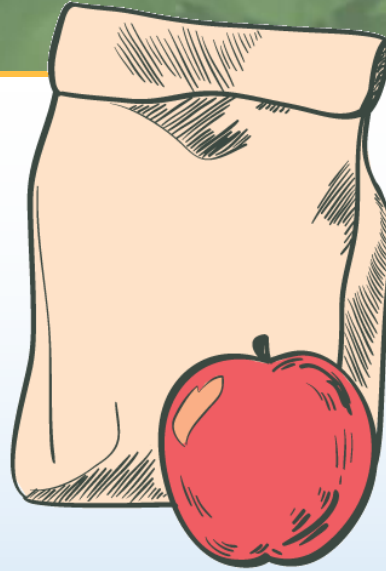


# The Academy Capitol Forum: Meet the Experts



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

**Presenter:** Gregory Martin, Deputy Directory of Stakeholder Engagement  
Patient Centered Outcomes Research Institute

**Moderator:** Cori Uccello, Senior Health Fellow  
American Academy of Actuaries





# Which Health Treatments Improve Outcomes? A PCORI Overview

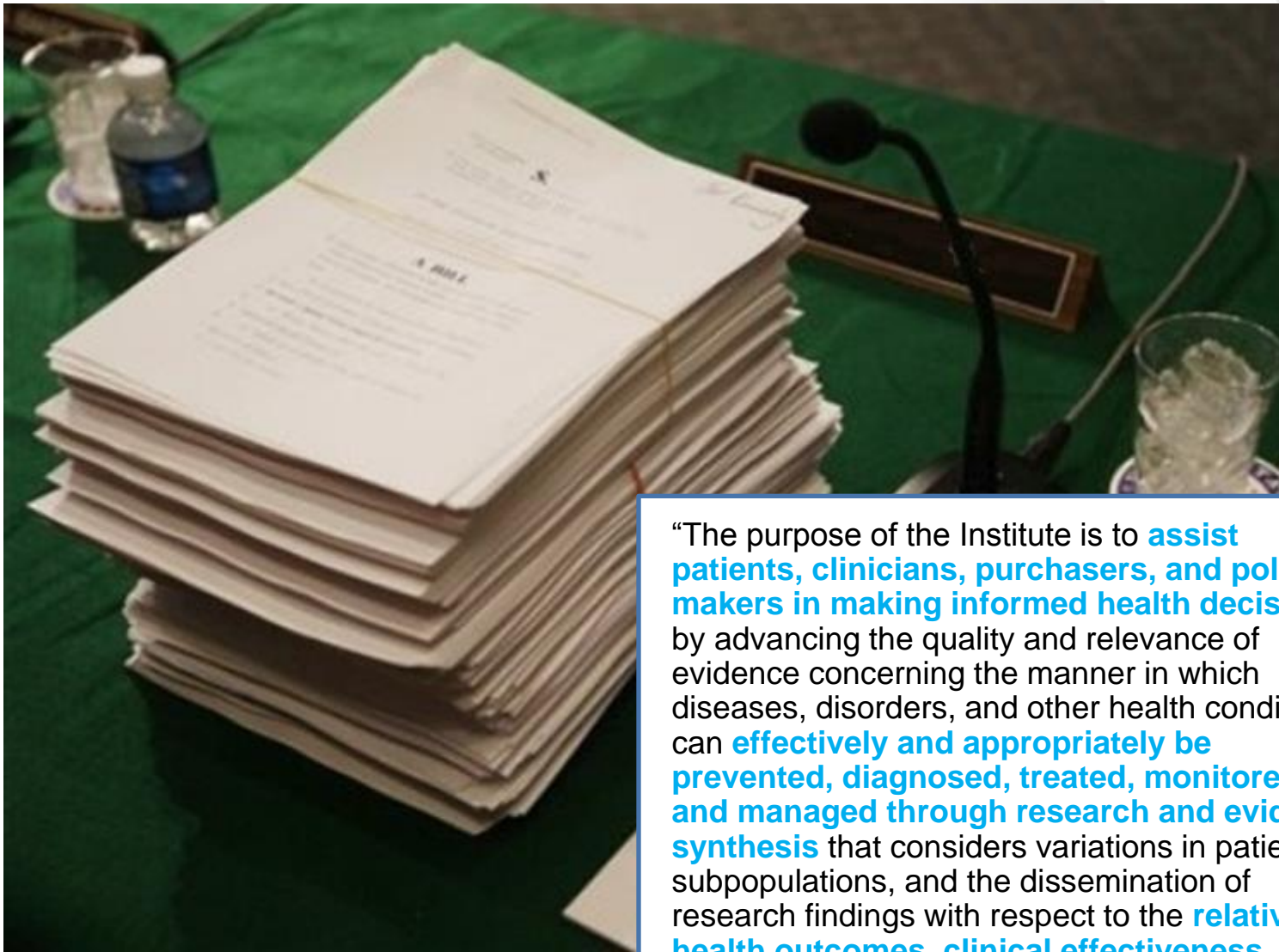
*Greg Martin, Deputy Director, Stakeholder Engagement  
American Academy of Actuaries  
October 16, 2013*

