Diagnosing Which Health Treatments Improve Outcomes: A PCORI Overview and How to Get Involved

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Which Health Treatments Improve Outcomes? A PCORI Overview

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Why PCORI?

- Research has not answered many questions patients face
- People want to know which treatment is right for them
- Patients need information they can understand and use
“The purpose of the Institute is 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, and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments...”
Our Mission

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

Comparative Clinical Effectiveness Research

- Patient-centered
- Answering questions that matter to patients and other clinical decision makers
- Comparisons of outcomes that matter to patients
Our Work Answers Patients’ Questions

Given my personal characteristics, conditions and preferences...

“What should I expect will happen to me?”

“What are my options and what are the potential benefits and harms of those options?”

“What can I do to improve the outcomes that are most important to me?”

“How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”
Comparative Clinical Effectiveness Research (CER)

PCORI’s Definition

- Compares two or more health and healthcare choices faced by patients, caregivers, clinicians, and other healthcare stakeholders
- Seeks to answer a medical research question for which there is not sufficient evidence or there is contradicting information
- Is likely to provide evidence that can reduce uncertainty, support decision making, change practice, and improve patients’ health outcomes
- Studies an issue or condition that is highly prevalent or places a heavy burden on patients, their families and the healthcare system
The Simplest Explanation

Treatment A

Treatment B
The Simplest Explanation

Treatment A
- Preferences
- Characteristics
- Risks
- Side Effects

Treatment B
- Preferences
- Characteristics
- Risks
- Side Effects

Preferences
Characteristics
Risks
Side Effects

Patient-Centered Outcomes Research Institute
Getting to the Comparative Question

**Health Question**

Am I taking the right blood pressure medication?

What are the side effects and benefits of different blood pressure medications?

**CER Question**

My elderly mother has dementia. Should I place her in a nursing home?

What are comparative benefits and risks of nursing home, assisted living and home-based care for elderly patients with dementia?
We Use Engagement as a Path to Rigorous Research
Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication & Dissemination Research

Addressing Disparities

Accelerating PCOR and Methodological Research
How We Pick Research Questions to Study

1. Gather Suggestions
   - Patient Stakeholder Community

2. Evaluate Suggestions
   - PCORI Staff

3. Prepare Topic Briefs
   - AHRQ and Academic Centers

4. Prioritize Topics
   - Advisory Panels

5. Select Suggestions
   - PCORI Board

6. Release Funding Announcements
   - PCORI Staff

PCORI: Patient-Centered Outcomes Research Institute
Our Advisory Panels

- Leverage knowledge and experience of clinicians, researchers, patients, and other experts to help PCORI achieve its goals

- Assure meaningful patient engagement in:
  - Identifying research priorities and topics
  - Monitoring research activities
  - Conducting randomized clinical trials
How We Review Research Proposals

Who: Each proposal is reviewed by two scientists, a patient, and another stakeholder.

What: Reviewers critique and score applications, making recommendations that our Board of Governors and staff consider carefully in deciding which applications to approve.

How: We provide training so all participants are prepared and understand PCORI’s review criteria.

When: You can apply at any time to serve as reviewer.

Learn more
pcori.org/reviewers

Contact us
reviewers@pcori.org
Our Review Criteria

Priorities 1-4:

- Impact of the condition on health of individuals and populations
- Potential for improving care and outcomes
- Technical merit
- Patient-centeredness
- Patient and stakeholder engagement

Priority 5:

- Impact on field of patient-centered outcomes research
- Potential for the study to improve PCOR methods
- Technical merit
- Patient-centeredness
- Patient and stakeholder engagement
Questions Outside PCORI’s Scope of Work

**Cost Effectiveness:** PCORI will consider the measurement of factors that may differentially affect patients’ adherence to the alternatives such as out-of-pocket costs, but cannot fund studies related to cost effectiveness, costs of treatments or interventions.

**Medical Billing:** PCORI cannot fund studies about an individual’s insurance coverage or about coverage decisions from third party payers.

**Disease Processes and Causes:** PCORI cannot funding studies that pertain to risk factors, origin and mechanisms of diseases.
Coverage Recommendations: PCORI cannot issue guidance regarding which products or services should be covered under any insurance.

