCORI Application Guidelines because there is an extra step for such organizations to register within the PCORI online system.

In addition, investigators may serve as Principal Investigator (PI) on only one application for any individual PFA per cycle. An individual who is a PI may, however, participate in other applications (from the same or other organizations) in a different role, such as co-investigator or consultant.

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# Improving Healthcare Systems

If you are interested in applying for an award under this program, follow PCORI's five-step process.



- ✓ **Review the Program Detail:** Get familiar with the program announcement and PCORI's areas of interest. Look at the example questions.
- ✓ **Consider the Components:** Consider the applicant eligibility requirements and PCORI's specific requirements to see if your organization, your interests, and your project fit within this program.
- ✓ **Develop Your Application:** Design the project. Determine and document who will be involved, the research strategy, and the budget needs. To see the Application Guidelines, go to <http://www.pcori.org/assets/PFAguidelines.pdf>.
- ✓ **Know the Review Criteria:** Understand the PCORI merit-review assessment criteria. The criteria are provided at the end of this document.
- ✓ **Submit Your Application:** Compile and submit your application. To see the Application Guidelines go to <http://www.pcori.org/assets/PFAguidelines.pdf>. You can register for the online system and submit a Letter of Intent (LOI) beginning September 17, 2012. A link to the online system will be available on the PCORI Funding Announcements (PFAs) web page at <http://www.pcori.org/funding-opportunities/funding-announcements/>.



## Step 1: Review Program Detail Overview

We are soliciting comparative outcomes studies of strategies (e.g. policies, interventions, service designs) employed by healthcare systems to improve the quality of care, the outcomes of care, or the efficiency of care for the patients they serve. Healthcare systems include health plans; physician groups; hospitals; academic medical centers; integrated delivery systems; community-based and safety net clinics; federal, state, and municipal providers and payers; and other entities organized to deliver, arrange, or coordinate healthcare services. PCORI seeks studies that will provide information of value to patients, their caregivers, and clinicians, as well as to healthcare leaders and decision makers on which types of systems and which system strategies lead to better patient outcomes. More recent and less-studied system strategies are of particular interest.

### Research Areas of Interest

We are interested in the following broad topical areas:

- Research that compares alternative system-level approaches to supporting and improving patient access to care; receipt of appropriate evidence-based care; the quality, timeliness, and safety of the patient care experience; decision-making based on patients' personal values; and self-care. Research that compares alternative approaches to models of care delivery or coordination of care across healthcare services or settings, including care for patients with complex, chronic, and/or multiple conditions, are of interest. The emphasis is on comparing approaches for their effect on patients and, when relevant, their caregivers, in ways that they experience and think are important.
- Research that compares alternative system-level approaches that aim to improve the efficiency of health care delivery to patient populations. These may include efforts to reduce the use of ineffective or wasteful care, to reduce redundant and duplicative care, to shorten waiting times, or enhance the timeliness and quality of communications during referrals and transitions in care.

Strategies of interest include, but are not limited to, novel applications of health information systems, including electronic health records, patient systems, and personal health records; the use of incentives directed at clinicians or patients; of payment reforms such as value-based purchasing and bundled payments; reconfigurations (redesign) of care, such as the patient-centered medical home and accountable care organizations; models of care coordination and integration; organizational decision-making protocols to guide care referral and specialized assessment of patients with specific complex conditions; and new

*I have a patient who has a knee problem and, by chance, went to a chiropractor and was there for four sessions; and now she is normal. She was in need of knee surgery, and now she isn't.*

—Cardiologist

*I think every single day I've taken somebody off a brand name drug and put them on a generic. Yesterday I took somebody off three brand name drugs and put him on generics because he was paying \$95 co-payments on them. He said, "I can't do it. My son lost his job. I spent my retirement trying to keep them afloat. I don't have any money."*

—Primary care physician

*If the primary care is not talking to his cardiologist, then what good is it to the patient? We're kind of talking around each other and doing the same task over and over and over.*

—Primary care physician

and extended roles for allied health professionals (e.g. pharmacists, nurses, physician assistants, dentists, chiropractors, complementary and alternative medicine providers, patient navigators, health coaches, social and service coordinators, volunteers, etc.).