Patient-Centered Outcomes Research Institute

# 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.



## 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?”**

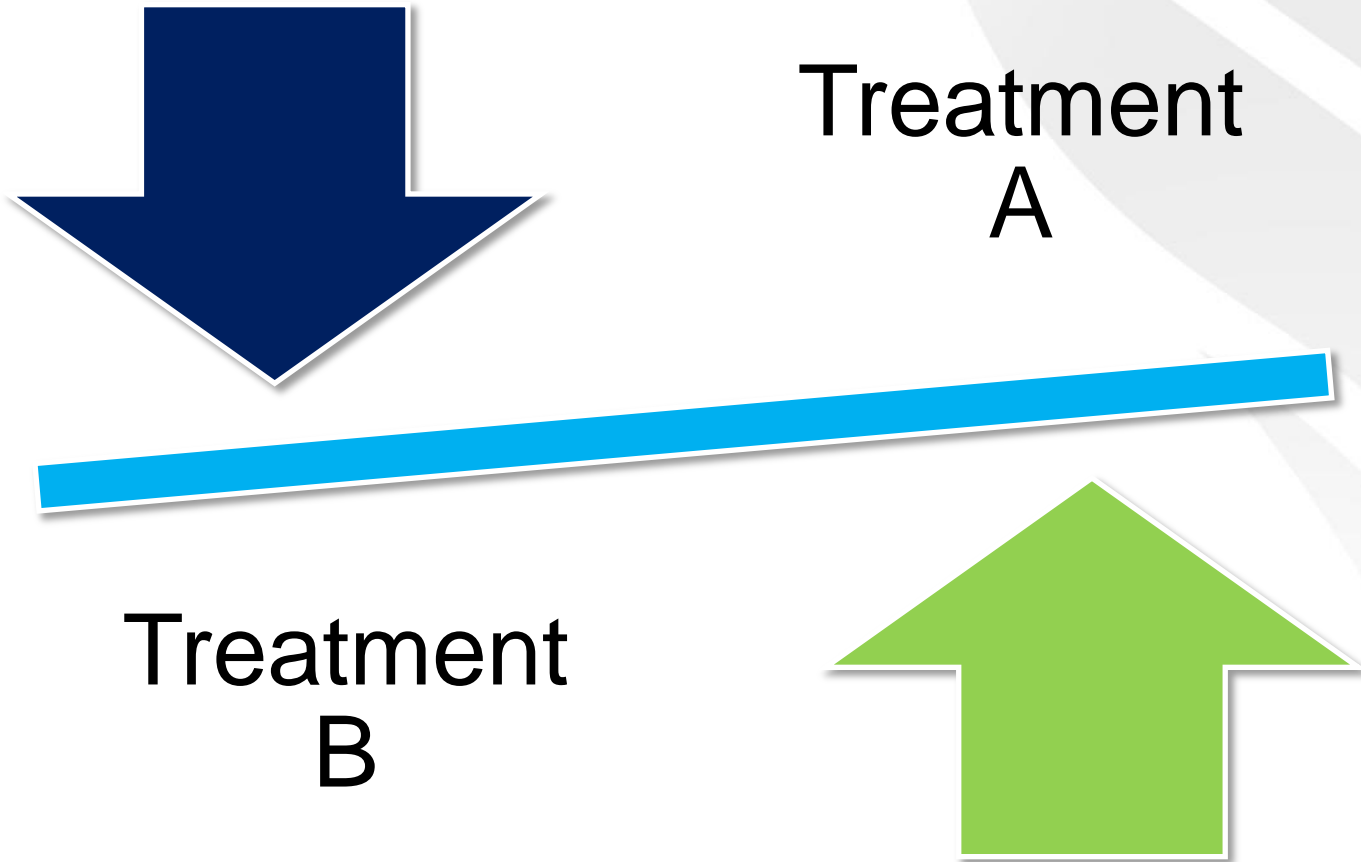
# Comparative Clinical Effectiveness Research (CER)