Practice Guidelines: PCORI cannot issue recommendations regarding healthcare practice.

Payment and Policy: PCORI cannot issue recommendations on changes to payment or policy regarding healthcare products or services.
Our Methodology Standards

Research we fund must adhere to the highest methodological standards.

- Formulating Research Questions
- Patient-Centeredness
- Systematic Reviews
- General and Crosscutting Methods For All PCOR
- Causal Inference Standards
- Heterogeneity of Treatment Effect (HTE)
- Preventing and Handling Missing Data
- Data Networks
- Adaptive and Bayesian Trial Designs
- Data Registries
- Studies of Diagnostic Tests
How We Promote Participation in Research

- **Pilot Projects**: address a broad range of questions about methods for engaging patients in various aspects of the research and dissemination process.

- **Matching Challenge**: two challenge winners created innovative ways to connect patients and researchers as partners in research.

- **Engagement Awards**: Pipeline to Proposal Awards will build a national community of patients and stakeholders who have the expertise and passion to participate in PCORI research, and create partnerships within that community that lead to high-quality research proposals.
Our Growing Research Portfolio

Total number of research projects awarded to date: 197
Total funds committed to date: $273.5 million
Number of states where we are funding research: 36 states (including the District of Columbia)
Minimum research commitment in 2013: $400 million
PCORI Pilot Projects

Advance methods for engaging patients in the research process and identify gaps in comparative clinical effectiveness research that need further development

By Health Topic

- Chronic Conditions 25%
- Cardiovascular Disease 13%
- Cancer 11%
- Behavioral Health 13%
- Acute 8%
- Geriatrics 10%
- Pediatrics 10%
- Rare Diseases 4%
- Other 6%
- Cardio-vascular Disease 13%

50 Awards
$31,000,000
Improving Healthcare Systems

Projects that address critical decisions that face healthcare systems, the patients and caregivers who rely on them, and the clinicians who work within them.

32 Awards

$58,978,563

By Health Topic

- Cardiovascular Disease: 18%
- Endocrine System: 16%
- Mental Disorders: 11%
- Cancer: 16%
- Nervous System Disorders: 8%
- Other: 24%
Projects that address critical elements in the communication and dissemination process among patients, their caregivers, and clinicians.

20 Awards
$33,502,693

By Population

- Racial / Ethnic Minority: 26%
- Elderly: 20%
- Rural: 11%
- Socio-economic: 9%
- Other: 17%
- Children: 17%

Some studies address multiple populations.
Addressing Disparities

Projects that inform the choice of strategies to eliminate disparities.

23 Awards
$38,409,798
Examples From our Portfolio (Comparative Clinical Effectiveness)

- Cognitive AED Outcomes in Pediatric Localization Related Epilepsy (COPE) [Project Detail]
- Long Term Outcomes of Lumbar Epidural Steroid Injections for Spinal Stenosis [Project Detail]
- Improving Patient Decisions about Bariatric Surgery [Project Detail]
- Evaluation of a Patient-Centered Risk Stratification Method for Improving Primary Care for Back Pain [Project Detail]
- Effect of Glucose Monitoring on Patient and Provider Outcomes in Non-insulin Treated Diabetes [Project Detail]
- Developing and Testing a Personalized, Evidence-Based, Shared Decision-Making Tool for Stent Selection in PCI [Project Detail]
Examples From our Portfolio (Improving Healthcare Systems)

- Using Telehealth to Deliver Developmental, Behavioral, and Mental Health Services in Primary Care Settings for Children in Underserved Areas  [Project Detail]
- The Family VOICE Study: A Randomized Trial of Family Navigator Services Versus Usual Care for Young Children Treated with Antipsychotic Medication  [Project Detail]
- Improving Childhood Obesity Outcomes: Testing Best Practices of Positive Outliers  [Project Detail]
- Changing the Healthcare Delivery Model: A Community Health Worker/Mobile Chronic Care Team Strategy  [Project Detail]
- Project: PATient Navigator to rEduce Readmissions (PArTNER)  [Project Detail]
- A Patient-Centered Approach to Successful Community Transition After Catastrophic Injury  [Project Detail]
Examples From our Portfolio (Other)