Strategies may focus on patient or system enrollee populations with a single condition or with a range of conditions. Systems strategies to support or improve care for patients with rare conditions are of interest. Rare diseases are defined as life-threatening or chronically debilitating diseases that are of such low prevalence in populations that special efforts, such as combining data across large populations, may be needed to address them. The term *low prevalence* is defined as meaning conditions that affect fewer than 200,000 individuals in the United States or have a prevalence of less than 1 in 1,500 persons.

## Background

A healthcare system is characterized by the organization of people and resources to deliver services to meet the health needs of target populations. Systems may be publicly or privately organized. Systems are often evaluated in terms of their ability to deliver accessible, high quality, efficient, and equitable care. Governmental policies and market forces motivate health systems to improve these parameters, and these pressures in turn help to shape the clinical, organizational, workforce, and financing decisions made by systems. Importantly, the demographic and clinical characteristics of the populations served and the scope and mission of the organization also have strong influences on each of these system-level metrics by altering the access, patterns of use, and effectiveness of various health services. Over the past two decades, the Institute of Medicine (IOM) and others have sharpened the focus on ensuring that systems are also designed and oriented toward achieving the health outcomes most desired by individual patients—that is, to become more patient-centered.

Innovation and change in healthcare systems and in the behavior of health system actors (patients, caregivers, clinicians, payers, purchasers, industry, researchers, and policy makers) is often driven by economic, political, and social needs to improve access to care or quality of care, to attract patients or enrollees, and to contain costs. In recent times, rising healthcare costs, demographic trends, and the implementation of the Affordable Care Act have been important catalysts to many new approaches to change how healthcare systems respond to the current and expected future health needs of the US population.

Healthcare systems interventions, however, are often initiated and adopted without robust evaluation of their impact on the health and well-being of the population—especially of more vulnerable patients within populations, such as the elderly, disabled, low-income patients, and those with multiple chronic illnesses. In the IOM's 2009 recommendations for initial priorities for comparative clinical effectiveness research (CER), nearly half of the 100 priorities identified related to healthcare delivery systems, underscoring uncertainty on what constitutes best practice and a collective appreciation of the critical role of systems in implementing clinical evidence and improving care.

The importance of integrated care models, such as the patient-centered medical home (PCMH) and accountable care organizations (ACOs), for improving system performance and patient outcomes has been widely discussed, but understanding of the impact of these new models remains underdeveloped. As an

example, while elements of the PCMH have been associated with improved outcomes and care processes, there is relatively little evidence to date on the impact of implementing comprehensive PCMH interventions on outcomes that matter to patients. New payment models, such as bundled payments, are also being adopted as strategies for delivery reform, but their impact on patient care and outcomes remains unclear. The consolidation of physician practices by hospitals and the integration of health insurers and providers, most notably hospital operators, have begun to alter the organizational landscape, again with little information on the impact to patients or clinicians.

Other system-level interventions have focused on helping patients and clinicians make more appropriate decisions. Performance measurement is widely used to enhance implementation and adherence to evidence-based guidelines. New tools are emerging, such as clinical and patient decision support systems that address both of these goals. The overall effectiveness of these tools as implemented within healthcare systems, and particularly for patient populations such as those with limited English language proficiency or education, is less well understood. Use of patient navigators has been shown to have promising effects in relatively limited applications, such as cancer screening, but could have promise in multiple therapeutic areas. A variety of system-level interventions have focused on the important goal of improving patient adherence to chronic medications, but most have had limited effects on clinical outcomes and have not measured more patient-centered outcomes.

To improve access to health care and the match of skills with patient needs, new and expanded roles for allied health professionals are increasingly being developed and refined within systems. The roles of nurse practitioners (NPs) or physician's assistants in providing primary and limited specialty care, and the role of pharmacists and other allied health professionals in care management and adherence counseling, deserve greater scrutiny with a particular emphasis on outcomes that matter to patients, including symptom management; self-efficacy; satisfaction; physical, emotional, and social functioning; as well as biometric measures of risk factor control; adherence; and clinical endpoints, including survival.

