



Patient-Centered Outcomes Research Institute

Draft National Priorities for
Research and Research Agenda
Version 1

Presented for Public Comment

January 23, 2012

Draft National Priorities for Research and Research Agenda

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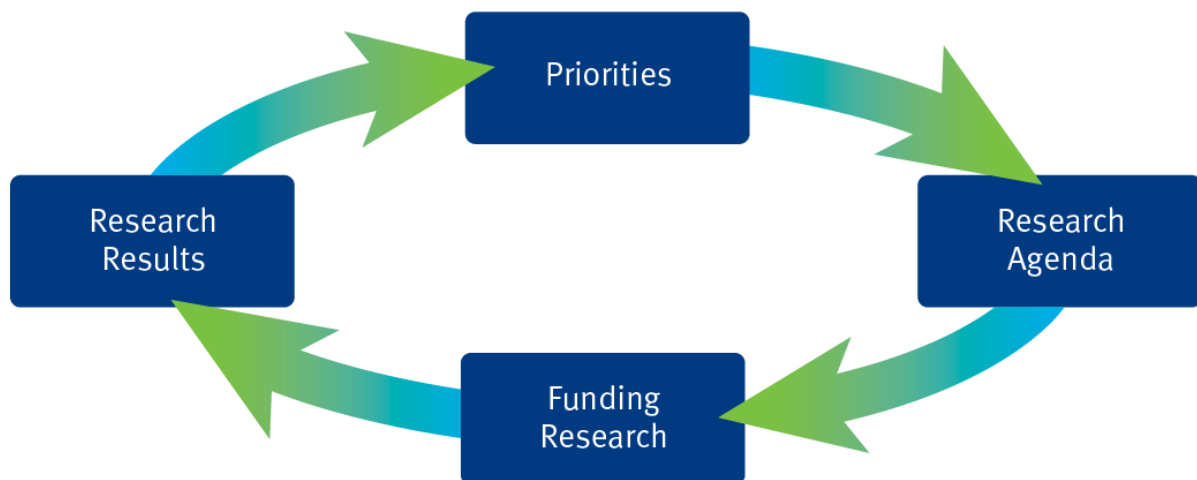
Introduction and Invitation for Public Comment

The **Patient-Centered Outcomes Research Institute (PCORI)** is an independent, non-profit health research organization. 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. These are studies that compare options for preventing disease and providing treatment and care. PCORI does so 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's Path from Priorities to Research Patients Can Use



PCORI invites you to help. PCORI is looking for comments about its first set of research priorities and agenda from patients, caregivers, professionals, and the general public. This document includes information about PCORI and ways you can help.

PCORI's Proposed National Priorities for Research

PCORI has prioritized five research areas. These focus on information that patients and caregivers need in order to make important healthcare decisions. PCORI strives to meet the needs of all patients.

PCORI's proposed national priorities for patient-centered comparative clinical effectiveness research are:

- **Comparative Assessments of Prevention, Diagnosis, and Treatment Options.** The research goal is to determine which option(s) work best for distinct populations with specific health problems.
- **Improving Healthcare Systems.** Focuses on ways to improve healthcare services, such as the coordination of care for patients with multiple chronic conditions.
- **Communication and Dissemination.** Looks at ways to provide information to patients so that they, in turn, can make informed healthcare decisions with clinicians.
- **Addressing Disparities.** Assures that research addresses the healthcare needs of all patient populations. This is needed as treatments may not work equally well for everyone.
- **Accelerating Patient-Centered and Methodological Research.** Includes patients and caregivers in the design of research that is quick, safe, and efficient.

PCORI's Proposed Research Agenda

This is the first version of PCORI's Research Agenda. PCORI expects to learn and update this as we move forward. We are not specifying or prioritizing any particular condition or disease for research, although we may do so in the future. Consistent with the criteria outlined in the Patient Protection and Affordable Care Act (also known as "health care reform"), PCORI's first research agenda looks at:

- **Comparisons of Prevention, Diagnosis, and Treatment Options.** Research should focus on 1) clinical options with emphasis on patient preferences and decision-making, 2) biological, clinical, social, economic, and geographic factors that may affect patient outcomes.
- **Improving Healthcare Systems.** Research should focus on 1) ways to improve access to care, receipt of care, coordination of care, self-care, and decision-making, 2) use of non-physician healthcare providers, such as nurses and physician's assistants, and the impact on patient outcomes, 3) system-level changes affecting all populations, diseases, and health conditions.
- **Communication and Dissemination.** Research should focus on 1) strategies to improve patient and clinician knowledge about prevention, diagnosis and treatment options, 2) methods to increase patient participation in care and decision-making and the impact on health outcomes, 3) communication tools that enhance decision-making and achieve desired outcomes, 4) ways to use electronic data ("e-health records") to support decision-making, 5) best practices for sharing research results.
- **Addressing Disparities.** Research should focus on 1) ways to reduce disparities in health outcomes, 2) benefits and risks of healthcare options across populations, 3) strategies to address healthcare barriers that can affect patient preferences and outcomes.

