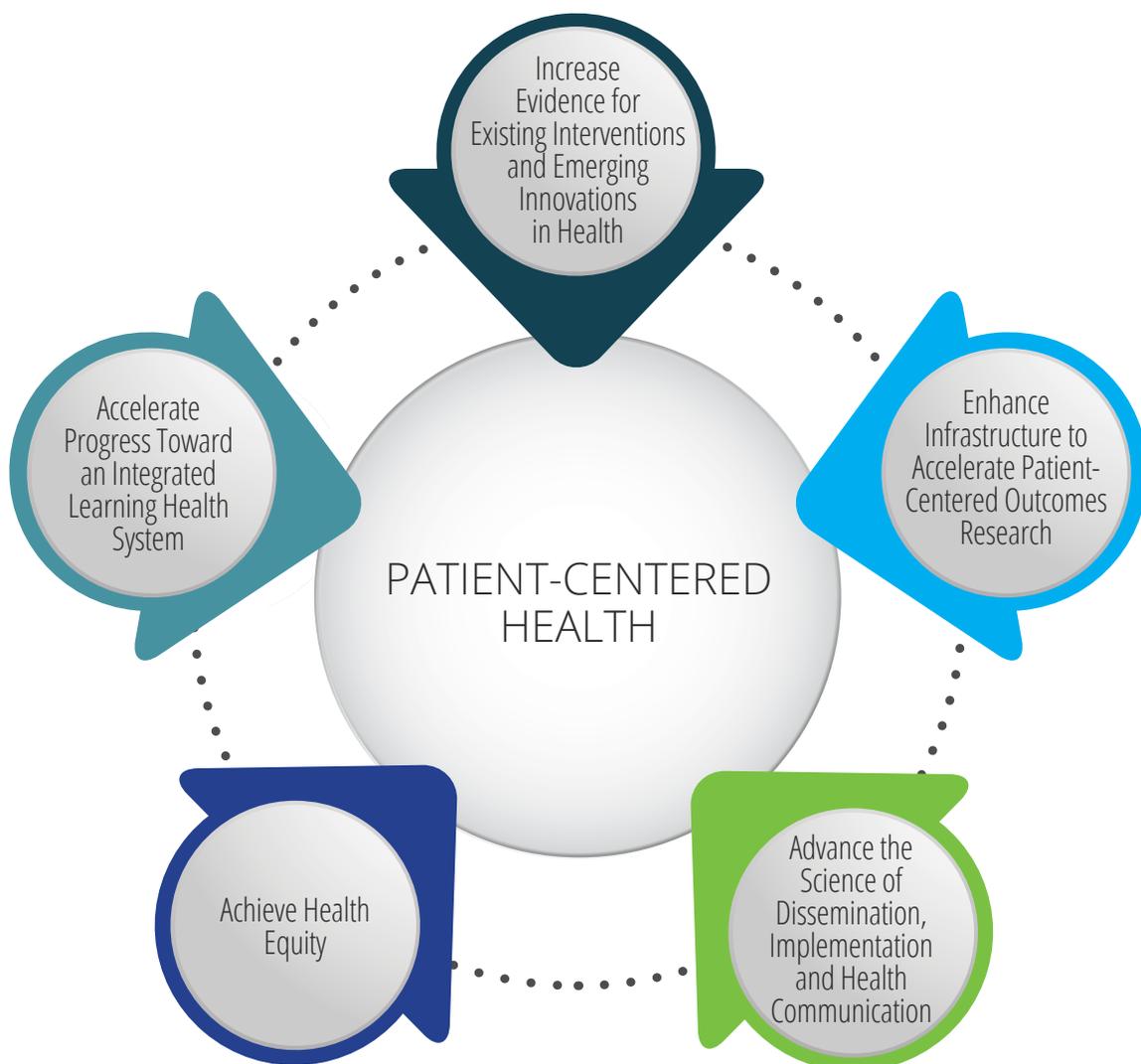pcori

PROPOSED NATIONAL PRIORITIES FOR HEALTH

Introduction

The Patient-Centered Outcomes Research Institute (PCORI) occupies a unique place in the healthcare and health research ecosystem. PCORI funds patient-centered comparative clinical effectiveness research (CER) to assist patients, clinicians, purchasers, and policymakers in making informed health decisions. The goal is for patients, caregivers, and the public to have practical, reliable, and trustworthy information needed to make informed decisions that reflect their desired health outcomes. Being patient and stakeholder guided, PCORI keeps its research relevant, its stakeholders engaged, and its communications both proactive and responsive.

