



Patient-Centered Outcomes Research Institute

Draft National Priorities for
Research and Research Agenda

Revised in Response to Public Comment
April 25, 2012

For Consideration for Approval by PCORI Board of Governors
May 21, 2012

Draft National Priorities for Research and Research Agenda

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Introduction

The **Patient-Centered Outcomes Research Institute (PCORI)** is an independent, nonprofit health research organization authorized by the Patient Protection and Affordable Care Act of 2010. Its mission is to fund research that offers patients and caregivers the information they need to make important healthcare decisions.

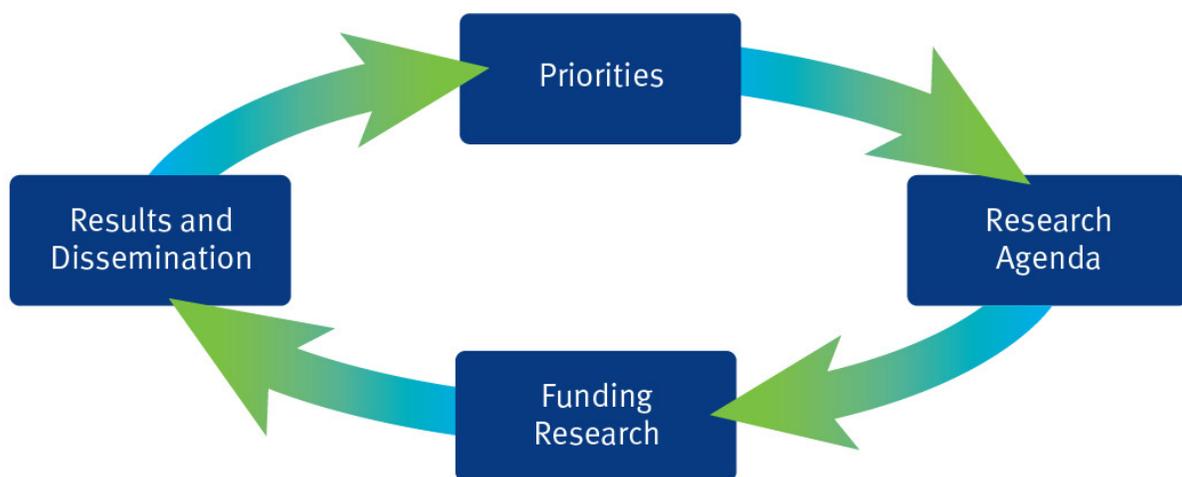
PCORI focuses on comparative clinical effectiveness research, studies that compare options for preventing and diagnosing disease and providing treatment and care. The institute is charged with pursuing this mission by:

1. Identifying National Priorities for Research.
2. Creating a Research Agenda based on identified priorities.
3. Funding research consistent with these priorities and agenda.
4. Providing patients and their caregivers with useful research information.

PCORI sees this as a natural set of mutually supportive activities that provide not just baseline guidance for its efforts but a process by which that work, informed by a broad array of stakeholder input, can evolve to meet the needs of patients, caregivers and clinicians over time.

This document outlines how PCORI's National Priorities for Research and initial Research Agenda were drafted, offered for and revised based on public comment. The draft National Priorities for Research and Research Agenda will be considered for approval by the Board of Governors at its public meeting in Denver, CO, on May 21, 2012.

Figure 1: PCORI's Path from Priorities to Research Patients Can Use



National Priorities for Research and Research Agenda

I. Statutory Requirements and Draft Development Process Overview

As described in the Patient Protection and Affordable Care Act of 2010 (the Act), one of the first responsibilities of the Patient-Centered Outcomes Research Institute (PCORI) was to establish and publish for comment a draft National Priorities for Research and a Research Agenda. The priorities and agenda are intended to lay the foundation for a portfolio of comparative clinical effectiveness research (CER) that addresses PCORI's statutory purpose:

“To assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations.”

The Act does not specify either the content or form of the Priorities or Agenda. Previous priority-setting and research agenda-setting efforts have varied greatly in form and degree of specificity in their final recommendations, depending on their intended uses. The Act does, however, point out a set of criteria (the PCORI Criteria) to be considered in formulating the Priorities and Agenda:

“...identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care, the potential for new evidence to improve patient health, well-being, and the quality of care, the effect on national expenditures associated with a health care treatment, strategy, or health conditions, as well as patient needs, outcomes, and preferences, the relevance to patients and clinicians in making informed health decisions, and priorities in the National Strategy for quality care established under section 399H of the Public Health Service Act that are consistent with this section.”

In the fall of 2011, workgroups of PCORI's Board of Governors formed to address both the National Priorities for Research and the Research Agenda. Along with PCORI staff and members of the Methodology Committee, these workgroups:

- examined the processes and products of other, recent priority- and agenda-setting efforts;
- reviewed the PCORI criteria specified above;
- worked to ensure that the working definition of patient-centered outcomes research (PCOR) and its core premise of keeping the patient’s voice central are implemented in PCORI-funded research;
- presented the status of deliberations and requested feedback at PCORI’s public board meetings; and
- engaged with, updated and received input from stakeholder groups through a number of public presentations and other modes of communication.

