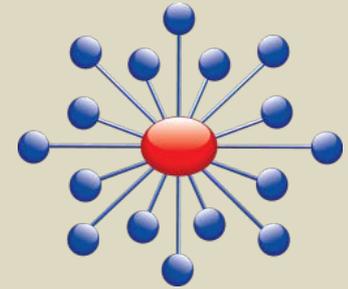


National Drug Abuse Treatment

*Clinical Trials Network*

*Forging partnerships to improve the  
quality of drug abuse treatment  
throughout the nation.*



# Overview of PCORI

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January 8, 2015  
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# PCORI's Vision for Patient Centered Research

*Joe Selby, MD, MPH*

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*Bethesda, MD*

*January 8, 2015*

Patient-Centered Outcomes Research Institute

# Key Questions for this Presentation

- What are the important features of patient centered outcomes research (PCOR)?
- What funding programs has PCORI launched?
- How is PCORI promoting best practices in research?
  - Methodology standards
  - Methodological Research Program
- What types of projects have the best chance of receiving funding from PCORI?

# About PCORI

- An independent research institute authorized by Congress through the Patient Protection and Affordable Care Act.
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process. Treatment of opioid substance abuse
- Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns.



# Why PCORI?

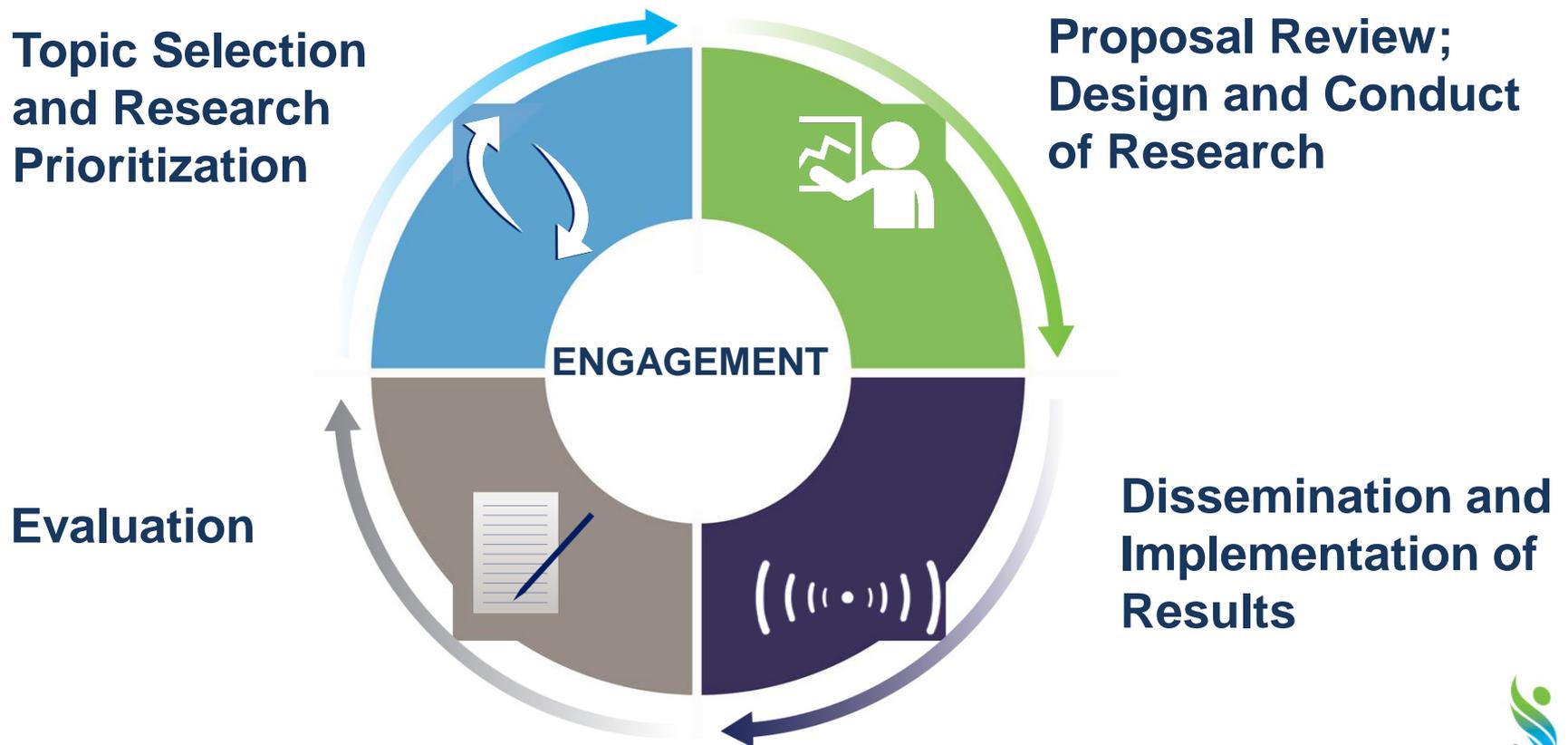
- 🌍 For all the advances it produces, research still has not answered many questions patients face.
- 🌍 People want to know which treatment is best for them.
- 🌍 Patients and their clinicians need information they can understand and use.



