PCORI Funding Announcement: Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma

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<td>PCORI Online System Opens*</td>
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<td>Applicant Town Hall Session (Webinar)</td>
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* Application guidelines and application templates will be available on July 1, 2013, in the Funding Center (http://www.pcori.org/funding-opportunities/funding-center/).
About PCORI

PCORI was authorized by the Patient Protection and Affordable Care Act of 2010 as a non-profit, nongovernmental organization and is charged with helping 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.” It does this by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

PCORI’s strong patient-centered orientation directs attention to individual and system differences that may influence research strategies and outcomes. PCORI is charged with producing useful, relevant clinical evidence through the support of new research and the analysis and synthesis of existing research.

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.

Note: References were added to this announcement on July 5, 2013.
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1. Introduction

1.1. Purpose
The Addressing Disparities Program at the Patient-Centered Outcomes Research Institute (PCORI) seeks to fund comparative effectiveness research (CER) that will improve patient-centered outcomes among African-American and Hispanic/Latino individuals with asthma.*

Asthma is a chronic condition characterized by reversible airflow obstruction due to both airway hyper-responsiveness and inflammation of airways. It affects nearly 26 million Americans and disproportionately affects racial and ethnic minorities.1 African Americans are one of the populations at highest risk, with almost 4.5 million people (approximately 11%) affected in 2010 and significantly higher asthma-related morbidity and mortality rates compared to whites.2 Hispanic/Latino populations are also disproportionately affected; in 2010, 3.6 million US Hispanics/Latinos (approximately 7%) reported having asthma. Hispanic/Latino children have a greater chance of dying from asthma compared to non-Hispanic/Latino white children, though these increased risks are not evenly distributed across Hispanic/Latino subgroups.3

While evidence-based guidelines for asthma care have been available for 20 years, both African-American and Hispanic/Latino children have a lower likelihood than white children to be prescribed, or to follow, courses of asthma treatment.4 Complicating matters is evidence that across and within racial/ethnic groups, one-size-fits-all asthma care might not be optimal. Treatments beneficial to some groups may be less effective for others.5

In this PCORI Funding Announcement (PFA), we seek to fund studies that focus on reducing adverse outcomes due to poorly controlled asthma in African-American and Hispanic/Latino individuals, populations, and subgroups. More specifically, we seek CER that tests interventions to improve clinician and patient adherence to guidelines produced by the National Asthma Education and Prevention Program (NAEPP) of the National Heart, Lung, and Blood Institute.6 PCORI’s goal is to fund CER to identify optimal strategies for leveraging the NAEPP evidence-based guidelines to achieve equitable outcomes in asthma care. Studies that assess methods to improve adherence to these evidence-based guidelines coupled with interventions that are tailored to the needs of specific individuals and populations are of particular interest. Recognizing that the guidelines require a multidimensional approach to patient care, we are interested in studies that incorporate interventions at the community, home, and health system levels and that assess combinations of patient-education tools, home-environment interventions, asthma medications, and team-based approaches.

We are also particularly interested in CER that examines multidimensional interventions because the literature shows that disparities in health and health care are generally due to multifactorial causes. In

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*This funding announcement originated from PCORI’s accelerated process to generate PCORI Funding Announcements on specific high-impact topics. More information on this process is available at [Summary of Accelerated Process](https://pcori.org/assets/PCORI-Accelerated-Process-to-Generate-Targeted-Funding-Announcements1.pdf).
this regard, we are interested in interventions targeting enhanced communication, tailored patient educational or outreach materials, and other interventions focused on specific populations that might reduce disparities by improving adherence to guidelines. PCORI encourages CER that evaluates combinations of different therapies and mechanisms for integrating care, enhancing communication, and linking systems of care. In sum, PCORI intends to support comparisons of comprehensive and coordinated approaches to addressing disparities in asthma outcomes rather than research focused on comparing single factors related to improving adherence to guidelines.

Proposals may compare different interventions known to be effective that have been tailored to better meet the needs of one or more of the populations of interest, that are presented in an alternative context (e.g., home versus clinic visit, or different types of providers, such as nurse, social worker, or community health worker), or that compare strategic combinations of these.

A recently published Agency for Healthcare Research and Quality (AHRQ) Comparative Effectiveness Systematic Review states that “there is low to moderate evidence to support the use of decision support tools, feedback and audit, and clinical pharmacy support to improve the adherence of health care providers to asthma guidelines, as measured through health care process outcomes, and to improve clinical outcomes. There is a need to further evaluate health care provider-targeted interventions with a focus on standardized measures of outcomes and more rigorous study designs.”