PCORI posted a draft National Priorities for Research and Research Agenda document for public comment from January 23, 2012 to March 15, 2012. Public comment was solicited through the PCORI website, email, postal mail and a National Patient and Stakeholder Dialogue event (and webcast) held in Washington, D.C. on February 27, 2012. PCORI received and analyzed a total of 474 formal comments; a summary of the analysis is available on the PCORI website: www.pcori.org/what-we-do/priorities-agenda.

At a special public webinar and teleconference held on April 25, 2012, the PCORI Board of Governors voted to accept the recommendations of the Board of Governors Program Development Committee for changes to draft National Priorities and Research Agenda, based on the analysis of public comments, and to revise the draft accordingly. This document reflects those changes and will be considered for approval by the Board of Governors at its public meeting in Denver, CO, on May 21, 2012.

II. PCORI’s National Priorities for Research

The development of the first set of PCORI’s first set of National Priorities for Research was led by the National Priorities Workgroup, a subgroup of the Board of Governors Program Development Committee. The workgroup received input from the committee, the Board, PCORI’s Methodology Committee, and stakeholders.

A Strong Foundation of Research Prioritization

To develop the National Priorities for Research, PCORI reviewed nine previous national efforts to prioritize comparative effectiveness research and related healthcare activities. Five of the efforts were by non-governmental organizations: the Institute of Medicine (twice); National Pharmaceutical Council; National Priorities Partnership; and the National Quality Forum. Four were by federal agencies or councils: Agency for Healthcare Research and Quality (AHRQ);

Federal Coordinating Council for Comparative Effectiveness Research; National Prevention, Health Promotion and Public Health Council; and U.S. Department of Health and Human Services (HHS). PCORI examined the degree to which each of these efforts had involved significant stakeholder engagement and public input. Table 1 lists these efforts and their method of receiving public input.

Table 1: Comparative Effectiveness Research Prioritization Efforts	
Priority Setting Organization	Method of Receiving Public Input
Institute of Medicine: Priority Areas for National Action: Transforming Health Care Quality (2003)	<ul style="list-style-type: none"> ▶ No formal public comment period
Agency for Healthcare Research and Quality (AHRQ): What is the Effective Health Care Program? and the Medicare Prescription Drug, Improvement, and Modernization Act of 2003	<ul style="list-style-type: none"> ▶ In-person and webinar town hall meetings ▶ Docket for comment submission ▶ Patient/consumer/clinician involvement
National Quality Forum: National Priorities for Healthcare Quality Measurement and Reporting (2004)	<ul style="list-style-type: none"> ▶ Patient/consumer involvement
National Priorities Partnership: National Priorities and Goals: Aligning Our Efforts to Transform America’s Healthcare (2008)	<ul style="list-style-type: none"> ▶ Input from 50 stakeholder organizations
Institute of Medicine: Initial National Priorities for Comparative Effectiveness Research (2009)	<ul style="list-style-type: none"> ▶ In-person stakeholder meeting ▶ Web-based questionnaire
Federal Coordinating Council for Comparative Effectiveness Research: Report to the President and the Congress on Comparative Effectiveness Research (2009)	<ul style="list-style-type: none"> ▶ 3 listening sessions ▶ 92 panelists testified ▶ >300 entities commented
U.S. Department of Health and Human Services (HHS): National Strategy for Quality Improvement in Health Care (2011)	<ul style="list-style-type: none"> ▶ >300 entities commented
National Prevention, Health Promotion, and Public Health Council: National Prevention and Health Promotion Strategy (2011)	<ul style="list-style-type: none"> ▶ Engagement sessions ▶ National webinars ▶ Sector outreach ▶ Email/Web ▶ Letters from organizations
National Pharmaceutical Council: Lessons from Prior Efforts and Opportunities for Prioritization of Comparative Effectiveness Research (2011)	<ul style="list-style-type: none"> ▶ No formal public comment period

In reviewing these nine priority-setting processes, PCORI noted that seven of them included significant public input. These seven were reviewed further to determine their key priorities and criteria for prioritizing health research. Ten priorities were identified (see Table 2). The first five priority areas (prevention, acute care, care coordination, chronic disease care, and palliative care) encompass the complete health cycle from staying healthy to treating conditions to reducing pain and suffering. The second five priority areas (patient engagement, safety, overuse, information technology (IT) infrastructure, and the impact of new technology) include issues that are systemic in nature to health care. All but one of the 10 priorities (impact of new technology) appear in at least three of the seven processes. Prevention appears in all seven of the processes and patient engagement in six.