## PCORI's Definition

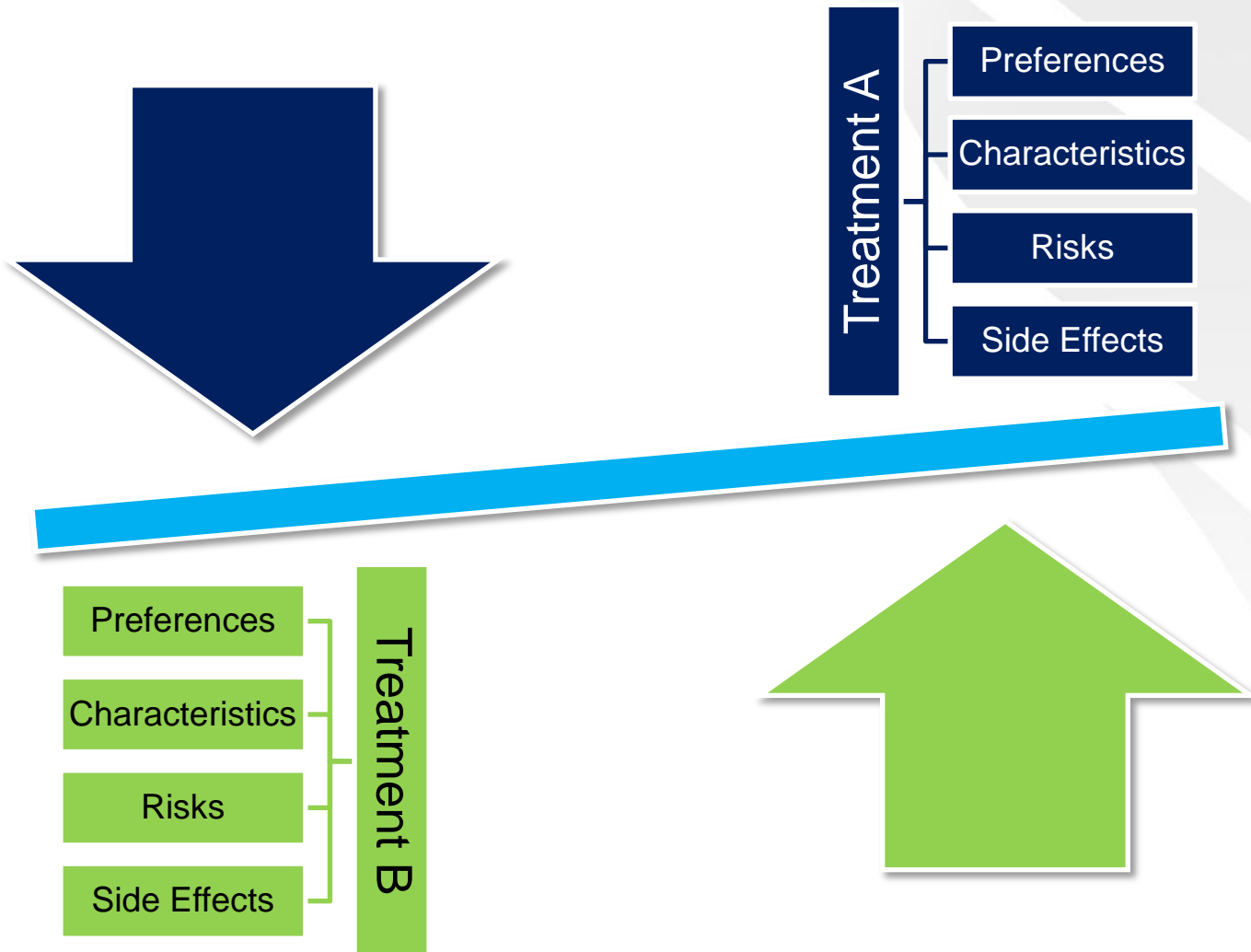
- Compares two or more health and healthcare choices faced by patients, caregivers, clinicians, and other healthcare stakeholders
- Seeks to answer a medical research question for which there is not sufficient evidence or there is contradicting information
- Is likely to provide evidence that can reduce 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



# The Simplest Explanation



# Getting to the Comparative Question

**Health  
Question**

Am I taking the right blood pressure medication?