PROPOSED NATIONAL PRIORITIES

Increase Evidence for Existing Interventions and Emerging Innovations in Health

Accelerate Progress Toward an Integrated Learning Health System

Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

PATIENT-CENTERED HEALTH

Achieve Health Equity

Advance the Science of Dissemination, Implementation and Health Communication

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PCORI's congressional authorization requires identification of National Priorities and establishment of a Research Agenda outlining how it will address each of the National Priorities. In 2012, the first National Priorities for Research and Research Agenda were developed by PCORI's Board of Governors, with input from the members of PCORI's Methodology Committee and informed by substantial public comment. With a 10-year reauthorization in 2019, PCORI is undertaking a comprehensive strategic planning process. Building on the foundation of its first decade of work, PCORI is identifying its National Priorities and updating its Research Agenda to guide its future work.
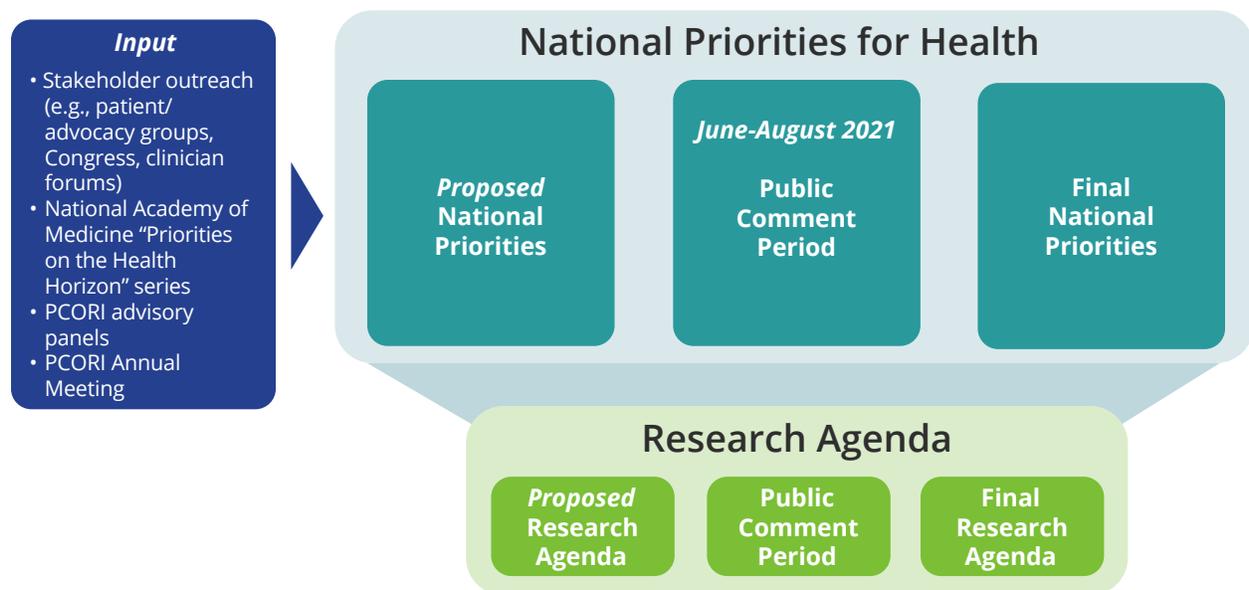
PCORI has framed the proposed National Priorities as long-term goals for health with progress to be achieved through PCORI's Research Agenda (e.g., funding of CER) and other strategies (e.g., stakeholder engagement, dissemination and implementation, and health communication). The proposed National Priorities for Health are broad, ambitious goals that include CER focused on outcomes important to patients. They reflect stakeholder guidance and encompass PCORI's congressional mandate.