Other important trends in recent years include more sophisticated use of systems and technology, such as electronic health records, personal health records, web-based applications, and other forms of digital communication for enhancing both efficiency and effectiveness in delivering information to patients and clinicians about evidence-based care for their conditions, including chronic and multiple conditions. Each of these deserves further study, especially when interventions have been taken to scale within healthcare systems.

Many system-level concerns require better understanding of the relationship between the organization and delivery of care within the system and the circumstances, characteristics, and values of the patient. For example, reducing unplanned and inappropriate hospital readmissions has importance to patients and families, as well as potential economic impact, and studying the effects of structured, individualized discharge planning on outcomes that patients care about is needed, along with a broader understanding of other contributors to readmission. Similarly, identifying and reducing the factors within health systems that contribute to patients' development of disability or chronic conditions may significantly improve the lives of patients and their families. System interventions to deliver evidence-based practices, such as substance abuse or depression screening, or other early or supportive interventions (screening, coaching, navigation,

coordination, management, knowledge sharing, and activation) to prevent or slow progression of conditions and to understand their impact on outcomes important to patients is lacking.

The emergence and continued expansion of retail clinics and other “retail” and “self-service” modes of healthcare delivery, such as home diagnostics, has received very little attention, despite concerns that these interventions may have mixed consequences, perhaps especially for populations lacking adequate alternatives, such as access to traditional primary care.

With respect to all of the interventions outlined above, concerns remain that effectiveness may vary among population subgroups. Among the groups of particular concern are patients with multiple chronic conditions; the disabled; frail elderly; patients with low literacy, numeracy, or health literacy; and those of low income, including the dually eligible (Medicare/Medicaid); and rural populations. Research in this area has typically been less focused on outcomes that may be of great interest to patients, such as enhancing their confidence and ability to navigate the healthcare system, ability to maintain functioning in the home, and productivity.

## Definition of Patient-Centered Outcomes Research

PCORI has defined patient-centered outcomes research, posted the definition for public comment, and incorporated these comments into the revised definition. Applications for research projects to PCORI must align with this definition, which is provided here and available at [www.pcori.org/what-we-do/pcor/](http://www.pcori.org/what-we-do/pcor/).

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

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the potential benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

To answer these questions, PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people
- Is inclusive of an individual’s preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health related quality of life
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination
- Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, availability of services, technology, and personnel, and other stakeholder perspectives

## Example Questions

The following research questions are meant as examples of the types of questions that patients, clinicians, or healthcare managers might ask, and which your research might help answer. They are expressed from the perspective of the patient and healthcare system. The list is by no means exhaustive.

- A 27-year-old man with diabetes, chronic back pain, and depression has been invited by his public hospital-based clinic to participate in a group visit program for patients with chronic conditions led by a behavioral care specialist and a health educator. How likely is it that he will benefit from this program compared to the current care offered, and what is the nature of the benefit?
- A 32-year-old metal fabricator is being encouraged by her employer to consider switching to a health plan that will cost less per month in terms of premiums, but have high deductibles and more copayments. Considering her personal situation, preferences, and concerns, what are the possible benefits and risks of the competing choices, and which makes most sense for her?
- A 47-year-old woman with rheumatoid arthritis has learned that her primary care doctor recently joined a large medical group affiliated with the community hospital. The previously freestanding office has become part of the system's patient-centered medical home, with a switch from paper records to the hospital's electronic health record and the addition of several new staff members, who sometimes call to discuss health issues. What should this woman know about the potential benefits or possible risks of this new way of primary care practice compared to her current or other care approaches?
- An 84-year-old woman with several chronic diseases is having increasing difficulties managing at home alone, but does not want to leave her home or neighborhood for a nursing home. What are the benefits and drawbacks of different programs or services that might help her stay at home and remain independent safely?
- An elderly man has been hospitalized four times in the past year for congestive heart failure. One challenge seems to be related to delays and poor communication during the transition from the hospital back to the primary care doctor. What could the hospital and primary care physician do to help this man reduce his chances of being hospitalized again?
- A world-class athlete has been advised by her sports medicine physicians that she needs arthroplasty in each knee. She is referred to a group of orthopedic surgeons and a hospital that are part of an accountable care organization. How will this organizational model impact her care, and what information about the ACO should she know to determine whether they will be likely to honor her strong preference for treatment that will return her to maximal function as quickly as possible?
- A 60-year-old man with end-stage renal disease is considering peritoneal home dialysis rather than hemodialysis to avoid transportation and having to miss so much work. What resources,

including telemonitoring or home visits by allied health personnel, might his medical center employ to support him in safely performing his dialysis?