**CER  
Question**

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

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**



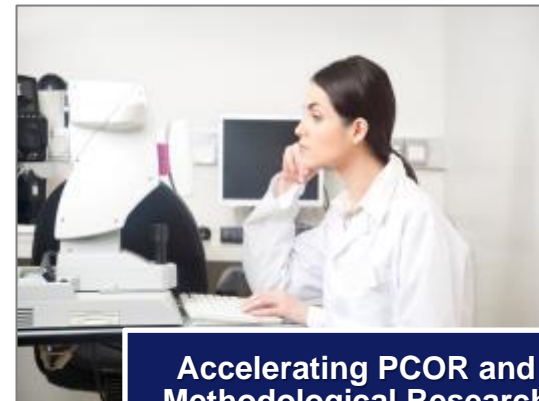
**Improving Healthcare Systems**



**Communication & Dissemination Research**



**Addressing Disparities**



**Accelerating PCOR and Methodological Research**





**1. Gather Suggestions**

**Patient Stakeholder Community**

**2. Evaluate Suggestions**

**PCORI Staff**

**3. Prepare Topic Briefs**

**AHRQ and Academic Centers**

# How We Pick Research Questions to Study

**6. Release Funding Announcements**

**PCORI Staff**

**5. Select Suggestions**

**PCORI Board**

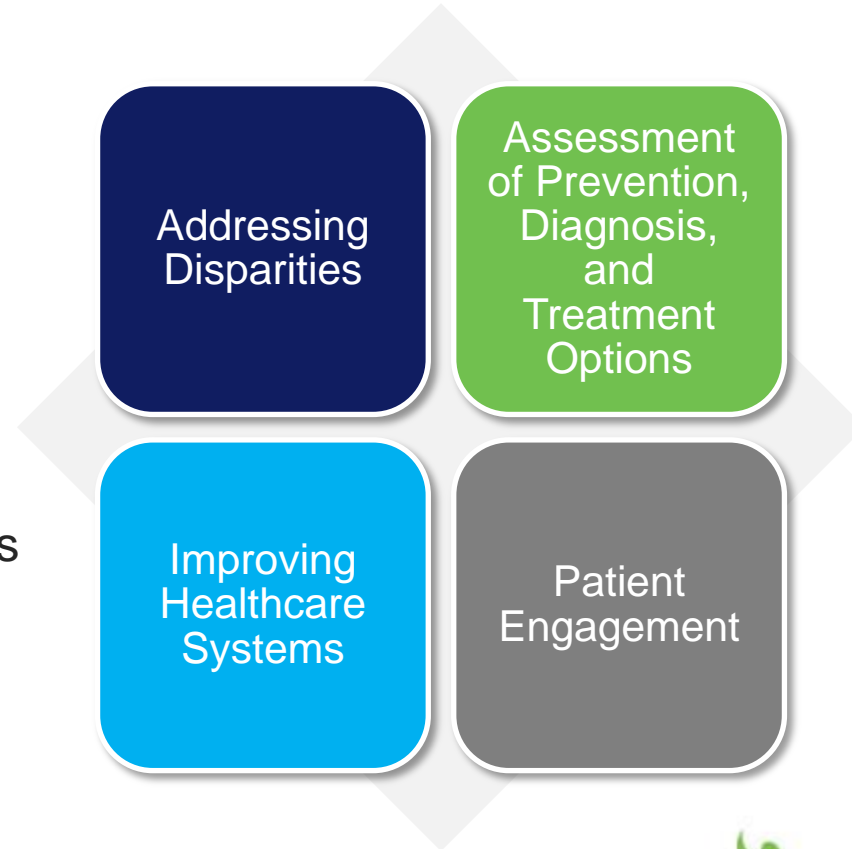
**4. Prioritize Topics**

**Advisory Panels**

# 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](https://www.pcori.org/reviewers)

[Contact us  
reviewers@pcori.org](mailto: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.

# Actions Outside PCORI's Scope of Work

- 🌱 **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.