PCORI’s aim is to fund CER to meet this need for evidence on how best to stimulate provider adherence to guidelines, but we also seek to examine methods to improve patient adherence (including factors such as family/community support of individuals) and to understand other factors that might contribute to reductions in asthma disparities. We recognize that adherence to practice guidelines alone will not be enough to achieve optimal asthma-related outcomes and eliminate disparities. Yet, improving adherence to appropriate guidelines can reduce inappropriate variations in care and is an important and necessary step toward reducing disparities, while improving quality and outcomes for all patients.

1.2. Funds Available
PCORI expects to fund projects totaling up to $17 million in total costs under this PFA.

1.3. Budgets and Project Period
Projects may not exceed three years in duration. Budgets may not exceed $4 million in total costs, including indirect costs, over the three-year period. We encourage projects of shorter duration or requesting less than this amount. Proposals will be reviewed and evaluated in their totality; however, project funds will be disbursed in two stages, with disbursal of Stage 2 funds (covering years 2 and 3)
contingent on successful performance in Stage 1 (year 1). Budgets in Stage 1 may not exceed $500,000 in total costs, and budgets for each subsequent year may not exceed $1.75 million in total costs.

We expect that Stage 1 will be used for setting a solid foundation for successful completion of the project. Stage 1 activities could include developing materials and protocols, conducting focus groups, tailoring educational tools, and obtaining clearances from all institutional and community partners. Stage 2 activities would focus on refining, implementing, and evaluating the interventions.

Because PCORI is seeking comprehensive and coordinated approaches to address disparities in asthma outcomes, applicants to this funding announcement need to have agreement/consensus among all partners (e.g., community-based organizations) regarding the intervention and approach described within the application. PCORI would like applicants to submit documentation of well-established, proven partnerships.

The application must include clearly specified, well-defined milestones and timelines for assessing progress in both Stage 1 and 2. In the application, milestones and timelines must be provided in a separate heading at the end of the “Approach” section for each stage.

Below are three points to consider regarding this staged approach:

1. Prior to funding an application, the PCORI Program Officer will contact the applicant to discuss the Stage 1 and 2 milestones and suggested recommendations from PCORI’s staff or review panel. The Program Officer and the applicant will negotiate and agree on a final set of approved Stage 1 milestones, which will be specified in the Notice of Award. These milestones will be the basis for judging the successful completion of the work proposed in Stage 1 and progress toward milestones in Stage 2.

2. Project Director(s)/Principal Investigator(s) will submit a progress report to PCORI during Stage 1 at the end of 11 months. It will include any revisions to the previously proposed Stage 2 aims. Receipt of this progress report will trigger an administrative review and a site visit by PCORI program staff to determine whether or not Stage 2 should be awarded.

3. Projects that have met the milestones for Stage 1 will be eligible for rapid transition to Stage 2. To ensure continuity of the work, PCORI will aim to release Stage 2 funds by month 12 or 13 of the project.

Applicants wishing to propose studies that will require more than $500,000 in total costs in year 1 or more than $1.75 million in total costs in years 2 and/or 3 may contact PCORI before the deadline for the Letter of Intent. PCORI does not guarantee that permission will be granted.

The nature and scope of the proposed research is expected to vary widely from application to application. It is also expected that 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.

PCORI strongly encourages studies that can deliver findings promptly, including studies that take advantage of tools previously developed, populations recruited for prior studies, or existing infrastructure (e.g., school-based programs or community-based healthcare workers). Currently funded studies may be considered for PCORI funding to significantly extend the scope of work to support distinctive comparative effectiveness research related to addressing treatment for uncontrolled asthma in African-American and Hispanic/Latino populations. However, PCORI is only interested in supporting and extending highly innovative studies that push beyond traditional concepts to move the scientific field forward and that show great promise for accelerating opportunities for improving asthma outcomes and reducing disparities.

1.4. Organization Eligibility

Applications may be submitted by any private sector research organization, including any:

- Non-profit organization
- For-profit organization

Applications also may be submitted by any public sector research organization, including any:

- University or college
- Hospital or healthcare system
- Laboratory or manufacturer
- 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. Organizations may submit multiple applications for funding. Individuals may not apply.