- A 32-year-old uninsured single father and his 8-year-old daughter receive their primary and urgent care through a community health clinic. He wonders what impact (benefits and risks) the clinic's extensive use of cross-trained community health workers has on their care.

## Deadlines and Submission

This is a standing announcement, with three application deadlines per year. For this round, applicants must submit a Letter of Intent to PCORI no later than 5:00 PM EST on the due date shown in the Key Dates table (<http://www.pcori.org/funding-opportunities/funding-announcements/apply/>) via PCORI's online system. Full applications must be submitted to PCORI no later than 5:00 PM EST on the due date shown in the Key Dates table via the PCORI online system.

## Funding and Project Period Limits

We expect to fund approximately 27 projects totaling up to \$24 million in total costs under this PFA. Because the nature and scope of the proposed research is expected to vary widely from application to application, it is anticipated that the size and duration of each award will also vary.

Projects may not exceed three years in duration. Budgets may not exceed \$500,000 in direct costs per year. It is expected that, within these limitations, project budgets and duration will vary substantially, depending on the study design, needs for recruitment and/or primary data collection, required length of follow-up, and analytic complexity. To that end, PCORI will reserve a portion of funding for smaller (less than \$500,000 in total costs) and intermediate sized projects (less than \$1 million in total costs). PCORI encourages studies that can deliver findings promptly, including studies that take advantage of research infrastructure already in place and of longitudinal studies already underway. Currently funded CER studies may be considered for PCORI funding to support distinctive work related to extending follow-up, adding additional outcomes, or examining outcomes in key patient subgroups. Efficient use of research resources is a criterion that will be considered by merit reviewers and will also be reviewed by PCORI staff. The total amount awarded and the number of awards will depend on the quality, duration, and costs of the applications received.

Applicants wishing to propose prospective randomized trials or other complex studies that they believe will require more funding or longer duration may contact PCORI before the required deadline for the Letter of Intent to request permission to increase the budget beyond \$500,000 in direct costs in any project year or to extend the study duration beyond three years. PCORI does not guarantee that permission will be granted, and applicants should expect that the deliberative process may result in delaying the submission for one or more cycles.



## Step 2: Consider the Components

Now that you understand the research focus and priorities, you will need to determine if your organization and approach meet PCORI's other eligibility requirements. To do that, please consider the following important issues.

### Organizational Eligibility

Applications may be submitted by:

- Any private sector research organization, including any:
  - Non-profit organization
  - For-profit organization
- Any public sector research organization, including any:
  - University or college
  - Hospital or healthcare system
  - Laboratory
  - Unit of state or local government

All US applicant organizations must be recognized by the Internal Revenue Service. Foreign organizations and nondomestic components of organizations based in the United States may apply, as long as there is demonstrable benefit to the US healthcare system and US efforts in the area of patient-centered research can be clearly shown. Individuals may not apply.

In addition, investigators may serve as Principal Investigator (PI) on only one application for any individual PFA per cycle. An individual who is a PI may, however, participate in other applications (from the same or other organizations) in a different role, such as co-investigator or consultant.

### Characteristics of PCORI-Funded Research

Successful applicants for PCORI funds must:

1. Have a research team that includes patients and/or caregivers, as well as clinicians, health system managers, or other potential end-users of the study findings, along with researchers—each contributing the expertise that they have and participating actively in the design and implementation of the study and the dissemination of its results. A key concept here is ensuring that the research remains true to the interests of those who would use it.
2. Be familiar with the four questions of our patient-centered outcomes research definition, which can also be found at <http://www.pcori.org/what-we-do/pcor/>:
  - a. “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?”
  - b. “What are my options, and what are the potential benefits and harms of those options?”

- c. “What can I do to improve the outcomes that are most important to me?”
- d. “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?”

These questions articulate the needs of people as they make health care decisions. Applicants must clearly explain how their proposed research aligns with one or more of these questions.