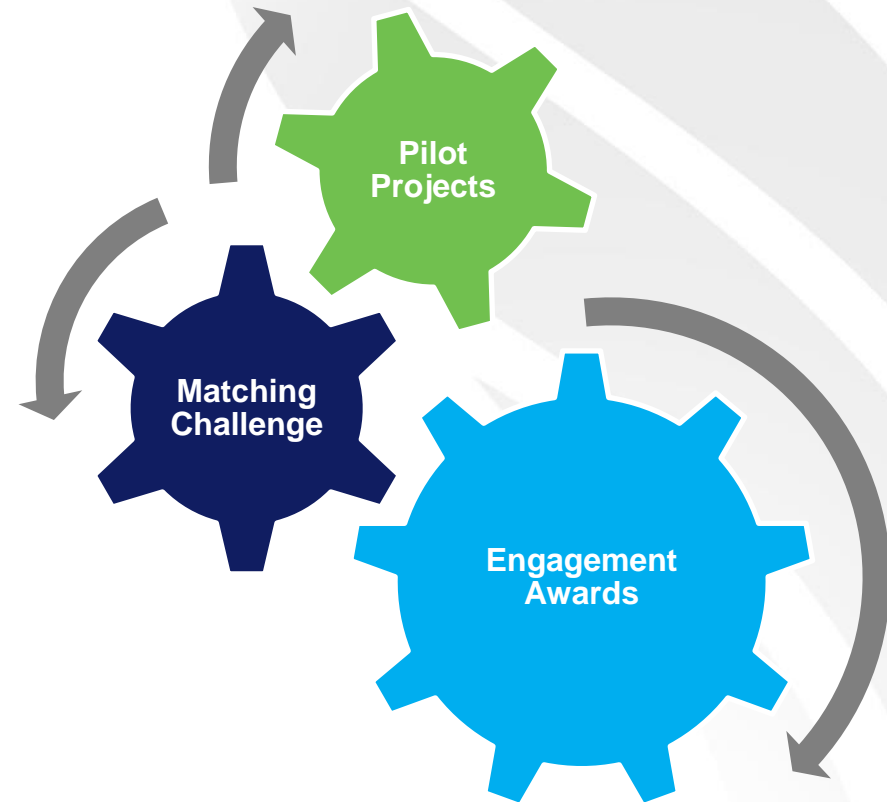


- Formulating Research Questions
- Patient-Centeredness
- Systematic Reviews
- General and Crosscutting Methods For All PCOR
- Causal Inference Standards
- Heterogeneity of Treatment Effect (HTE)
- Preventing and Handling Missing Data
- Data Networks
- Adaptive and Bayesian Trial Designs
- Data Registries
- 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:

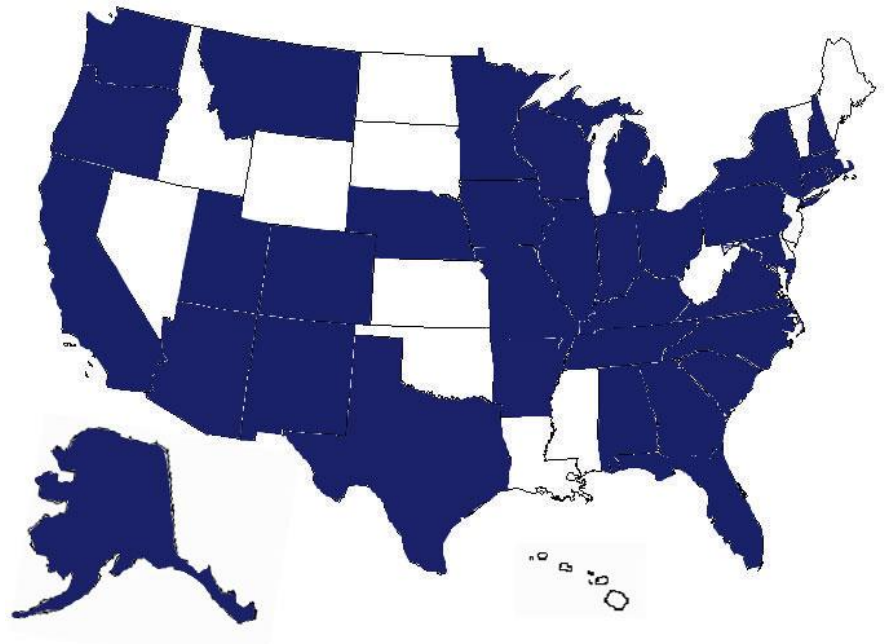
**\$273.5 million**

Number of states where we are funding research:

**36 states** (including the District of Columbia)

Minimum research commitment in 2013:

**\$400 million**

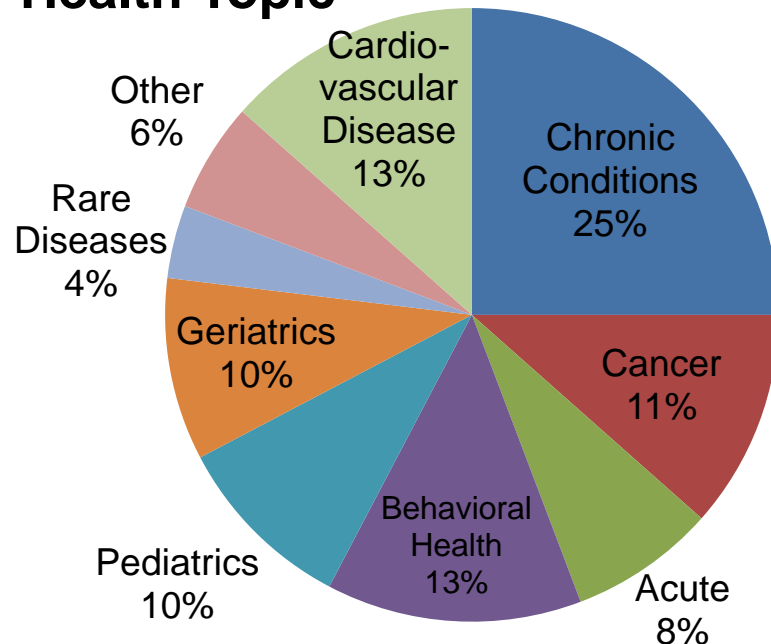


# PCORI Pilot Projects



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

## By Health Topic



**50 Awards**  
**\$31,000,000**



Patient-Centered Outcomes Research Institute



# Improving Healthcare Systems

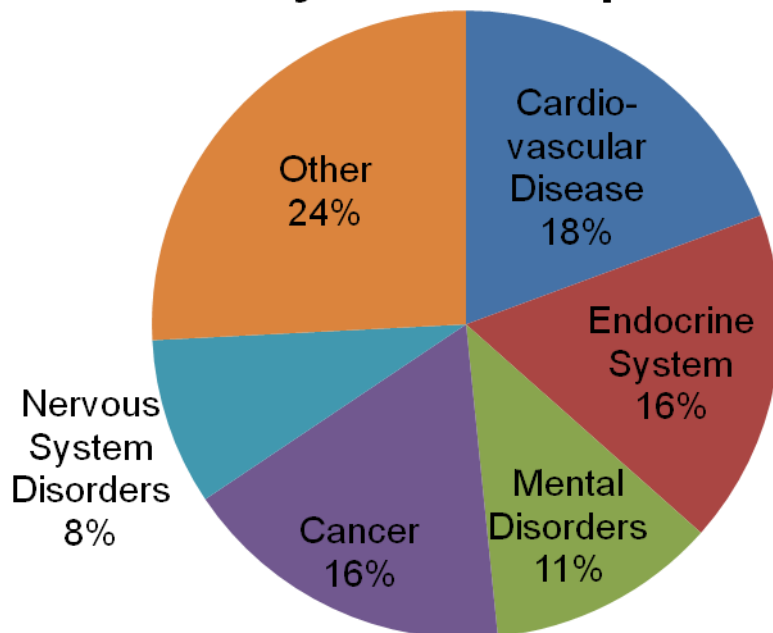


Projects that address critical decisions that face healthcare systems, the patients and caregivers who rely on them, and the clinicians who work within them.

## 32 Awards

## \$58,978,563

By Health Topic



# Communication & Dissemination Research

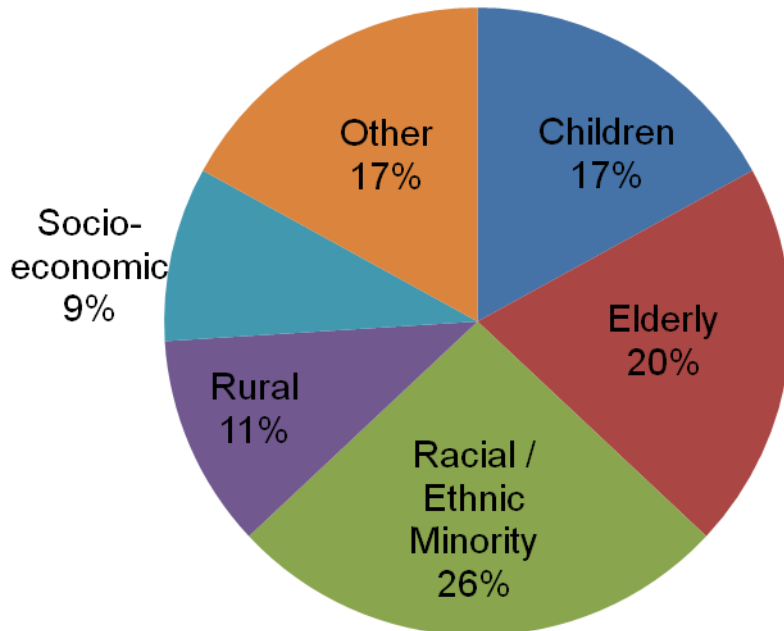


Projects that address critical elements in the communication and dissemination process among patients, their caregivers, and clinicians.