2. Overview

The Addressing Disparities Program at PCORI seeks to fund CER to improve patient-centered outcomes and reduce disparities in health and health care. This funding announcement focuses on African Americans and Hispanics/Latinos with uncontrolled asthma and aims to help these groups receive care according to their needs and achieve the best possible asthma-related health outcomes. Asthma is a condition that disproportionately affects African Americans and Hispanics/Latinos. These populations are less likely to receive guideline-concordant care for this condition, and asthma outcomes, including quality of life and mortality, are worse for African Americans and Hispanics/Latinos than their white counterparts.2,3 CER on how to improve guideline-concordant care for these populations might help improve asthma outcomes for these populations, thereby reducing disparities.
PCORI is entrusted by the public to fund research that matters to patients, their caregivers, and other stakeholders, including clinicians and their professional societies, hospitals, health systems, payers (insurance providers), purchasers (businesses and governments), industry, researchers, policy makers, and training institutions. PCORI seeks to change how research is conducted by emphasizing the role of diverse research teams that include varying perspectives. PCORI distinguishes itself by supporting research in which patients, caregivers, practicing clinicians, and the broader stakeholder community are actively engaged in generating research questions, reviewing proposals, conducting research, disseminating findings, promoting implementation of findings, and using results to understand and address patient and other stakeholder needs.

As such, PCORI is particularly interested in applications that involve organizations or programs that can help researchers design, implement, disseminate, and sustain effective interventions. We encourage proposals that include novel collaborations and testing of innovative delivery models, including by working with accreditation organizations, credentialing bodies, educational enterprises, patient advocacy groups, professional societies, and subspecialty societies. Applicants are strongly encouraged to collaborate with appropriate institutions or organizations to achieve this end.

2.1. Background
Asthma is a disease that causes variable and recurrent narrowing and inflammation of the airways, and it affects nearly 26 million Americans. Asthma is a chronic condition that affects children and adults of all ages. Symptoms vary in strength and frequency and include coughing, wheezing, shortness of breath, and tightening of the chest. While asthma cannot be cured, all patients need medications for treating acute symptoms and exacerbations, and patients with persistent asthma require daily, long-term-control medications (of which there are several classes, given according to the level of asthma severity and asthma control). In 2008, almost 3.2 million adults and children reported missing work or school and visiting an emergency department or urgent care center for asthma-related illness. Of those visiting an emergency department or urgent care center, approximately 850,000 adults and children required urgent outpatient care for an acute asthma attack.

Asthma disproportionately affects some racial and ethnic minorities. African Americans are one of the populations at highest risk, with almost 4.5 million people (approximately 11%) reported to be affected in 2010. Hispanic/Latino populations are also disproportionately affected. In 2010, 3.6 million US Hispanics/Latinos (approximately 7%) reported having asthma. Importantly, however, these increased rates of asthma are not evenly dispersed across ethnic subgroups. For example, the asthma rate among Puerto Ricans is 2.6 times that of the broader Hispanic/Latino population. The high prevalence of asthma is accompanied by worse health outcomes in both African-American and Hispanic/Latino populations. Overall rates of asthma-related hospitalizations and deaths in the United States have declined over the last 20 years due to advancements in asthma research and treatment and the development and use of evidence-based guidelines. Compared to whites with asthma, African Americans with the condition have poorer quality of life, more asthma control problems, and higher rates of emergency department visits and hospitalizations. African-American children are three times as likely as white children to seek asthma care in the emergency department and to have an asthma-related hospitalization.
children are almost twice as likely as white children to seek asthma care in the emergency department and to have an asthma-related hospitalization. Hispanic/Latino children also have a greater chance of dying from asthma compared to non-Hispanic/Latino whites.  

Various social determinants, including socioeconomic and systems factors, have been associated with disparities in asthma prevalence, management, morbidity, and mortality. These determinants also include cultural and language barriers, health system barriers, poverty, genetics, health literacy and numeracy, and exposure to environmental stressors.  

Asthma outcomes for these populations might be improved by increased adherence to evidence-based guidelines. First published in 1991 and most recently updated and re-issued in 2007, the NAEPP guidelines provide clinicians with a range of acceptable approaches for the diagnosis and management of asthma and define general practices that meet the needs of most patients. In asthma care, NAEPP guideline recommendations have been shown to be efficacious in inner-city and poor pediatric populations and other groups at risk for experiencing disparities. Yet the NAEPP guidelines are not widely implemented, particularly for these groups. Barriers contributing to non-adherence to guidelines may include lack of clinician awareness and doubts about whether the guidelines are effective, limited clinician confidence in patients’ ability to implement the guidelines, clinical inertia, practice barriers, and time constraints. Patient preferences and a variety of constraints on patient adherence may also play important roles.  