The proposed National Priorities for Health are presented by the PCORI Board of Governors in this document for public comment. They have been developed in consultation with numerous stakeholder groups, advisors, and experts and build upon PCORI's experience in its first decade. They are designed to be mutually reinforcing with complementary elements to create synergistic opportunities for progress.

The feedback and input gathered during this public comment period will be considered in finalizing the National Priorities for Health which serve as the foundation for updating PCORI's Research Agenda, which will also be presented for public comment.

STAKEHOLDER AND PUBLIC INPUT INFORMS NATIONAL PRIORITIES AND RESEARCH AGENDA

Input
- Stakeholder outreach (e.g., patient/ advocacy groups, Congress, clinician forums)
- National Academy of Medicine "Priorities on the Health Horizon" series
- PCORI advisory panels
- PCORI Annual Meeting

National Priorities for Health

Proposed **National Priorities**

June-August 2021

Public Comment Period

Final National Priorities

Research Agenda

Proposed **Research Agenda**

Public Comment Period

Final Research Agenda

Proposed National Priorities for Health

Increase Evidence for Existing Interventions and Emerging Innovations in Health

Goal: Strengthen and expand ongoing comparative clinical effectiveness research focused on both existing interventions and emerging innovations to improve healthcare practice, health outcomes, and health equity.

Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

Goal: Enhance the infrastructure that facilitates patient-centered outcomes research to drive lasting improvements in health and transformation of both the research enterprise and care delivery.

Advance the Science of Dissemination, Implementation, and Health Communication

Goal: Advance the scientific evidence for and the practice of dissemination, implementation, and health communication to accelerate the movement of comparative clinical effectiveness research results into practice.

Achieve Health Equity

Goal: Expand stakeholder engagement, research, and dissemination approaches that lead to continued progress toward achieving health equity in the United States.

Accelerate Progress Toward an Integrated Learning Health System

Goal: Foster actionable, timely, place-based, and transformative improvements in patient-centered experiences, care provision, and ultimately improved health outcomes through collaborative, multisectoral research to support a health system that serves the needs and preferences of individuals.

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Background on Proposed National Priorities for Health

Increase Evidence for Existing Interventions and Emerging Innovations in Health

In Brief

PCORI aims to fund comparative effectiveness research on both new and existing healthcare approaches. This type of research compares two or more ways to prevent, diagnose, or treat a health condition. It can also compare ways to deliver health care. This research will help to close gaps in what is known, so that people have better information when making health decisions. Filling these gaps for both current and emerging approaches will improve health care, health outcomes, and health equity.

PCORI aims to strengthen and expand ongoing CER focused on both existing interventions and emerging innovations to improve healthcare practice, health outcomes, and health equity. Including both emerging innovations and existing interventions to close evidence gaps will expand the impact of PCORI's evidence generation, dissemination, and implementation efforts.

Rationale: Innovations in health and health care include new or improved health practices, policies, systems, products, technologies, services, and delivery methods that have potential to transform care and improve health through increased effectiveness, efficiency, quality, safety, and/or affordability. PCORI defines emerging innovations as interventions with some evidence of efficacy but not having yet achieved substantive translation into care; they are often characterized by significant uncertainty in their evidence or appropriate use due to incomplete examination of population(s), conditions, care settings, outcome measures, or comparison with existing intervention or approaches to care.

Evaluating a range of innovations—from clinical care interventions to systems changes and innovations in healthcare delivery, public health, and social determinants of health—has the potential to inform health-related decision making and improve healthcare delivery and patient outcomes, including burdens and economic impacts. Increasing evidence generation to include innovations in health will expand the scope of the stakeholders engaged in PCORI's work. In this National Priority, PCORI reemphasizes the need for research on existing interventions and innovations to include populations who are underserved, underrepresented, and disadvantaged. Innovation can revolutionize health care in positive ways; it can also result in unintended consequences (e.g., adverse events, barriers

to care, burdens and economic impacts, delayed uptake, and widened disparities in outcomes). As innovations emerge, evidence of their potential benefits and harms is typically incomplete and uncertain, as is their comparative effectiveness relative to the landscape of existing interventions. Once identified, promising innovations need to be assessed for their potential to improve outcomes and evaluated to determine their comparative effectiveness relative to   existing interventions and the evidence used to encourage, discourage, or selectively target further adoption into practice.