3. Demonstrate that the proposed research question and project has the potential to provide truly important information that patients need to make decisions but that is not currently available. Think about what kind of information or support patients, clinicians, or health systems may need to effect changes that will bring us closer to the elimination of disparities—or about interventions that may make it feasible to adopt this information widely. What information would make the biggest difference to those who seek this change?
4. Propose to use PCORI resources efficiently in producing new knowledge. We aim to stretch our resources as far as possible because we recognize the vast information needs of patients. Research budgets will be used as one component to evaluate the use of resources, as detailed in Review Criterion #8 (see PCORI Review Criteria on pages 18-19). We are looking for approaches that are highly efficient without sacrificing methodological rigor. We are also interested in the potential for findings to be applied in multiple areas, independent of the disease studied.
5. Make clear how you are accounting for individual differences among patients and patient groups. Average results are useful, but we are also very interested in providing evidence that can be tailored to patient subgroups and individuals based on their clinical and demographic characteristics. We want products of the research that are scalable and generalizable—and can be customized for sites. We recognize that there are challenges in seeking evidence at these levels, but we hope that many applications will seek to provide insights about how individual patients may make use of the products of the research.

PCORI is interested in research that can be rapidly disseminated and implemented into clinical and community settings, yielding prompt improvements in patients’ decisions and the outcomes experienced. To that end, projects of shorter duration and projects that take advantage of existing research infrastructure and data are of great interest. Applications must include a dissemination and implementation assessment that discusses prospects for dissemination and considers possible barriers as well. For projects that produce important findings deserving dissemination, PCORI will consider subsequent applications that evaluate additional dissemination and implementation efforts.

A variety of study designs and analytic methods may contribute valid new knowledge. These include evidence syntheses, randomized comparisons at either the individual or cluster level, or various observational approaches (e.g. quasi-experimental studies). Qualitative methods may also be employed, either in mixed methods approaches or, potentially, as qualitative comparative studies. Evidence syntheses should follow rigorous standards accepted in the field, such as those published by the Agency for Healthcare Research and Quality (AHRQ) or the Institute of Medicine (IOM). Issues of possible

heterogeneity of treatment effects must be considered and discussed. Any planned analyses of subpopulations should be discussed. Inclusion of previously understudied population groups, including the elderly, children (if appropriate), and vulnerable populations, is particularly important. Randomized evaluations must be generalizable either by virtue of considering entire populations or by efficiently recruiting highly representative study populations rather than selected volunteers. Observational comparisons must employ study designs and analytic methods that convincingly protect against selection bias and other threats to validity. Applicants should specifically discuss the need to measure factors such as differential adherence to chosen treatments that could create apparent differences in effectiveness in clinical populations. Regardless of the particular methods employed, proposals are expected to use rigorous methodology. Applicants are encouraged to refer to the contents of the PCORI draft Methodology Report, which is posted at <http://pcori.org/assets/MethodologyReport-Comment.pdf>, in developing their research plan. However, since the draft report will not be finalized integrating feedback from public comment before the November 30<sup>th</sup>, 2012 application deadline, applications for this funding cycle are not required to adhere to the Report's standards. Adherence to the finalized Methodology standards will be required in future funding cycles.

Comparisons must be to relevant alternatives, which may include other interventions or clinical strategies designed to treat the same need, or to "usual care," or in some instances to no therapy. The research will ideally provide information about the range of outcomes that are experienced by and important to patients. These outcomes may include quality of life, ability to participate in desired activities, degree of suffering from pain or other symptoms, ability to live independently, and satisfaction with health care.

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 various populations. Populations of interest include those that are less frequently studied (e.g. the elderly; children, if appropriate; patients with multiple chronic conditions; patients with rare conditions) and other vulnerable populations, including those of low socioeconomic status, low literacy and/or numeracy, and patients groups known to experience disparities in health care and outcomes, such as racial/ethnic minorities. Alternatively, the study may focus primarily on comparative effectiveness of strategies for prevention, treatment, screening, diagnosis, or management in one or more of these populations of interest.

Please note that your application will be organized by and scored against the eight PCORI review criteria found at the end of this document.