- **Accelerating Patient-Centered and Methodological Research.** Research should focus on 1) ways to improve the quality and usefulness of clinical data in follow-up studies, 2) methods to combine and analyze clinical data that follow patients over time, 3) use of registries and clinical data networks to support research about patient-centered outcomes, including rare diseases, 4) strategies to train researchers and enable patients and caregivers to participate in patient-centered outcomes research.

Public Comment: How You Can Help

PCORI encourages bold thinking about research projects. This means that PCORI is looking to you—patients, caregivers, professionals, and the general public—for help in shaping its national priorities and research agenda. Here are ways you can help:

- **Provide comment through the online survey.** You can do so from January 23-March 15, 2012, at the PCORI website, <http://www.pcori.org/provide-input>
- **Attend the National Patient and Stakeholder Dialogue.** This will be held February 27, in Washington, D.C. A webcast and teleconference will be provided, if you cannot attend in person. Learn more and register at the PCORI website, <http://www.pcori.org/meetings-events/event/pcori-national-patient-and-stakeholder-dialogue/>
- **Learn more.** You can find more information at the PCORI website, www.pcori.org

Thank you for your interest in PCORI. Together, we offer patients and caregivers the information they need to make important healthcare decisions. Questions about the Proposed National Priorities for Research and Research Agenda may be directed to PCORI by email at info@pcori.org.

Draft 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 (the Act), one of the Patient-Centered Outcomes Research Institute's (PCORI's) first responsibilities is to establish and publish for comment 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 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.”

Over the past five months, workgroups of PCORI's board 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, updated and received input from stakeholder groups through a number of public presentations and other modes of communication.

II. PCORI’s Draft National Priorities for Research

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

A Strong Foundation of Research Prioritization

To develop the initial 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; Federal Coordinating Council for Comparative Effectiveness Research; National Prevention, Health Promotion and Public Health Council; and U.S. Department of Health and Human Services. 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 the nine included significant public input. These seven processes 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 (patient engagement, safety, overuse, information technology (IT) infrastructure, and the impact of new technology) include issues that are systemic in nature to healthcare. 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 and patient engagement in six.

Table 2: Ten CER 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
Institute of Medicine (2009)	✓		✓	✓	✓	✓	✓	✓	✓	✓
Federal Coordinating Council for Comparative Effectiveness Research	✓	✓				✓	✓		✓	
U.S. Department of Health and Human Services	✓		✓			✓	✓			
Agency for Healthcare Research and Quality	✓	✓	✓	✓	✓			✓		
National Quality Forum	✓	✓	✓	✓	✓	✓	✓		✓	
National Prevention, Health Promotion and Public Health Council	✓					✓				
National Priorities Partnership	✓		✓		✓	✓	✓	✓		

PCORI's Proposed National Priorities for Research

PCORI analyzed the 10 priority areas from previous prioritization processes 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 working definition of PCOR is provided on the PCORI website: www.pcori.org/patient-centered-outcomes-research/.) 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.

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

1. **Comparative Assessment of Options for Prevention, Diagnosis, and Treatment** - 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 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 first proposed national priorities for research and encompass the patient-centered comparative clinical effectiveness research PCORI will support. As PCORI's work progresses, and particularly as PCORI continues to engage, in a transparent manner, with the broad range of stakeholders in healthcare, and particularly with patients, it is possible that PCORI may develop additional national priorities for research as needs evolve.

III. PCORI's Draft Research Agenda

This draft Research Agenda, version 1, 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 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 that PCORI publish an agenda to describe and guide the research it will fund. By design, it 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 the Agency for Healthcare Research and Quality (AHRQ) and the U.S. Department of Health and Human Services (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. This initial Research Agenda, when finalized after public comment, presents 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 grants (the PCORI Pilot Projects Grants Program) to gauge community interests and needs and to determine and apply lessons learned from those funding opportunities.