Strategies to address this priority include:

1. Monitor the research landscape for potentially high-impact innovations

2. Evaluate existing and emerging innovations in clinical care interventions, systems changes, healthcare delivery, technologies, public health, and social determinants of health

3. Study unintended consequences, adverse events, barriers to care, burdens and economic impacts, and widened disparities in care outcomes associated with existing and emerging innovations

4. Expand the scope of stakeholders engaged in PCORI's work from topic inception through implementation of the results

5. Emphasize inclusion of populations who are underserved, underrepresented, and disadvantaged in CER research endeavors

6. Support CER of evidence gaps in diverse populations, geographic areas, and settings to foster equitable uptake practices

Examples of Illustrative Areas and Activities that Could be Explored under This Priority

- Engage stakeholders to determine key decisional dilemmas and barriers to adoption of existing interventions and emerging innovations and enable targeted research that will inform decision making.

- Expand prioritization of CER to include clinical innovations compared to existing interventions using broad and targeted research funding of new preventive, diagnostic, prognostic, and therapeutic interventions (e.g., new imaging tests, genomic and proteomic testing).

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Enhance Infrastructure to Accelerate Patient-Centered Outcomes Research

In Brief

PCORI aims to improve the nation's capacity for health research. Goals include expanding:

- The use of real-world data, such as health records or public health data, for research
- The number and diversity of people and communities who lead or partner in research
- Policies and practices that help people work together and learn from each other

A strong infrastructure like this can improve the research process and lead to better study results. Doing so can improve the healthcare system and people's health.

PCORI aims to enhance the infrastructure that facilitates patient-centered outcomes research (PCOR) to drive lasting improvements in health and transformation of both the research enterprise and care delivery. PCORI is uniquely positioned to strengthen and connect the elements of the health research enterprise that need to work in concert for improved health outcomes to be realized.

Rationale: Health research requires a robust infrastructure that connects effectively with healthcare systems and responds to patient needs to improve health. This infrastructure should evolve to incorporate advances in technology and science, as well as public and community priorities.

Research infrastructure has multiple components. It involves people—patients, communities, clinicians, researchers, purchasers, policy makers, and representatives of payers, industry, hospitals and health systems, and training institutions—who collectively represent the health and research workforce as well as information

(knowledge and data) and methods. It also involves policies and processes to promote connectedness across the research endeavors and how research findings are used—national, state, and local health policy, laws, regulations, and agreements that govern privacy, security, data, research, and human subjects and institutions.

When these elements operate in a proactive, coordinated fashion, they can produce tremendous benefits. However, when the health research infrastructure does not connect effectively with the healthcare system, there are missed opportunities to address important issues (e.g., clinician burnout, health disparities, and poorly coordinated care).

Strategies to address this priority include:

1. Develop and expand the universe of engaged patients and communities and their representative leadership, research workforce, and clinician partners

2. Advance PCOR methods and the accessibility and utilization of real-world data

3. Build synergies and leverage current work within health systems and by stakeholders

4. Integrate patient-centered outcomes research findings into learning health systems

Examples of Illustrative Areas and Activities that Could be Explored under This Priority

- Build a strong and sustainable PCOR workforce pipeline that represents the diverse backgrounds of individuals in the health research ecosystem. This includes patients, communities, clinicians, researchers, purchasers, payers, industry, hospitals and health systems, policy makers, and training institutions that together represent the research workforce.

- Enhance technology to optimize utilization of real-world data and promote accessibility, quality, and standardization in research, while maintaining strict data security and privacy.