Table 2: Ten Comparative Effectiveness Research Priority Areas from Existing Literature

Priority-Setting Organization	Prevention	Acute Care	Care Coordination	Chronic Disease Care	Palliative Care	Patient Engagement	Safety	Overuse	IT Infrastructure	Impact of New Technology
Agency for Healthcare Research and Quality	✓	✓	✓	✓	✓			✓		
National Quality Forum	✓	✓	✓	✓	✓	✓	✓		✓	
National Priorities Partnership	✓		✓		✓	✓	✓	✓		
Institute of Medicine (2009)	✓		✓	✓	✓	✓	✓	✓	✓	✓
Federal Coordinating Council for Comparative Effectiveness Research	✓	✓				✓	✓		✓	
U.S. Department of Health and Human Services	✓		✓			✓	✓			
National Prevention, Health Promotion, and Public Health Council	✓					✓				

Developing the National Priorities for Research

The 10 priority areas from previous prioritization processes were analyzed in light of PCORI's working definition of "patient-centered outcomes research," (PCOR) which approaches research questions as information needs, without regard to where a patient is in the disease continuum, from healthy, to symptomatic, to facing a chronic condition. The PCOR definition approved by PCORI's Board of Governors is provided on the PCORI website (www.pcori.org/what-we-do/pcor/). This prioritization process identified five cross-cutting areas where additional health research is needed to give patients and those who care for them more information to support decision-making.

PCORI's national priorities can be applied to and used to advance the quality of information for any health condition or disease where evidence is lacking or current decision-making is suboptimal. PCORI, at this early stage of its work and of patient-centered outcomes research as a discipline, does not want to focus on a narrower set of questions or healthcare conditions, nor does it want to exclude any diseases or conditions.

The five priorities were developed in light of PCORI's statutory requirements, its working definition of patient-centered outcomes research, and previous research prioritization efforts. They are:

1. **Assessment of Prevention, Diagnosis, and Treatment Options** - Comparing the effectiveness and safety of alternative prevention, diagnosis, and treatment options to see which ones work best for different people with a particular health problem.
2. **Improving Healthcare Systems** - Comparing health system-level approaches to improving access, supporting patient self-care, innovative use of health information technology, coordinating care for complex conditions, and deploying workforce effectively.
3. **Communication and Dissemination Research** - Comparing approaches to providing comparative effectiveness research information, empowering people to ask for and use the information, and supporting shared decision-making between patients and their providers.
4. **Addressing Disparities** - Identifying potential differences in prevention, diagnosis or treatment effectiveness, or preferred clinical outcomes across patient populations and the healthcare required to achieve best outcomes in each population.
5. **Accelerating Patient-Centered Outcomes Research and Methodological Research** - Improving the nation's capacity to conduct patient-centered outcomes research, by building data infrastructure, improving analytic methods, and training researchers, patients and other stakeholders to participate in this research.

These five broad areas comprise PCORI's National Priorities for Research and encompass the patient-centered comparative clinical effectiveness research PCORI will support. As PCORI's work progresses and needs evolve, and particularly as PCORI continues to engage in a transparent manner with a broad range of stakeholders in health care – patients especially – it is possible that PCORI may develop additional national priorities for research.

III. PCORI's Research Agenda

This initial Research Agenda was developed by a second working group of the Program Development Committee, in collaboration with the Methodology Committee and PCORI staff. The process began by considering the five areas proposed as National Priorities for Research described in the first part of this document. To these priorities, PCORI applied the criteria provided in the Act. The resulting Research Agenda contains a set of more specific research areas within each priority. Each area represents a line of research inquiry that addresses currently unmet needs of patients, their caregivers, clinicians and other healthcare system stakeholders in making personalized healthcare decisions across a wide range of conditions and treatments.

Establishing the Scope of the Research Agenda

This document is intended to address the statutory requirement for PCORI to publish an agenda to describe and guide the research that it will fund. By design, this document does not cover all the activities that PCORI sees as part of its mission. For instance, it does not describe how PCORI intends to invest in efforts to maintain active engagement with patients and all other stakeholder groups over time. It does not describe how PCORI, in partnership with AHRQ and HHS will invest in efforts to disseminate research findings or build capacity for patient-centered outcomes research through training programs or infrastructure development.

This document is the foundation upon which the first set of PCORI funding announcements will be developed. Taken together, the series of funding announcements that PCORI will produce, beginning in mid-2012, will create a portfolio of research that builds from this agenda and is consistent with Congress' intended purpose.