## **Relevance to Patients**

Research proposals should clearly identify the relevant patient population, the health decision(s) examined in the proposed study, and the patient outcomes that will be affected by the research, as outlined in PCORI Review Criterion #4. As patient-centered research, the focus should be on the identification of the primary concerns and questions of patients and their caregivers.

Details of the required plan for patient and stakeholder engagement are described in the Application Guidelines (<http://www.pcori.org/assets/PFAguidelines.pdf>).

## Patient and Stakeholder Involvement

Persons representative of the population of interest, referred to here as patients, their caregivers, and clinicians, should be engaged in all phases of the research process, as outlined in PCORI Review Criterion #7: Research Team & Environment. Patients may include individuals who have or had the condition or who are at risk of the condition under study; it may also include patient surrogates or caregivers. In some instances, representatives of patient advocacy organizations may be appropriate research collaborators. Clinicians who face these decisions in collaboration with their patients are also relevant team members. Engagement should include participation in formulation of research questions; defining essential characteristics of study participants, comparators, and outcomes; monitoring of study conduct and progress; and dissemination of research results. In essence, patients and stakeholders must be important contributors throughout the research enterprise.

A key goal of patient engagement in research is to present information that best supports health decisions through generation of evidence relevant to patients, their caregivers, and clinicians. Patients and other key stakeholders should be meaningfully involved in the research team. The specific members of the team will vary from study to study.

Details of the required plan for patient and stakeholder engagement are in the Application Guidelines (<http://www.pcori.org/assets/PFAguidelines.pdf>).

## Dissemination and Implementation Assessment

PCORI is interested in funding studies that produce findings that can be readily disseminated and implemented—and are highly likely to be valued by patients and caregivers. To that end, it is important that potential facilitators and barriers to dissemination and incorporation into practice be assessed and anticipated. Applicants must provide a dissemination and implementation assessment as described in the PCORI Application Guidelines (<http://www.pcori.org/assets/PFAguidelines.pdf>).

## Reproducibility and Transparency of Research

The ability to replicate potentially important findings from PCORI-funded studies in other datasets and populations is essential to building confidence in the accuracy of these findings. To that end, we will support policies to promote sharing of study documentation (e.g. study protocol, programming code, data definitions) so that other researchers may replicate the findings in other populations. For large studies—those with direct costs greater than \$500,000 in any year—we will also require that applicants propose a plan for sharing of de-identified data, so that results may be reproduced by others in the same dataset. Whether data sharing is ultimately requested will depend on study findings and the availability of funds to support the process. Details of both requirements are in the Application Guidelines (<http://www.pcori.org/assets/PFAguidelines.pdf>).

## Inclusiveness of Different Populations

PCORI seeks to fund research that includes diverse populations with respect to age, gender, race, ethnicity, geography, or clinical status. PCORI recognizes that some proposed studies may represent important PCOR opportunities even in the absence of a broadly diverse population. However, the burden is on the applicant

in such cases to justify the importance of the study given the absence of diversity. Alternatively, PCORI is interested in the inclusion of previously understudied populations for whom effectiveness information is particularly needed, such as “hard-to-reach” populations or patients with multiple conditions.

## Protection of Human Subjects

PCORI adopts, by reference, the Human Subjects requirements of 45 CFR Part 46.

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### Step 3: Develop Your Application

There are three main parts of designing your project: (1) defining your research question and research strategy, including the study population and analytic approach; (2) describing the people who will comprise your research team and the institutions, organizations, and locations that will be involved; and (3) determining the budget. To better understand each of these steps and to find and complete the application forms, please see the PCORI Application Guidelines (<http://www.pcori.org/assets/PFAguidelines.pdf>).

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### Step 4: Know the Review Criteria

It is PCORI’s goal to make its funding decisions in a way that best supports our mission of improving patient-centered outcomes and in the most fair and transparent way possible. Below is an overview of PCORI’s review and decision-making process.

The PCORI review process includes four stages:

- Completeness, Compliance, and Eligibility Check
- Merit Review
- PCORI Deliberations
- Business Review

You should carefully read and thoroughly understand the PCORI review criteria, at the end of this document, before applying.