# Who Are Our Stakeholders?



# Engagement as a Path To Useful, High-Quality Research



# What Types of Research Does PCORI Support

From the Authorizing Legislation:

“The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and **comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items...**”

# What is Evidence-based Information?

- 🌐 Clinical evidence: Valid data about the outcomes experienced by patients who receive medical care.
  - The population is well defined.
  - The clinical interventions are well defined.
  - We have information about the most important outcomes (both benefits and harms).
- 🌐 Comparative effectiveness
  - Starting point is the choices people make about the options for managing a disease.
  - These choices inform the focus of new research.
  - The research compares the benefits and harms associated with each option.

# Perspectives on Comparative Effectiveness Research

- Comparative Effectiveness Research should be a public good that:
  - Gives health care decision makers – patients, clinicians, purchasers and policy makers – access to the latest open and unbiased evidence-based information about treatment options
  - Informs choices and is closely aligned with the sequence of decisions patients and clinicians face

# What Healthcare Decision Makers Need To Know

- 🌐 *Can* it work?
- 🌐 *Will* it work?
  - For this patient?
  - In this setting?
- 🌐 Is it *worth* it?
  - Do benefits outweigh harms?
  - Do benefits justify costs?
  - Does it offer important advantages over existing alternatives?

# Necessary Steps in Developing New Comparative Effectiveness Research

- ④ Understand the choices made by patients and clinicians
- ④ Define the important patient sub-groups
- ④ Define the outcomes (benefits and harms) that are important to patients
- ④ Assess the available evidence about important outcomes
  - Systematic reviews
  - Evidence gaps that are important to decision makers
- ④ Design a study that can feasibly close the evidence gap
  - If the gap is not important, the research will not be useful.

# What is Patient-Centered Outcomes Research?

- Helps people and their caregivers communicate and make better-informed healthcare decisions.
- Actively engages patients and key stakeholders throughout the research process.
- Compares the effectiveness of important clinical management options.
- Evaluates the outcomes that are the most important to patients.
- Addresses implementation of findings in clinical care environments.

# Key Features of Research Supported by PCORI

## The research should:

- Study the benefits and harms of interventions and strategies delivered in *real-world* settings
- Be likely to improve current clinical practices

## Special topics of interest:

- Conditions that heavily burden patients, families and/or the health care system.
- Chronic or multiple chronic conditions
- Rare and understudied conditions
- Conditions for which outcomes vary across subpopulations
- Conditions having important evidence gaps

# Development of PCORI's Funding Programs

- National research priorities (April 2012)
  - Broad framework that provides overall direction to the funding initiatives
  - Not based on clinical priorities
  - Majority of the funding thus far
- Initiatives based on stakeholder-derived research priorities
  - Single-cycle targeted announcements
  - Infrastructure: PCORnet
  - Pragmatic studies initiative (prioritized clinical topics)

# 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**

# PCORI's Research Programs

## CER

- Assessment of Prevention, Diagnosis and Treatment Options
- Pragmatic Studies

## Application of Best Evidence to Improve Care

- Communication and Dissemination Research
- Improving Healthcare Systems
- Addressing Disparities

## Improving the infrastructure for CER

- Data Infrastructure (PCORNet)
- Methods



# New Initiatives Derived from Stakeholder-Based Clinical Priorities

- Opportunity to identify important evidence gaps
  - Nomination of clinical topics
  - Advisory panels
- Pragmatic Studies Announcement
  - PFA first released in January 2014.
    - Third cycle is underway.
    - Fourth cycle in first half of 2015.
  - Competitive LOIs.
  - Larger budgets and longer project durations.
  - Up to \$90 million per cycle.