Similarly, disparities in health care are multifaceted, and solutions to improve guideline-directed care and address disparities in treatment and outcomes will need to address these complex factors.  

2.2. Research Areas of Interest  
PCORI encourages applicants to examine and compare the relative effectiveness of various models and tools to enhance clinician and patient communication (e.g., use of mobile technology), improve systems of care (e.g., evaluate models that look at data integration), improve integration of care (e.g., team-based care), or any combination of these that might have an impact on adherence to evidence-based guidelines.  

PCORI is interested in CER and interventions within the following domains, particularly in terms of how they facilitate adherence to evidence-based asthma guidelines:  

- Physician and patient communication is a key component of the clinical encounter and can affect patient behavior and adherence. Limited language skills and/or literacy can influence patient and clinician communication, leading to poor outcomes. Implementations of technology (e.g., mobile messaging), social media, and other innovative education methods tailored to specific groups are promising ways to improve communication and patient-centered outcomes. Few studies have examined whether or how different patient and clinician communication or engagement strategies might affect outcomes in asthma, and even fewer studies have evaluated how technology and innovative methods to facilitate self-management and promote patient-centered care may improve outcomes for uncontrolled asthma in these
populations. PCORI is particularly interested in how improvements in this domain may facilitate increasing adherence to evidence-based asthma guidelines.

- Systems and settings for asthma care delivery—including healthcare delivery organizations, schools, community-based organizations, work places, and homes—can interact to affect the health outcomes of individuals with asthma. Some interventions to improve asthma outcomes within these systems or environments have been studied, but the integration of data sources within and across these systems poses great challenges. It has been difficult for researchers, clinicians, and patients to harness the necessary data to inform the care of patients with asthma. Some of these challenges include variable data quality, lack of standardized data elements, compliance with the Health Insurance Portability and Accountability Act (HIPAA) and data-protection requirements, ineffective exchange and linking of information, and lack of real-time access to information. PCORI is interested in CER and interventions that address approaches to integrating and using existing data in multiple systems to identify high-risk asthma populations, which could serve to facilitate adherence to evidence-based asthma guidelines.

- Effective integration of care could influence response to therapy for uncontrolled asthma, but little research has been conducted in this area. For asthma treatment and care, a variety of different health professionals have been shown to influence health outcomes, overall quality of life, and productivity in children, adolescents, and adults. The roles of nurse case managers, community health workers, and physicians making home visits have been evaluated. Pharmacists can also play an important role in patient education and engagement. Trials conducted outside the United States reported that using community pharmacists as educators can decrease healthcare utilization and reduce asthma severity. Few studies have examined integration of physician care and care provided by other healthcare providers in patient education, medication management, symptom control, quality of life, and patient-centered outcomes. Also, little research has compared models of integrating care (e.g., nurse case managers versus community health workers) to understand the mechanisms by which they might improve care or to determine whether one model is superior to another. A limited number of studies have evaluated models to improve transitions in care for patients with asthma (e.g., from hospital or emergency department to primary care; pediatric to adolescent to adult care), particularly for African-American and Hispanic/Latino populations. Studies are needed that evaluate the use of electronic technologies to improve health literacy and asthma self-management skills or to link care provided in different settings.

Research studies should focus on improving patient-centered outcomes and reducing disparities in asthma outcomes in African-American and Hispanic/Latino populations. We recognize that adherence to guidelines alone may not be enough to achieve optimal asthma-related patient-centered outcomes and reduce disparities; however, optimizing and tailoring interventions to improve adherence to guidelines for minority patients and populations is an important and necessary step. PCORI’s goal is to fund CER to identify optimal strategies for leveraging the NAEPP evidence-based guidelines to achieve equitable outcomes in asthma care.
We seek applications that specifically examine relevant patient-centered outcomes that can be measured within a three-year period. Applicants are encouraged to use the standardized definitions and methodologies for data collection described in *Standardizing Asthma Outcomes in Clinical Research: Report of the Asthma Outcomes Workshop*. These outcomes include, but are not limited to:

- Missed days of school or work
- Medication use
- Activity limitations
- Parental/patient perceptions of care
- Quality of life
- Asthma control scores, symptom frequency, or pooled symptom scores (e.g., cough, night symptoms, sleep quality, and maximum tolerated activity)
- Experienced side effects of drugs
- Patient self-management skills and efficacy
- Medication adherence
- Emergency department and hospital visits

PCORI is particularly interested in applications that include organizations or programs that can help researchers design, implement, disseminate, and sustain effective interventions. We encourage proposals that include novel collaborations and the testing of innovative delivery models, including working with accreditation organizations, credentialing bodies, educational enterprises, patient advocacy groups, professional societies, and subspecialty societies. Applicants are strongly encouraged to collaborate with appropriate institutions or organizations to achieve this end.

### 2.3. Sample Questions

The following research questions are meant as examples of the types of questions that your research may help answer. This list is by no means exhaustive. **All questions must have a comparative component.**

- Among patients with frequent emergency department visits, does the addition of a team-based, patient-centered, and culturally and socially tailored care-management intervention improve adherence and asthma outcomes, compared to an educational intervention in the context of usual care?

- Do different models for team-based care (e.g., using different combinations of nurse case managers community health workers, physicians, and pharmacists) and combining clinical care with home visits improve provider and patient adherence to guidelines?

- Does targeting interventions to “hot spots” of high-risk communities increase improvements in asthma outcomes for individuals?

*Available at jacionline.org/issues?issue_key=S0091-6749%2812%29X0003-4*
• Does a patient-centered medical home with tailored case management and home visits improve outcomes compared to standard clinic care?

• Do providers outside the clinical system (e.g., community health workers, home visitors for environmental assessments and counseling, patient navigators) improve adherence and asthma outcomes compared to optimal pharmacologic therapy alone?

• Do programs linking provider behavior change, adherence programs for patients, and school monitoring and support programs result in greater improvements in asthma outcomes than patient-adherence programs alone?

• Compared to traditional educational methodologies, do interventions using electronic medical records improve provider adherence and do electronic tools improve health literacy and adherence?

• Do organizational changes in the healthcare system result in greater improvements in provider adherence and patient asthma outcomes than provider education alone?

• As compared to usual care, do patient adherence and asthma outcomes improve when patients engage (or their caregivers engage) with current technologies such as video storytelling, smart phones, or social media for communication about asthma?

• Do asthma-control programs integrated with broader health and wellness programs (e.g., obesity initiatives or healthy housing/communities programs) or with disease-management programs for patients with other health problems (e.g., obesity, diabetes, hypertension) improve outcomes compared to programs focused exclusively on asthma?

• Do asthma programs provided in different service settings (e.g., hospitals, child care centers and schools, pharmacies, community centers, churches) generate differential outcomes?

Certain physiological measurements, such as lung function (measured as FEV1), are strongly linked to complications or other outcomes that patients care about. Therefore, an application to PCORI that proposes to conduct a study comparing two approaches to helping people control their asthma using the FEV1 as a measure of control would be well aligned with PCORI’s focus on patient-centeredness, assuming that the study would also compare the two approaches’ effects on other relevant outcomes that are also important to patients, such as side effects, activity limitations, or patient-reported level of asthma control.
To be competitive for a PCORI contract, an application must demonstrate that its proposed research question(s) and outcomes will matter to patients and/or other stakeholders, such as clinicians, payers, and policymakers.

2.4. Other Programmatic Consideration

Applications to this PFA will be considered non-responsive if research is proposed that:

- Conducts a formal cost-effectiveness analysis in the form of dollar-cost per quality-adjusted life-year (including non-adjusted life-years) to compare two or more alternatives
- Directly compares the costs of care between two or more alternative approaches as the criteria for choosing the preferred alternative

However, PCORI does have an interest in studies that address questions in conditions that lead to high costs to the individual or to society. This is included in our criterion on impact of the condition on the health of individuals and populations. PCORI is also interested in studies that examine differentials in healthcare resources or costs as a determinant of, or barrier to good outcomes. Examples include ways in which out-of-pocket costs may constitute a barrier to the receipt of care. PCORI also considers it important for applicants to discuss cost-related issues such as resources needed to replicate or disseminate a successful intervention. And PCORI is interested in evaluation of interventions to reduce health system waste or increase health system efficiency. Proposals that include studies of these issues without utilizing a formal cost-effectiveness analysis or directly measuring and comparing costs of care of alternatives will be considered responsive.