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### Step 5: Submit Your Application

To apply with PCORI, you must register with PCORI’s online system and submit both a timely Letter of Intent and a timely application. To learn more about the application process, go to the Application Guidelines at <http://www.pcori.org/assets/PFAguidelines.pdf>.

## PCORI Review Criteria

PCORI Criteria	Brief Description
<b>RESEARCH STRATEGY: Background and Significance</b>	
1. Impact of the condition on the health of individuals and populations	Refers to the current impact of the condition on the health of individuals and populations. Is the condition or disease associated with a significant burden in the US population, in terms of prevalence, mortality, morbidity, individual suffering, or loss of productivity? A particular emphasis is on patients with chronic conditions, including those patients with multiple chronic conditions.
2. Innovation and potential for improvement	Refers to the potential that the proposed research may lead to meaningful improvement in patient health, well-being, or quality of care. 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 change practice? Does the research question address a critical gap in current knowledge as noted in systematic reviews, guidelines 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? Do preliminary studies indicate potential for a sizeable benefit of the intervention relative to current practice? How likely is it that positive findings could be disseminated quickly to effect changes in current practice?
3. Impact on health care performance	Refers to the potential that the proposed research could lead to improvements in the efficiency of care for individual patients or for a population of patients. Does the research promise potential improvements in convenience or elimination of wasted resources, while maintaining or improving patient outcomes?
<b>RESEARCH STRATEGY: Relevance to Patients</b>	
4. Patient-centeredness	Is the proposed research focused on questions and outcomes of specific interest to patients and their caregivers? Does the research address one or more of the key questions mentioned in PCORI's definition of patient-centered outcomes research? Is the absence of any particularly important outcomes discussed?
<b>RESEARCH STRATEGY: Approach</b>	
5. Rigorous research methods	Refers to the use of appropriate and rigorous research methods to generate patient-centered evidence, including appropriate choice of study design and of analytic methods. How likely is it that the proposed study population, study design, and available sample size will yield unbiased, generalizable information with sufficient precision to be useful and reliable for patients, their caregivers, and clinicians
<b>RESEARCH STRATEGY: Inclusiveness of Different Populations</b>	
6. Inclusiveness of different populations	Does the proposed study include a diverse population with respect to age, gender, race, ethnicity, geography, or clinical status? Alternatively, does it include a previously understudied population for whom effectiveness information is particularly needed?

	<p>Does the study have other characteristics that will provide insight into a more personalized approach to decision making based on a patient’s unique biological, clinical, or sociodemographic characteristics.</p>
<p><b>PEOPLE AND PLACES</b></p>	
<p>7. Research Team and Environment</p>	<p>The research team must be appropriately trained and experienced to carry out the planned studies. Does the study team have complementary and integrated research expertise in implementing the study? Are relevant patients and other key users of the study information (e.g. caregivers, clinicians, health system, community, or policy makers) appropriately included on the team? Will the research environment contribute to the probability of success? Are features of the research environment, such as health system or community involvement or collaborative arrangements, described? Are institutional and community investment in the success of the research described?</p>
<p><b>BUDGET</b></p>	
<p>8. Efficient use of research resources</p>	<p>Does the budget appear to be reasonable in relation to the potential contribution of the research? Does the justification address the efficiency with which PCORI resources would be used? Are there opportunities to make the study more efficient? Are there additional benefits to a PCORI investment in this study through the creation of common data or infrastructure that could support future research?</p>

## About PCORI

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization created to help people make informed health care decisions and improve health care delivery. PCORI will commission research that is guided by patients, caregivers, and the broader healthcare community and will produce high integrity, evidence-based information.

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI will use a variety of forums and public comment periods to obtain public input throughout its work.

**Our Mission:** PCORI helps people make informed health care decisions and improves health care delivery and outcomes by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader health care community.

**Our History:** PCORI was created by the Patient Protection and Affordable Care Act of 2010 as a non-profit, nongovernmental organization. PCORI's purpose, as defined by the law, 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."

The statutory language defining PCORI is broad and authorizes research that will support a strong patient-centered orientation, inform better choices among alternative treatment and prevention strategies, and direct attention to individual and system differences that may influence strategies and outcomes. PCORI was designed to produce knowledge through the analysis and synthesis of existing research or the support of new research.