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Advance the Science of Dissemination, Implementation, and Health Communication

In Brief

PCORI aims to advance the science of, and practices that support, the real-world use of research results. In this way, research can better inform health decisions and improve health care and health. These efforts will look at how to best communicate about and share study results with diverse audiences. They will also look at ways to get results to patients, clinicians, and others to use in their health decisions.

PCORI aims to advance the scientific evidence for and the practice of dissemination, implementation, and health communication to accelerate the movement of CER results into practice. This National Priority focuses on the science of dissemination, implementation, and health communication. Through this priority, PCORI will support findings generated through its funded research to ultimately be used to improve health care, better inform health decisions, and achieve improved health outcomes.

PCORI will leverage, expand, and advance the evidence base for and the practice of dissemination, implementation, and health communication related to PCOR. This evidence base directly underlies and furthers PCORI's mandated efforts in translation of findings; their dissemination to diverse audiences of patients, families, clinicians, and other healthcare decision makers; and the promotion of sustainable and scalable changes in healthcare delivery to accomplish improvements in health.

Rationale: This National Priority aims to address challenges to the communication and uptake of evidence-based practice. For example, research is needed to understand best ways to balance the tension between fidelity to original research and the need to adapt interventions to diverse local settings, so that improvements in health care will address disparate health needs.

This National Priority will center on supporting scientific inquiry and include studying approaches to communicate information effectively and meaningfully about the benefits and harms of interventions to populations confronting new stressors, competing versions of information, and questions about the trustworthiness of information. This National Priority will address both well-known and emerging concerns and impediments to dissemination and implementation.

Strategies to address this priority include:

1. Fund CER studies of delivery or implementation strategies

2. Communicate research findings effectively and in ways tailored to diverse audiences

3. Actively deliver information to targeted audiences to use to inform healthcare discussions and decisions

4. Promote the uptake of research findings into practice to contribute to improved health care and health.

Examples of Illustrative Areas and Activities that Could be Explored under This Priority

- Build the evidence base by developing evidence on implementation strategies that work. Studies investigating the effectiveness of implementation strategies can be undertaken as part of research on the comparative effectiveness of an intervention or independent of such research.

- Speed the uptake of evidence by preparing for implementation in concert with CER. This could include incorporating elements to prepare for implementation early in the research process.

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Achieve Health Equity

> **In Brief**
>
> PCORI aims to advance health equity in the United States. Health equity exists when all people have a fair and just opportunity to be healthy. Yet not all people and communities have such opportunities. Harmful societal policies and practices over generations have led to continued poor health. This affects people of color, people with low income, and people with disabilities more than others. Advancing health equity requires partnering with these communities. It also involves building on and creating new ways to do, share, and use research to improve health.

PCORI aims to expand stakeholder engagement, research, and dissemination approaches that lead to continued progress toward achieving health equity in the United States. The need for renewed and ongoing focus on this priority is evident from the persistently poor health outcomes disproportionately affecting people with low income, people with disabilities, and people of color   and entrenched negative influences on health in some communities that compound over generations. Leveraging existing research and implementation approaches and developing new ones will help to achieve this goal.

Rationale: Health equity exists when everyone has a fair and just opportunity to be as healthy as possible. Health equity concerns have been a focus of health departments, research organizations, research funders, hospitals, philanthropies, and community-based organizations and a longstanding goal of the public health community. The COVID-19 pandemic has only highlighted and magnified existing health disparities and inequities. The US Department of Health and Human Services, World Health Organization, and others have articulated the critical components of a strategy to achieve health equity. These include combatting racism and discrimination; addressing social determinants of health such as housing, income, education, etc.; providing high-quality care for all patients, regardless of background, at all points of contact with the healthcare system; and leveraging and maximizing opportunities for health outside of traditional health systems, such as social service organizations, public health programs, and school systems. This National Priority will bring a systems-level emphasis to this set of concerns and broaden the scope of how CER is conducted to address the critical issues challenging the advancement of health equity.