## 20 Awards

## \$33,502,693

### By Population



*Some studies address multiple populations.*

# Addressing Disparities

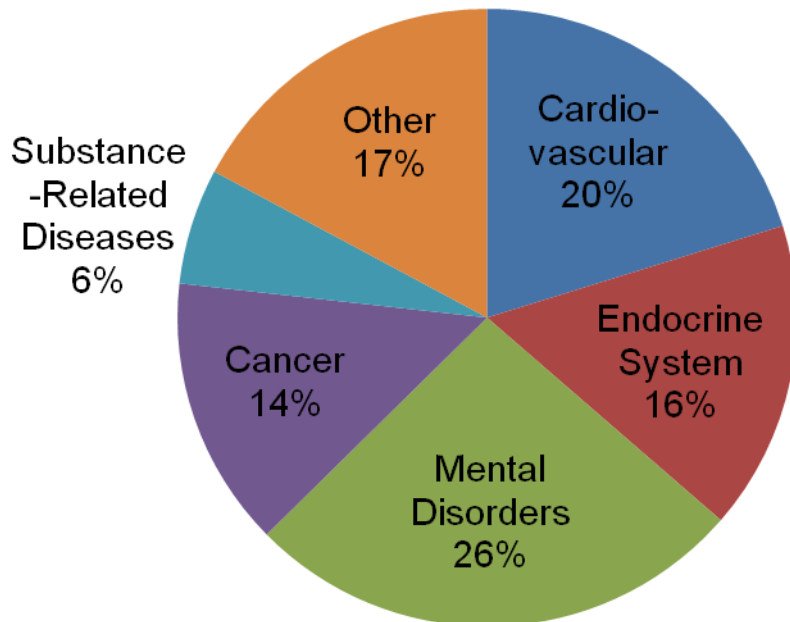


Projects that inform the choice of strategies to eliminate disparities.

## 23 Awards

## \$38,409,798

By Health Topic



# 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

Cooperative Agreement to address prevention of fall-related injuries in older adults <i>(with National Institute on Aging)</i>	<b>June 14</b>	<b>\$30 million</b>
PFA on Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	<b>December 17</b>	<b>\$17 million</b>

## In Development

Cooperative Agreement on improving treatment for uterine fibroids <i>(with Agency for Healthcare Research and Quality)</i>	<b>December</b>	<b>\$20 million</b>
Obesity Treatment Options in Diverse Populations	<b>TBD</b>	<b>TBD</b>
Transitions in Care	<b>TBD</b>	<b>TBD</b>
Treatment Options for Back Pain	<b>TBD</b>	<b>TBD</b>



# 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.

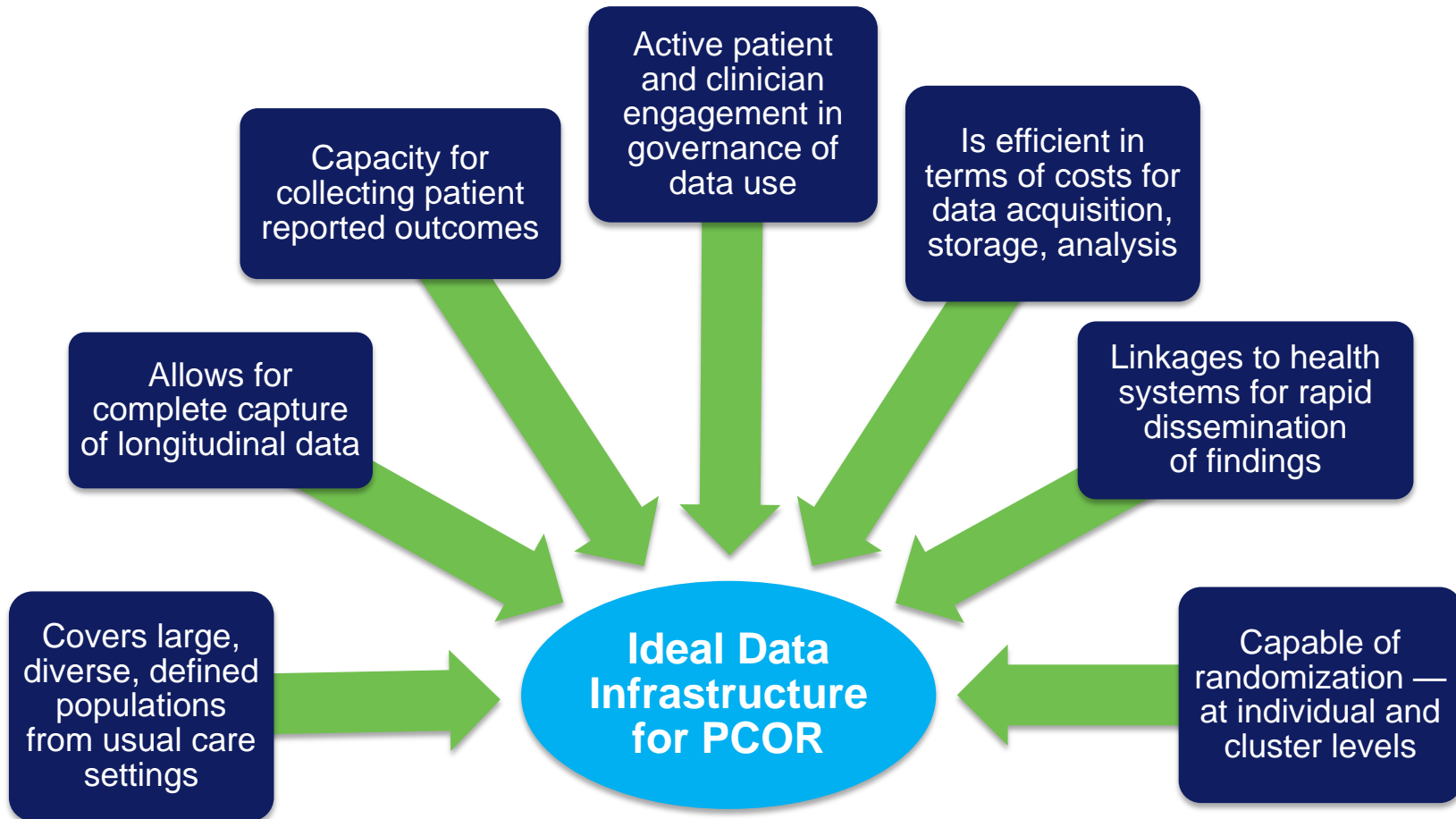
## Improving Methods for Conducting Patient-Centered Outcomes Research