Level of Specificity of the Research Agenda

By intent, this draft Research Agenda does not specify a restricted set of conditions or treatments that PCORI will study. Although some previous prioritization efforts have focused on specific high-prevalence or high-cost conditions, PCORI’s priorities and agenda do not place such limits on the scope of research that will be supported. The criteria specified in the legislation, when considered together, do not point strongly to such conditions, but suggest that a more diverse research portfolio that considers a range of conditions, interventions, and research methods, may be more appropriate.

Rather than focusing on specific conditions or treatments, PCORI’s initial approach specifies a set of questions and topics that we believe are most in need of attention – across a range of conditions and treatments. Within any topical area, studies may focus on specific diseases, conditions and interventions, or they may be cross-cutting, including broader study populations or examining interventions or other questions that apply across multiple diseases and conditions. This approach recognizes the likelihood that as PCORI begins its funding program, researchers partnered with stakeholders are well-positioned to present a range of compelling questions. 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 patient outcomes, as determined by alignment with PCORI criteria.

Over time, we anticipate that PCORI will develop a research portfolio that includes both broad calls for proposals as well as contracts or grants targeted to high-priority conditions or treatments 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 (PCOR) or comparative effectiveness research (CER), including the National Institutes of Health (NIH), AHRQ, private foundations and the pharmaceutical, life sciences and healthcare industries. PCORI will seek opportunities for collaboration with these entities. Additionally, the portfolio of research supported by PCORI will be balanced based upon the characteristics of study populations such as age, gender, race/ethnicity, socio-economic status, and disease or conditions. PCORI has a commitment to include studies of patients with rare conditions as well

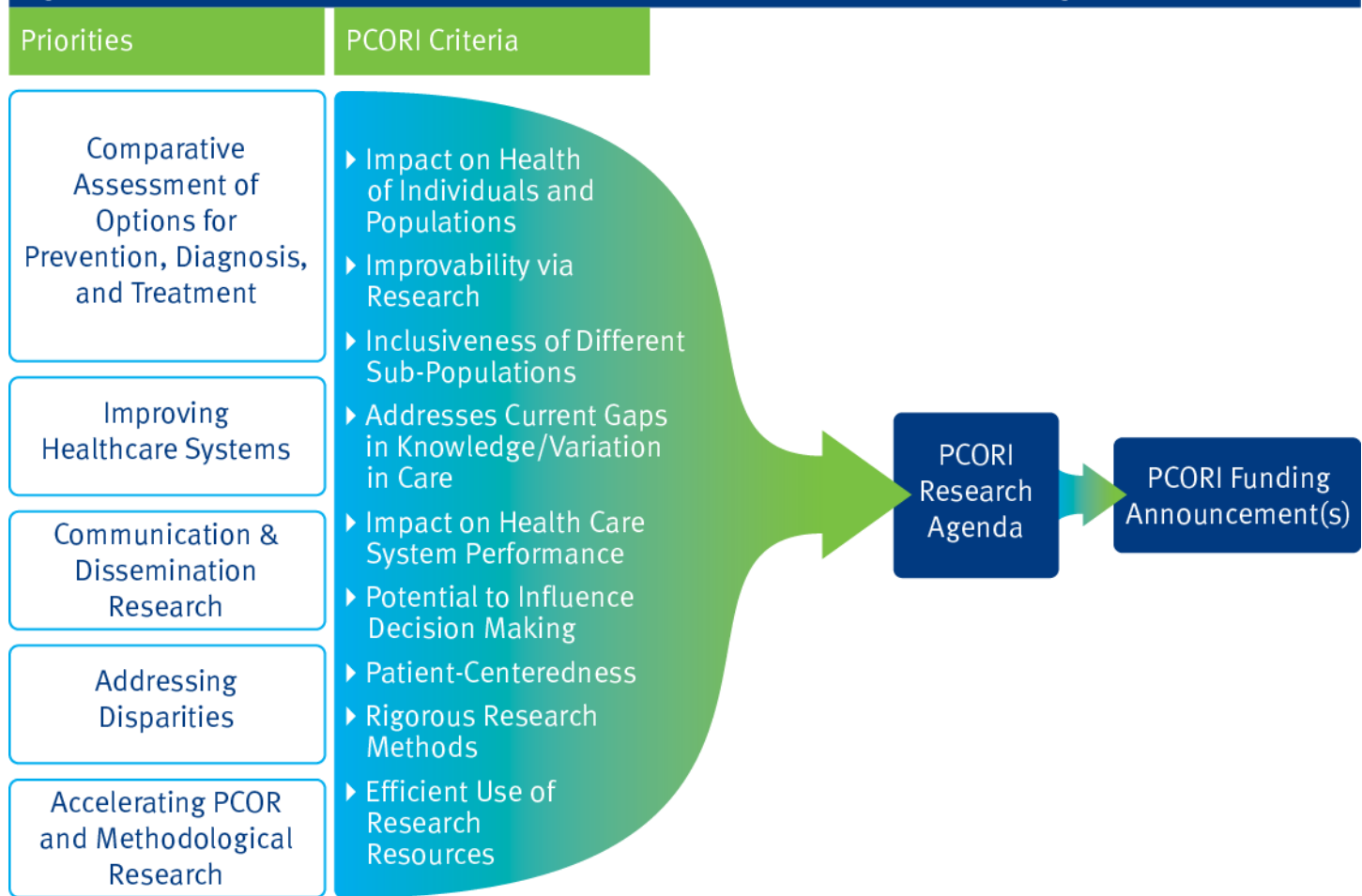
as those with more common illnesses. Especially needed are studies to improve care and outcomes for patients faced with multiple conditions. All funded studies will have a strong orientation to the patient perspective and all will have patients involved in the development of the research, its governance and oversight, and its dissemination strategy.