2.5. Definition of Patient-Centered Outcomes Research

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

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system features to inform decision making, highlighting comparisons of 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; and
• Investigates (or may investigate) optimizing outcomes while addressing burdens to individuals, availability of services, technology, and personnel, and other stakeholder perspectives.

PCORI funds patient-centered outcomes research, a type of comparative outcomes research. The research PCORI funds require inclusion of the patient perspective in the research. To be considered responsive to PCORI, applications to this PFA must describe research that:

• Studies the benefits and harms of different interventions and strategies, which can be delivered in actual settings. By “actual settings” we mean that the research evaluates treatments as they are delivered and received in typical clinical settings, not just in restricted trials of experimental care or at selected academic centers. PCORI is interested in innovative studies that can help patients and other stakeholders make informed decisions about their health care and health outcomes.

• Compares at least two alternative approaches. The types of interventions examined can include specific drugs, devices and procedures, as well as other types of alternatives, such as medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies. "Usual care" or no specific intervention may be an appropriate comparator, provided that this is a realistic choice faced by patients and other stakeholders (e.g., choosing not to have a Prostate-Specific Antigen [PSA] test).

• Compares health outcomes that are meaningful to the patient population under study.

3. Elements of PCORI-Funded Research

3.1. Technical Requirement and Review Criteria

Applicants will need to determine if their organization, proposed study, and approach meet PCORI's technical requirements and review criteria for a successful project, which are described below (also see Table 1, pages 16–17, for review criteria):

1. The application demonstrates that the condition imposes a significant burden on the health of individuals and/or populations.

2. The application explains how the results of the proposed study:
   • Would likely improve health care and patient outcomes
   • Would likely improve the efficiency of health care.

3. The application demonstrates strong technical merit, including:
   • A clear research plan with rigorous design and analytic methods
   • Key project milestones clearly articulated
• A strong research team
• A supportive research environment
• A diverse population with respect to age, gender, race, ethnicity, clinical status; or
• A defined population for which effectiveness information is particularly needed.

4. The application demonstrates patient-centeredness through:
   • Including outcomes that are meaningful to patients and other stakeholders
   • Research that addresses one or more questions of clear importance to patients

5. The application demonstrates a commitment to patient and stakeholder engagement through the integration of patients and stakeholders in key elements of the proposed project including:
   • Participation in formulation of research questions
   • Defining essential characteristics of the study, participants, comparators and outcomes
   • Monitoring study conduct and progress
   • Dissemination of research results

The specific research questions, specific populations to which the research is intended to apply, and specific research settings will all inform the nature of appropriate patient and stakeholder engagement.
### Table 1. PCORI Review Criteria and Description

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<td><strong>1. Impact of the condition on the health of individuals and populations</strong>&lt;br&gt;• Impact of the condition on the health of individuals and populations&lt;br&gt;  ▪ 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?&lt;br&gt;  ▪ Does it impose a significant burden on a smaller number of people who have rare diseases?&lt;br&gt;  ▪ A particular emphasis is on patients with chronic conditions, including those patients with multiple chronic conditions.</td>
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<td><strong>2. Potential for the study to improve healthcare and outcomes</strong>&lt;br&gt;Refers to the potential for the proposed research to lead to meaningful improvement in the quality and efficiency of care and to improvements in outcomes that are important to patients.&lt;br&gt;• Potential for the study to improve healthcare and outcomes&lt;br&gt;  ▪ Does the research question address a critical gap in current knowledge as noted in systematic reviews, guideline development efforts, or previous research prioritizations?&lt;br&gt;  ▪ Has it been identified as important by patient, caregiver, or clinician groups?&lt;br&gt;  ▪ Do wide variations in practice patterns suggest current clinical uncertainty?&lt;br&gt;  ▪ 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?&lt;br&gt;  ▪ Do preliminary studies indicate potential for a sizeable benefit of the intervention relative to current practice?&lt;br&gt;  ▪ How likely is it that positive findings could be disseminated quickly and effect changes in current practice?</td>
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<td><strong>3. Technical merit</strong>&lt;br&gt;Refers to inclusion of the following:&lt;br&gt;• Technical merit&lt;br&gt;  ▪ Clear research plan with rigorous methods and key milestones clearly articulated&lt;br&gt;  ▪ Research Team has appropriate expertise and project organizational structure is appropriate for the study&lt;br&gt;  ▪ Research Environment is sufficient to support conduct of the work; appropriate resources are available.&lt;br&gt;  ▪ Includes diverse population with respect to age, gender, race, ethnicity, and clinical status as appropriate for the study&lt;br&gt;  ▪ Focuses on defined population for whom effectiveness information is particularly needed</td>
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### 4. Patient-centeredness

- Is the proposed research focused on questions that affect 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?