PCORI intends to be a learning organization; it will continue to evolve as it gains experience. There is the strongest possible commitment to a patient-centered focus, which means that the interests of patients and caregivers will guide us. In each opportunity to improve, PCORI will seek to be ever more attentive to the needs of patients and caregivers. When adopted after public comment, this initial Research Agenda will present a broad sweep of current research possibilities, encouraging the community to think boldly about specific opportunities and to describe how a proposed project or initiative aligns with PCORI's criteria. With time, PCORI

expects its Research Agenda to be updated and refined based upon more specific analyses of where current gaps exist and where patient-centered outcomes research can have the most impact. Each update will be achieved through a “due diligence” process that includes: dialogue with a broad range of stakeholders; input through a formal public comment process and additional forums, including focus groups; PCORI presentations to various audiences; outreach through PCORI’s website and other vehicles; advisory panels; and stakeholder meetings. Additionally, PCORI is examining its initial round of funded contracts (the PCORI Pilot Projects Awards Program) to gauge community interests and needs, as well as to determine and apply lessons learned from those funding opportunities.

Research Agenda Overview

PCORI’s initial approach specifies a set of questions and topics that the multi-stakeholder Board of Governors, after the rigorous process set forth above, believes are most in need of attention – across a range of conditions and treatments. Within any National Priority and associated Research Agenda area, studies may focus on specific diseases, conditions and interventions, or they may be cross-cutting, including broader study populations or examining interventions or issues that apply across multiple diseases and chronic conditions. This approach recognizes a compelling, novel opportunity – that as PCORI begins its funding program, researchers, partnered with stakeholders, are best positioned to present a range of compelling questions that form a truly patient-centered research agenda. PCORI also recognizes that the National Priorities are not mutually exclusive, and believes the overlap can be highly beneficial and efficient. Ultimately, decisions about funding will depend on the quality of applications—with special attention to the likelihood that the research may lead to improvement in outcomes that patients experience and care about, as determined by alignment with PCORI criteria.

The portfolio of research supported by PCORI’s Research Agenda will be balanced based upon the characteristics of study populations such as age, gender, race/ethnicity, socioeconomic status, and disease or conditions. PCORI has a commitment to include studies of patients with rare conditions, as well as those with more common illnesses. Especially needed are studies to improve care and outcomes for patients faced with multiple conditions. All funded studies will have a strong orientation to the patient perspective, which includes an emphasis on evidence that enhances communication at the point of care, including self-care. PCORI-funded studies will have patients involved in the development of the research, its governance and oversight, and its dissemination strategy.

Research Agenda Implementation

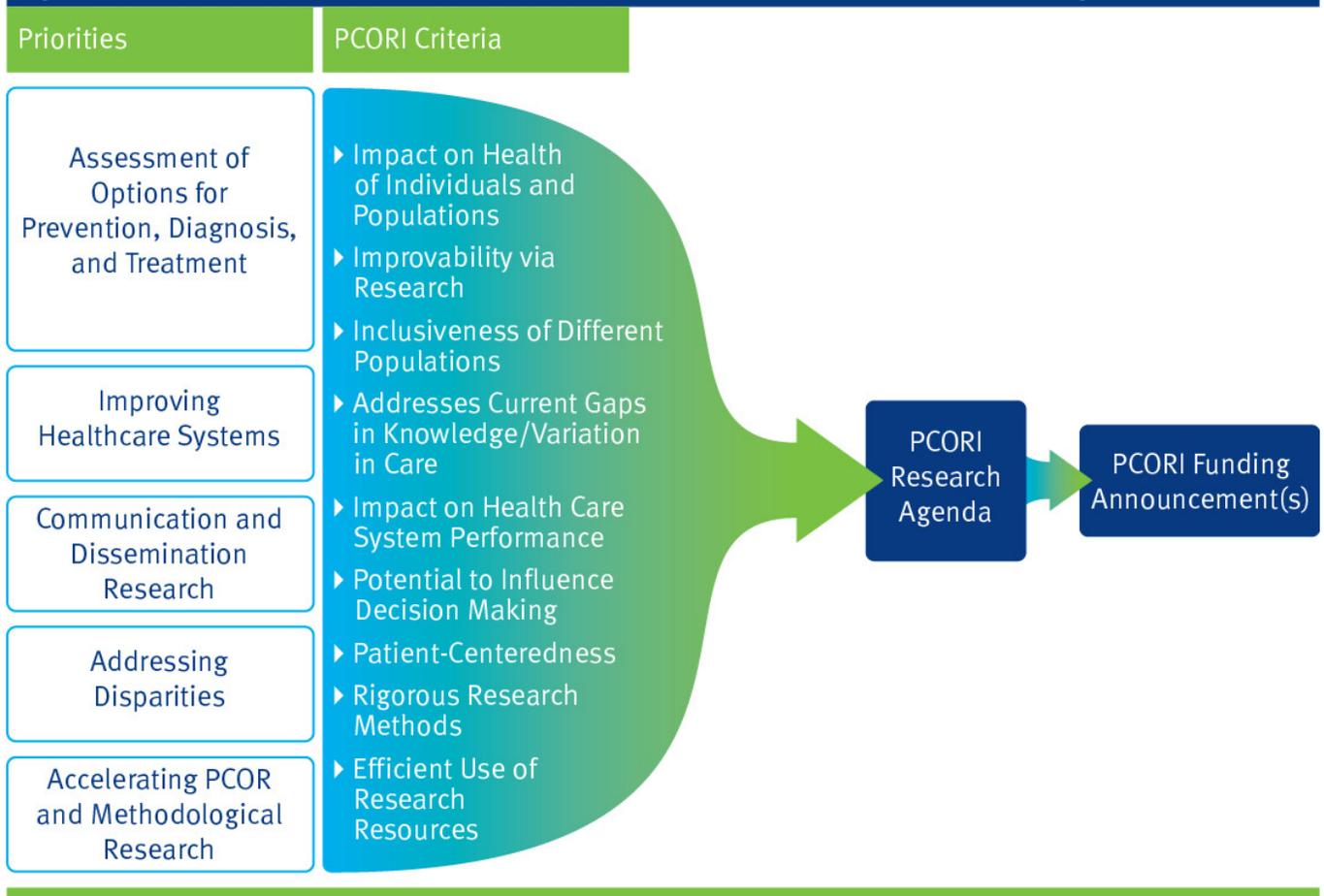
Over time, it is anticipated that PCORI will develop a research portfolio that includes both broad calls for proposals as well as contracts targeted specifically to high-priority questions identified from public input, dialogue with stakeholders, and public needs. Targeted opportunities may focus on specific conditions or diseases, treatment modalities, outcomes, or on themes that are cross-cutting. PCORI will work diligently to avoid redundancy and coordinate with other research entities that fund patient-centered outcomes research or comparative effectiveness research, including the National Institutes of Health, AHRQ, private foundations, and the pharmaceutical, life sciences and healthcare industries. PCORI will seek opportunities for collaboration with these entities.