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 Act (Section “(C) FUNCTIONS”), research focused on developing new methods and/or improving the science and methods of patient-centered outcomes research (PCOR) is specifically included in the Agenda. PCORI’s Methodology Committee is currently developing standards for the design and conduct of different types of PCOR. These standards will be disseminated and used in the evaluation of PCORI applications. Again, the 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 PCORI Board of Governors’ Program Development Committee. The Research Agenda Workgroup also solicited and gathered input from the committee, Board of Governors, and PCORI’s Methodology Committee. Following identification of the five PCORI National Priorities, the workgroup developed a framework for the translation of these Priorities into the Research Agenda, taking fully into account the statutory language in the Act regarding both the National Priorities and the Research Agenda. (See Figure 1). The process benefited from public comment about the definition and on-going dialogue about PCORI and patient-centered outcomes research.

Figure 1: Framework for 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.

The Act specified a set of criteria that must be considered in creating a research agenda. The Agenda is based on these criteria. PCORI will emphasize these criteria in funding announcements, in the review of applications, and in funding decisions. These criteria are described in the table below.

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?
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 Health 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 [health care systems support] improve health care 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 Proposed Research Agenda

1. Comparative Assessment of Options for Prevention, Diagnosis, and Treatment

Patients, caregivers and clinicians often lack the appropriate evidence on which to make the best choices regarding prevention, screening, diagnosis, monitoring, or treatment. This may be because strategies with new therapies or technologies have been approved and marketed with inadequate comparison with other approaches; because alternative longstanding approaches have not been rigorously and appropriately compared; because outcomes important to patients have not been evaluated; because existing studies have not assessed benefits and risks of treatment over an extended time period; or because 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. 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. 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. 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/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 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, and 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, coordination of care across healthcare services or settings for patients with complex chronic conditions, and personal decision-making and self-care. The examination of the comparative effectiveness of changes in communication and documentation with the implementation of electronic health records (EHRs) would also be appropriate. 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. (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 new and extended roles for allied health professionals (e.g., pharmacists, nurses, physician assistants, dentists, patient

navigators, volunteers, etc.) into the healthcare team. (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 PCOR 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 in 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, and implementation strategies that aim to improve shared decision-making by increasing clinician and/or patient awareness of healthcare options and use of comparative effectiveness research results at the point of decision-making. (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 healthcare persist in this country, based on race/ethnicity, gender, geographic location, socio-economic 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 socio-economic, 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, etc.) 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 CER 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. Future investments will not only cover the topic areas outlined below, but will also 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 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 will seek to fulfill the Research Agenda through a combination of grants and targeted contracts and will remain flexible and responsive to emerging challenges and community-generated questions that fall within priority areas and meet our selection criteria. PCORI’s initial proposal is for funds to be allocated as identified below, recognizing that there will be overlap between categories. PCORI will reassess the distribution in response to the quality of submissions. These allocations are guideposts rather than firm funding levels and the allocations will evolve in time with the Research Agenda and with community needs.

Priority	% Funding Allocation
Comparative Assessment of Options for Prevention, Diagnosis, and Treatment	▶ 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, we have identified several features that will be associated with PCORI research and will be emphasized in our 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 the patient 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 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.