### 5. Patient and stakeholder engagement

- Does the proposal describe how patients and stakeholders were or will be identified and engaged in the research?
- What are the roles of patients and key stakeholders in formulating the study’s hypotheses and design and in the study’s conduct and dissemination of results?
- What roles do patients and stakeholders have in any planned dissemination or implementation plans?

Applications need to demonstrate patient and stakeholder engagement through the integration of patients and stakeholders in the development of the research plan and in key elements of the proposed project including:

- Participation in formulation of research questions.
- Defining essential characteristics of the study, participants, comparators, and outcomes.
- Monitoring study conduct and progress.
- Dissemination of research results.

If the project has not included patient and stakeholder engagement (for example, in the area of analytic methods), has the application justified their non-inclusion?

If engagement is not applicable, explain why it is not.

### 3.2. Additional Guidance and Characteristics

#### Dissemination and Implementation Potential

In addition to the elements described above, which also represent the criteria by which we review proposed projects, PCORI is interested in research that can be rapidly disseminated and implemented into clinical and community settings, facilitating improvements in patients’ and stakeholders’ decision-making about health care. Therefore, applications should include a section that describes the potential for disseminating and implementing the results of your work in other settings. We also request that you describe possible barriers to dissemination and implementation of your work in other settings. Please note, we are asking you to describe the potential for dissemination and implementation. PCORI does not expect you to undertake this dissemination and implementation work at this juncture. For projects that produce important findings, PCORI will consider subsequent applications that support dissemination and implementation efforts through separate funding announcements.
Methodological Considerations

Regardless of study design, proposals must adhere to all relevant PCORI Methodology Standards. A variety of study designs and analytic methods may contribute valid new knowledge. These include 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. Issues of possible heterogeneity of treatment effects must be considered and discussed. 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. Comparisons must be to relevant alternatives—such as other interventions or clinical policies designed to address the same need in the same or in a different healthcare system, or to a previous approach used within the same system—or to “usual care.”

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 comparative effectiveness may be examined. 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. 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 various populations. Populations of interest include those that are less frequently studied. PCORI has developed the following list of priority populations to guide our efforts in research and engagement, which includes:

- Racial and ethnic minority groups
- Low-income groups
- Women
- Children (age 0-17)
- Older adults (age 65 and over)
- Residents of rural areas
- Individuals with special health care needs, including individuals with disabilities
- Individuals with multiple chronic diseases
- Individuals with rare diseases
- Individuals whose genetic make-up affects their medical outcomes
- Patients with low health literacy/numeracy and limited English proficiency
- Lesbian, gay, bisexual, or transgender (LGBT) persons

* Available at pcori.org/assets/PCORI-Methodology-Standards.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. PCORI 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—PCORI requires that applicants propose a plan for sharing of de-identified data, so that results may be reproduced by others in the same dataset.

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

If the proposed research will involve human subjects, refer to the Supplemental Instructions for Preparing the Protection of Human Subjects Section of the Research Plan in Part II of the Instructions for the PHS 398 Form, as found on the National Institutes of Health (NIH) website.*

Note: PCORI requires engagement in the research by patients and/or other stakeholders, as research partners. Research subjects protection requirements do not apply to co-investigators, members of the research team, or research partners.

4. Submission Guidelines

4.1. Submission Procedures
To apply, you must register with the PCORI Online System and submit both a required Letter of Intent and a full application in accordance with the key dates and funding described in this funding announcement.

For more information, please see the PCORI Funding Center† and the PCORI Application Guidelines‡.

4.2. Project Period
This is a one-time opportunity for studies lasting up to three years.

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* Available at grants.nih.gov/grants/funding/phs398/phs398.doc
† Available at pcori.org/funding-opportunities/funding-announcements/funding-center/
‡ Available at pcori.org/assets/2013/07/PCORI-Application-Guidelines-Asthma-PFA-062813.pdf
5. References