Similarly, the Research Agenda does not specify preferred study designs or analytic approaches. Instead, it recognizes that various PCOR questions might be investigated by a variety of scientifically valid methods and approaches. In accordance with the Act (Section C: Functions), PCOR is specifically included in the Research Agenda. PCORI's Methodology Committee developed standards, delivered to the Board of Governors as required by statute on May 10, 2012, for the design and conduct of different types of PCOR. At the Board's direction, these standards will be disseminated and eventually used in the evaluation of PCORI funding applications. Again, the Research Agenda does not preclude subsequent funding announcements that would specify a preferred or required methodological design for a specific research question.

Research Agenda Process

The development of the Research Agenda was facilitated by the Research Agenda Workgroup, a subgroup of the Board of Governors Program Development Committee. The Research Agenda Workgroup also solicited and gathered input from the Committee, Board, and Methodology Committee. Following identification of PCORI's five National Priorities, the workgroup developed a framework for the translation of these priorities into the Research Agenda, taking fully into account the relevant statutory language (see Figure 2). The process benefited from public comment about the definition and ongoing dialogue about PCORI and patient-centered outcomes research.

Figure 2: Framework for the Translation of PCORI National Priorities into the Research Agenda



Each of the five National Priorities was considered in light of the set of criteria specified in the Act, shown in the above framework, to create the Research Agenda (see Table 3). The resulting Research Agenda consists of a set of more specific statements of research interest within each of the five priority areas. Each Research Agenda statement maps to one or more of the Criteria. PCORI will develop funding announcements from these agenda statements and emphasize these criteria in those announcements, in the review of applications, and in funding decisions.

Table 3: PCORI Criteria

PCORI Criteria	Statutory Language	Questions
Impact on Health of Individuals and Populations	<i>disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions)</i>	How many people are impacted by this priority area? How severe are the consequences, in terms of mortality, symptoms, adverse effects of treatment, patient experience, and loss of function?
Probability of Improvability via Research	<i>the potential for new evidence to improve patient health, well-being, and the quality of care</i>	How likely is additional information in this priority area to make important improvements in patients' health status, the quality of their care, or the public's health?
Inclusiveness of Different Populations	<i>Research shall be designed, as appropriate, to take into account the potential for differences in the effectiveness of health care treatments, services, and items as used with various subpopulations, such as racial and ethnic minorities, women, age, and groups of individuals with different comorbidities, genetic and molecular sub-types, or quality of life preferences and include members of such subpopulations as subjects in the research as feasible and appropriate.</i>	Would new information in this priority area be particularly likely to increase understanding of differences in best treatments, prevention strategies, or a personalized assessment of an individual's unique biological characteristics and/or social circumstances?
Address Current Gaps in Knowledge/Variation in Care	<i>gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care</i>	Does medical care in this area currently show wide variations in practice or clinical outcomes, suggesting a lack of clear evidence on effectiveness or a lack of awareness about this evidence?
Impact on Healthcare System Performance	<i>the effect on national expenditures associated with a health care treatment, strategy, or health conditions</i>	Will more information in this priority area help [healthcare systems support] improve healthcare treatment or get better health outcomes for the money invested?
Potential to Influence Decision-Making	<i>the relevance to patients and clinicians in making informed health decisions</i>	Will more information in this priority area be particularly likely to help patients and clinicians address decisions that are currently difficult to make?
Patient-Centeredness	<i>patient needs, outcomes, and preferences</i>	Have patients or other key stakeholders explicitly identified a need for more research, or is there a lack of resources in this priority area?
Rigorous Research Methods	<i>The Institute shall make available to the public and disclose ... the process and methods for the conduct of research including ... research protocols, including measures taken, methods of research and analysis, research results and such other information...</i>	Does proposed research or study in this priority area use or develop optimal methodologic and analytic approaches to addressing patient-centered evidence?
Efficient Use of Research Resources	<i>taking into consideration the types of research ... and the relative value (determined based on the cost of conducting research compared to the potential usefulness of the information produced by research)</i>	Will the proposed study use PCORI resources efficiently? Might it create common data or infrastructure that could support future research?