Strategies to address this priority include:

1. Fund CER to improve health outcomes for individuals of all backgrounds

2. Strengthen efforts to support inclusive and diverse stakeholder engagement

3. Disseminate and implement research findings with the intention of informing broader health equity strategies

4. Collaborate with health, research, advocacy, social service, educational, and other organizations to reduce health inequities

5. Identify and fund novel ways to support the professional development and increase the engagement of investigators of color , investigators with disabilities, and populations who are historically underrepresented in research endeavors.

Examples of Illustrative Areas and Activities that Could be Explored under This Priority

- Solicit and fund studies across the health research spectrum (including comparing interventions focused on addressing racism, discrimination, and social determinants of health; evaluating health policies/programs; identifying approaches to improve healthcare access; and promoting disease prevention) while ensuring that the improvement of health outcomes remains central.

- Strengthen cross-cutting methodology standards to support representation of populations disproportionately affected by disparities in the development of the research question and throughout the study.

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Accelerate Progress Toward an Integrated Learning Health System

In Brief

PCORI aims to support a learning health system, or LHS, that works to improve patients' experience with health care and their health. Systems that affect people's health—including health care, public health, and social services—often work independently. An integrated LHS can help these systems work together. To do so, it centers on what matters most to people and communities. A successful LHS depends on a feedback cycle to improve health care and health. This cycle engages communities, makes changes, learns from those changes, and repeats.

PCORI aims to foster actionable, timely, place-based, and transformative improvements in patient-centered experiences, care provision, and ultimately improved health outcomes through collaborative, multisectoral research to support a health system that serves the needs and preferences of individuals. This will involve engaging, listening to, and partnering with multiple sectors, including the health care, public health, social, and environmental spheres, to develop interventions that generate evidence tailored to specific settings, populations, and needs. Efforts will include the development and implementation of evidence through a continuous cycle of engagement, knowledge generation, dissemination, and facilitation of implementation efforts.

Rationale: This National Priority acknowledges that health is affected by factors external to the care delivery environment such as equity, equality, and vulnerability. An integrated learning health system can foster improvement in health outcomes at the individual, organizational, and community levels. It can integrate the currently fragmented systems of health, health care, health research, and public health and enable a deeper understanding of the many factors that influence health and well-being.

A sustainable, flexible learning health system driven by actionable and transformative PCOR can catalyze novel multisector strategies to address the health and wellness needs centered on the individual and the communities in which they live and work. These goals can be met through purposeful collaboration, engagement, and partnerships to develop, test, and adapt strategies that engage diverse populations in an integrated health system that is equitable and patient centered. Rather than retrofitting the health system to these goals, transformation to a person- and patient-centered system requires different approaches.

Establishing connections across health, healthcare delivery, and social and environmental systems supported by a broad array of data is essential to an integrated learning health system focused on improved health outcomes. Often, health research funded to conduct a specific type of study aligned with the priorities of the funding entity may not meet the practical needs of the real-

world health system; and a health system that does not evolve based on contextually generated evidence through PCOR will struggle to provide individuals, families, and communities effective options to achieve the outcomes they desire.

The type of collaboration required is driven by shared goals and leverages all available tools: advancing innovative research designs, formalizing and sustaining partnerships, leveraging data across sectors, informing payment and reimbursement mechanisms, and defining and delivering what is valuable to individuals and other health and healthcare stakeholders with the ultimate goal of improved health and outcomes.

Strategies to address this priority include:

1. Fund multisector interventional CER focused on health outcomes and grounded in the context of specific settings, communities, and needs

2. Implement research on precision and personalized medicine and whole-person health into practice

3. Incorporate the full range of outcomes to influence value that encompasses diverse outcomes and perspectives among patients, families, caregivers, and providers

4. Formalize partnerships to ensure an integrated learning health system that meets the needs of patients and caregivers

5. Use data analytic and informatic tools to inform and enable real-time decision making.

Examples of Illustrative Areas and Activities that Could be Explored under This Priority

- Compare cross-sectoral strategies to integrate the formal healthcare system with public health and community-based systems to address clinical and social needs of individuals to reduce the fragmentation and barriers to better health.

- Evaluate care delivery strategies and payment mechanisms that have a direct impact on improved patient-centered health outcomes.

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