- CT Radiation Dose Registry to Ensure a Patient-Centered Approach for Imaging [Project Detail]
- Extension Connection: Advancing Dementia Care for Rural and Hispanic Populations [Project Detail]
- Effectiveness of DECIDE in Patient-Provider Communication, Therapeutic Alliance & Care Continuation [Project Detail]
- Reducing Disparities with Literacy-Adapted Psychosocial Treatments for Chronic Pain: A Comparative Trial [Project Detail]
- Peer Health Navigation: Reducing Disparities in Health Outcomes for the Seriously Mentally Ill [Project Detail]
- Patient Priorities and Community Context: Navigation for Disadvantaged Women with Depression [Project Detail]
- Improving Health Outcomes among Native Americans with Diabetes and Cardiovascular Disease [Project Detail]
- Statistical Methods for Missing Data in Large Observational Studies [Project Detail]
National Patient-Centered Clinical Research Network

- Up to 8 **Clinical Data Research Networks** (CDRNs), which are system-based networks (such as hospital systems) that have the potential to become an ideal electronic network, without structural impediments.

- Up to 18 **Patient-Powered Research Networks** (PPRNs), which are groups of patients interested in forming a research network and in participating in research.

- A **Coordinating Center** which will provide technical and logistical assistance under the direction of the Steering Committee and PCORI Staff.
## Targeted Funding Announcements

### Announced

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<thead>
<tr>
<th>Description</th>
<th>Date</th>
<th>Amount</th>
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<tbody>
<tr>
<td>Cooperative Agreement to address prevention of fall-related injuries in older adults <em>(with National Institute on Aging)</em></td>
<td>June 14</td>
<td>$30 million</td>
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<tr>
<td>PFA on Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma</td>
<td>December 17</td>
<td>$17 million</td>
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### In Development

<table>
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<th>Description</th>
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<tbody>
<tr>
<td>Cooperative Agreement on improving treatment for uterine fibroids <em>(with Agency for Healthcare Research and Quality)</em></td>
<td>December</td>
<td>$20 million</td>
</tr>
<tr>
<td>Obesity Treatment Options in Diverse Populations</td>
<td>TBD</td>
<td>TBD</td>
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<tr>
<td>Transitions in Care</td>
<td>TBD</td>
<td>TBD</td>
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<tr>
<td>Treatment Options for Back Pain</td>
<td>TBD</td>
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Accelerating PCOR and Methodological Research

Projects that aim to improve the nation’s underlying capacity to conduct patient-centered outcomes research rapidly, efficiently, and consistently over time.

Improving Methods for Conducting Patient-Centered Outcomes Research

September 10, 2013
19 projects, $18,045,991

National Patient-Centered Clinical Research Networks

Anticipated Award Date: December 2013
Data Infrastructure for Patient-Centered Outcomes Research

- Ideal Data Infrastructure for PCOR
  - Covers large, diverse, defined populations from usual care settings
  - Active patient and clinician engagement in governance of data use
  - Capacity for collecting patient reported outcomes
  - Allows for complete capture of longitudinal data
  - Linkages to health systems for rapid dissemination of findings
  - Is efficient in terms of costs for data acquisition, storage, analysis
  - Capable of randomization — at individual and cluster levels
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National Patient-Centered Clinical Research Network: Our Vision

Scientific Advisory Board

Steering Committee
- Awardees
- PCORI
- AHRQ, NIH, FDA, ONC, CMS, VA

Special Expert Group

Coordinating Center (CC)

CDRN

PPRN

Patient-Centered Outcomes Research Institute
Potential Participants

- Patient Organizations
- Practice Based Research Networks
- Medical Groups
- Medical Centers
- Health Plans
- Integrated Delivery Systems
- Disease Registries
- Centers for Medicaid and Medicare Services
- State and Local Health Agencies
Thank You!

Questions?

Keep in touch…
- gmartin@pcori.org

Get Involved
- pcori.org/get-involved