PCORI's Research Agenda in Detail

1. Assessment of Prevention, Diagnosis, and Treatment Options

Patients, caregivers, clinicians, and other healthcare system stakeholders often lack the appropriate evidence on which to make the best choices regarding prevention, screening, diagnosis, monitoring, or treatment. This may be a result of the following issues: strategies with new therapies or technologies have been approved and marketed with inadequate comparison with other approaches; alternative longstanding approaches have not been rigorously and appropriately compared; outcomes important to patients have not been evaluated; existing studies have not assessed benefits and risks of treatment over an extended time period; or previous research has not adequately attended to potential differences in effect among or within different patient groups, or research has not been relevant to the subgroups, settings, and conditions (e.g., comorbidity) to which results are applied. In other situations, many studies have been conducted, but their results have not been considered and synthesized as a cohesive body of evidence or analyzed in a way that allows for comparison.

Clinical effectiveness compares the effectiveness and safety of preventive, diagnostic, and treatment options to create a foundation of information for personalized decision-making. Personalized decisions acknowledge the centrality of patient preference and the need to provide information that is appropriate for each individual. For example, personalized information about what a patient might expect might take advantage of information about a patient's profile as defined by a wide range of characteristics that could affect their outcomes, including but not limited to biology, demography, culture, socioeconomic status, comorbidity, and geography. This research places emphasis on the practical utility of the comparisons, the examination of all outcomes that may be important to patients and the possible differences in outcomes across patient subgroups.

PCORI is interested in the following topics:

- A. Studies that compare situations in which the effectiveness of strategies for prevention, treatment, screening, diagnosis, or surveillance have not been adequately studied against alternative options and better evidence is needed to support decision-making by patients, caregivers, and healthcare professionals. Rare diseases are an area of interest. Special emphasis is placed on studies conducted in typical clinical populations considering the full range of relevant patient-centered outcomes and possibilities that results may differ among patient groups based on patient characteristics (understood broadly as possibly including clinical, psychosocial, demographic, and other domains) or preferences. Topics are not limited to medical or surgical therapy and may include a range of strategies including self-care. PCORI recognizes that a variety of study designs

and approaches may contribute valid new knowledge about the comparative clinical effectiveness of specific strategies. There is a particular interest in comparisons for which new knowledge could address individual differences in patient values and preferences and support shared decision-making. (Criteria addressed: Current Gaps in Knowledge/Variations in Care, Potential to Influence Decision-Making, Inclusiveness of Different Populations, Patient-Centeredness)

- B. Studies that compare the use of prognostication and risk-stratification tools with usual clinical approaches to treatment selection, as well as studies that investigate the key determinants of treatment outcomes, with attention to various patient and environmental factors, including demographic, biological, clinical, social, economic, and geographic factors that may influence the outcomes that patients experience. The purpose of this research should be to inform and improve decisions that patients, their caregivers, and healthcare professionals face and to improve clinical outcomes. (Criteria addressed: Potential to Influence Decision-Making, Inclusiveness of Different Populations, Improved Health System Performance)

2. Improving Healthcare Systems

Healthcare systems at all levels lack evidence on the most effective strategies to support patients in obtaining the outcomes they desire. New system-level strategies are developed and implemented that have not been rigorously evaluated or tested and are not yet ready for full-scale implementation. Comparative studies of healthcare system-level interventions, including disease management, telemonitoring, telemedicine, care management, integrative health practices, care coordination, performance measurement, quality improvement, use of incentives, protocols of treatment, clinical decision and self-management support, and others are lacking.

PCORI is interested in the following topics:

- A. Research that compares alternative system-level approaches to supporting and improving patient access to care, receipt of appropriate care, personalized decision-making and self-care. Research that compares alternative approaches to models of care delivery or coordination of care across healthcare services or settings, including for patients with complex, chronic, and/or multiple conditions. The examination of the comparative effectiveness of changes in communication and outcomes with the implementation of technology such as electronic health records (EHRs) would also be appropriate. The emphasis is on comparing approaches for their effects on patients and, when relevant, their caregivers, in ways that they experience and think are important.