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



# National Patient-Centered Clinical Research Network



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# National Patient-Centered Clinical Research Network: Our Vision

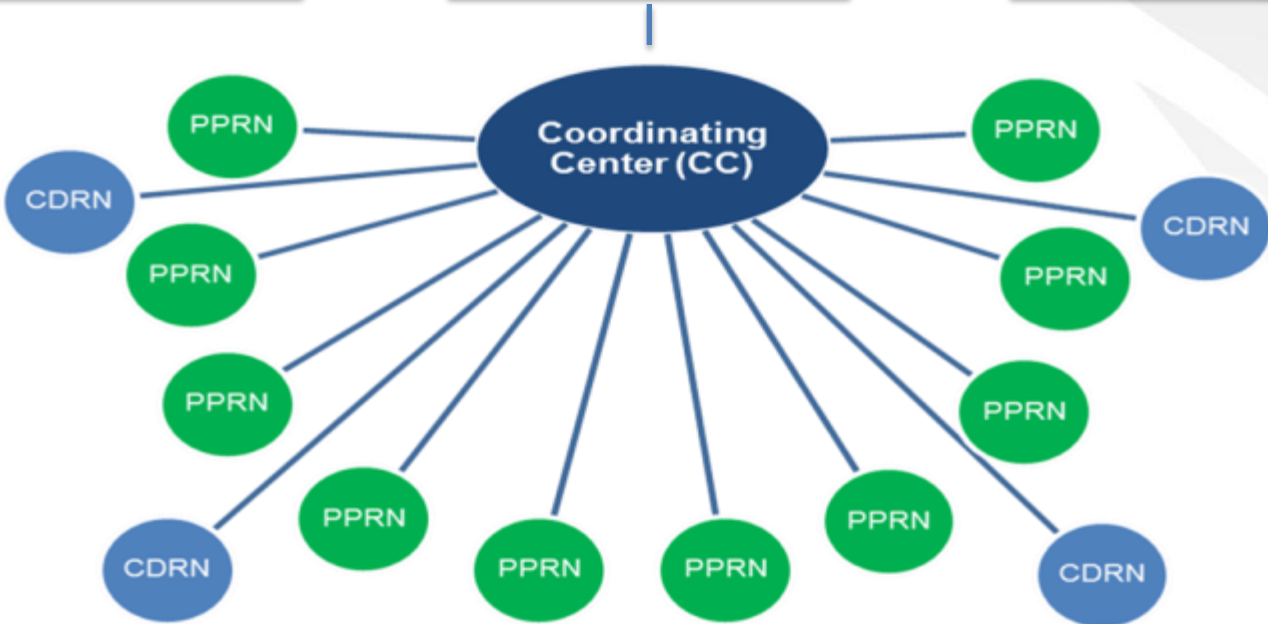


**Scientific Advisory Board**

**Steering Committee**

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

**Special Expert Group**



# 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](mailto:gmartin@pcori.org)

 Get Involved

- [pcori.org/get-involved](http://pcori.org/get-involved)