(Criteria addressed: Improve Healthcare System Performance, Inclusiveness of Different Populations, Gaps in Knowledge/Variations in Care, Potential to Influence Decision-Making)

- B. Research that compares the effectiveness on patient outcomes of a wide range of system-level strategies to incorporate alternative, new and/or extended roles for healthcare providers or other health professionals (e.g., pharmacists, nurses, physician assistants, dentists, integrative healthcare providers, complementary and alternative medicine providers, patient navigators, volunteers, etc.) into the healthcare team. Research that compares the effectiveness on patient outcomes of alternative strategies to composition of healthcare teams through alternative workforce deployment models, including care collaboration and team-based care approaches. (Criteria addressed: Improve Healthcare System Performance)
- C. Research that specifically seeks to compare patient outcomes across various populations in response to system-level interventions aimed at improving healthcare and outcomes for patient populations. (Criteria addressed: Improve Healthcare System Performance, Inclusiveness of Different Populations)

3. Communication and Dissemination Research

Knowledge about how to optimally communicate and facilitate the effective use of patient-centered outcomes research evidence by patients, caregivers, and healthcare professionals needs to be strengthened. There is a considerable barrier to the rapid transfer of evidence that could be useful in decision-making. For decisions to be informed, strategies are often needed to make existing PCOR knowledge available to patients and providers, and to make the application of this knowledge feasible outside of as well as within clinical settings. Research is needed that compares new and alternative approaches to facilitating uptake of information by patients, caregivers, communities, and healthcare providers in timely ways, by providing understandable language, and in a variety of settings to improve personalized and shared decision-making.

PCORI is interested in the following topics:

- A. Research that compares alternative communication, dissemination, health literacy, and implementation strategies that aim to improve shared decision-making by empowering people to be more engaged, increasing clinician and patient awareness of healthcare options, and use of comparative effectiveness research results at the point of decision-making. There is an interest in ensuring that research takes into account the health

literacy of individual patients. (Criteria addressed: Potential to Influence Decision-Making, Current Gaps in Knowledge/Variation in Care)

- B. Research that compares the effectiveness, across a range of patient-centered outcomes, of alternative approaches to increase or encourage effective patient participation in care decisions and in shared decision-making. (Criteria addressed: Potential to Influence Decision-Making, Current Gaps in Knowledge/Variation in Care)
- C. Studies to develop and compare alternative methods and tools to include patient-desired outcomes in the healthcare decision-making process. (Criteria addressed: Potential to Influence Decision-Making, Patient-Centeredness, Address Current Gaps in Knowledge/Variations in Care)
- D. Research that compares innovative approaches in the use of existing electronic clinical data and other electronic modalities from the healthcare system or from a network of systems to enhance clinical decision-making by patients and providers. (Criteria addressed: Improve Healthcare System Performance, Potential to Influence Decision-Making)

4. Addressing Disparities

Disparities in health status and health care persist in this country, based on race/ethnicity, gender, geographic location, socioeconomic status, and other factors. These disparities contribute to poor quality of care and poor overall health outcomes for specific populations. Solutions that can reduce persisting disparities have been elusive and are likely to be complex. Novel, patient-centered approaches to understanding and reducing disparities in health and in healthcare quality are needed.

PCORI is interested in the following topics:

- A. Research that compares interventions to reduce or eliminate disparities in health outcomes, for example, by accounting for possible differences in patient preferences or differences in response to therapy across socioeconomic, demographic, and other patient characteristics. (Criteria addressed: Impact on Health of Individuals and Populations, Inclusiveness of Different Populations)
- B. Research that compares benefits and risks of treatment, diagnostic, prevention, or service options across different patient populations, with attention to eliminating

disparities that are not a result of patient preference. (Criteria addressed: Impact on Health of Individuals and Populations, Inclusiveness of Different Populations)

- C. Research that compares strategies to overcome barriers (e.g., language, culture, transportation, homelessness, unemployment, lack of family/caregiver support that may adversely affect patients and is relevant to their choices for preventive, diagnostic, and treatment strategies or their outcomes. (Criteria addressed: Impact on Health of Individuals and Populations, Inclusiveness of Different Populations)
- D. Research that compares and identifies best practices within various patient populations for information sharing about treatment outcomes and patient-centered research. (Criteria addressed: Inclusiveness of Different Patient Populations)

5. Accelerating PCOR and Methodological Research

The nation's capacity to conduct patient-centered comparative effectiveness research quickly and efficiently remains extremely limited. Research that promotes a more comprehensive, complete, longitudinal data infrastructure; broader participation of patients, clinicians, health systems, and payers; and further improvements in analytic methods for both observational and experimental CER are needed. Methodological research to understand optimal approaches for identifying and addressing PCOR questions and better approaches to effectively engaging patients and other stakeholders are necessary. There is also a need to develop methods that are appropriate for rare conditions, as it may be particularly challenging to assemble cohorts and obtain large numbers of individuals. There is interest in producing novel methodologies that assist in areas where current approaches have limitations. Future investments will not only cover the topic areas outlined below, but also will contain a component of expanding the PCOR workforce, building research networks, and accelerating infrastructure.

PCORI is interested in the following topics:

- A. Research that identifies optimal methods for engaging and empowering patients, those at risk, and other stakeholders in PCOR, particularly those who have been historically hard to reach. This also includes research that determines methods for assuring study questions, outcomes, and interventions are meaningful to patients and other stakeholders. (Criteria addressed: Rigorous Research Methods, Impact on Individuals and Populations, Inclusiveness of Different Populations, Potential to Influence Decision-Making)

- B. Research that aims to improve the validity and/or efficiency of analytic methods for comparative effectiveness research or of outcomes commonly used in PCOR. (Criteria addressed: Rigorous Research Methods, Impact on Health of Individuals and Populations, Impact on Healthcare System Performance)
- C. Research that determines the validity and efficiency of data sources commonly used in PCOR. For example, research that seeks to improve the volume, completeness, comprehensiveness, accuracy, and efficiency of use of clinical data collected across healthcare systems, clinical data networks, registries, or payer databases, and the utility of this data for conducting longitudinal studies of patient outcomes; research that explores the potential of large clinical data networks to support PCOR; or research that develops and promotes the utility, performance, and efficiency of large clinical data networks or registries for supporting patient-centered outcomes research for patients with rare diseases. (Criteria addressed: Efficient Use of Research Resources, Impact on Healthcare System Performance, Impact on Health of Individuals and Populations)
- D. Research into methods to enhance the reproducibility, transparency, and replication of PCOR research. (Criteria addressed: Rigorous Research Methods, Current Gaps in Knowledge)
- E. Research that improves and possibly compares strategies for training researchers, patients, and other stakeholders in the methods of patient-centered outcomes research. (Criteria addressed: Inclusiveness of Different Populations, Potential to Influence Decision-Making, Efficient Use of Research Resources)
- F. Research to support the routine collection of key patient-reported and patient-centered outcomes in systematic ways. (Criteria addressed: Rigorous Research Methods, Potential to Influence Decision-Making)

Funding Model

PCORI's initial proposal is for funds to be allocated approximately as identified in the table below, recognizing that there will be some overlap in content between these priority categories. The ultimate distribution of funding may also change in response to the quality of submissions received and over time as the Research Agenda evolves and community needs change.

PCORI's Proposed Research Budget Distribution

Priority	% Funding Allocation
Assessment of Prevention, Diagnosis, and Treatment Options	▶ Approximately 40%
Improving Healthcare Systems	▶ Approximately 20%
Communication and Dissemination Research	▶ Approximately 10%
Addressing Disparities	▶ Approximately 10%
Accelerating PCOR and Methodological Research	▶ Approximately 20%

IV. Appendix

Features of the PCORI Research Agenda

As PCORI seeks to develop its Research Agenda, it has identified several features that will be associated its research and will be emphasized in its activities. PCORI will:

- I. Promote patients and their caregivers—and key stakeholders in implementation settings—as partners, with explicit roles in the design, governance, review, and dissemination of research.
- II. Seek to understand core questions from the expressed perspective of patients and their caregivers.
- III. Emphasize methods and structures that produce knowledge efficiently, seeking to make best use of our resources.
- IV. Focus on outcomes that are important to patients and their caregivers and likely to be useful in making health and healthcare-related decisions.
- V. Emphasize open and transparent science that involves participants in decisions about making data available for further study, seeking to ensure that the research produces as much new investigative activity as possible, and that sharing of information and knowledge among diverse investigators is required.
- VI. Commit to a diverse research portfolio with respect to patients, geography, healthcare professionals, investigators, and organizations, seeking to catalyze activity across a broad range of patients, sites, conditions, and questions.
- VII. Emphasize knowledge that is likely to make a positive difference in the lives of patients and their caregivers and is suitable for dissemination and application; and emphasize outcomes that are important to patients and their caregivers and likely to be useful in their decision-making.
- VIII. Fund efforts that produce practical tools, aids, and skills that will assist patients, their caregivers, and their healthcare professionals.
- IX. Emphasize ideas that emerge from the community of patients, caregivers, clinicians, and researchers, seeking to listen and learn from the wisdom of those whose lives are most affected by these conditions and those who are committed to generating new knowledge that will promote better decisions and outcomes.
- X. Measure eventual success by the impact on patient outcomes.
- XI. Require outstanding science, compelling relevance to decisions, and meaningful results to patients, but encourage a variety of methodological approaches.
- XII. Emphasize rapid cycle, efficient, innovative research and dissemination.