# Priority Topics for the Pragmatic Studies Program

- Treatments to prevent the transition from episodic to chronic migraine
- Smoking cessation therapies in high risk persons
- Treatments to prevent the transition from episodic to chronic low back pain
- Treatment strategies for osteoarthritis
- Treatment of opioid substance abuse

# PCORI's Interest in Treatment Options for Hepatitis C Infection

- Topic nominated by multiple stakeholder groups in mid-2014.
- PCORI held workshop for stakeholder input in October 2014.
  - Identified 4 areas of priority research questions
- The PCORI BoG approved development of a funding program in December 2014.
  - Planned release of announcement in February 2015

# Communication and Dissemination Research

## Seeks to fund investigator-initiated research in:

- Mechanisms for communicating complex information
- Risk communication, health literacy, and communicating uncertainty.
- Mechanisms to overcome issues of numeracy.

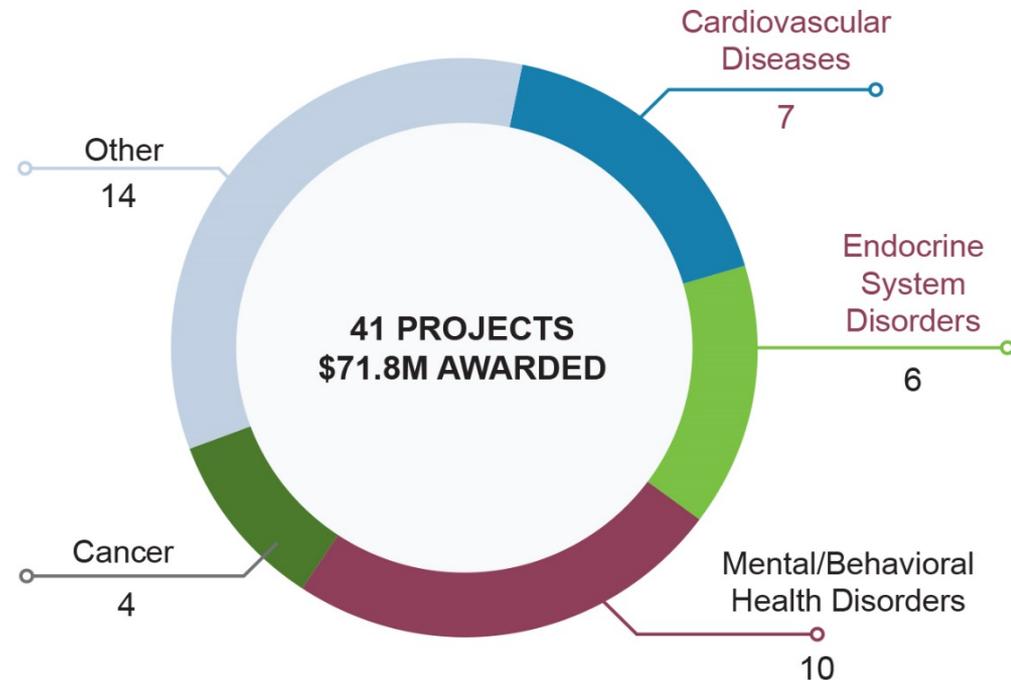
## Portfolio Snapshot

- **33 Projects**
- **\$56.7 Million Awarded**

# Addressing Disparities

## Seeks to fund investigator-initiated research that:

- Compares interventions to reduce or eliminate disparities across different patient populations.
- Identifies/compares promising practices that address contextual factors and their impact on outcomes.
- Compares and identifies best practices within various patient populations for information sharing about outcomes and research.

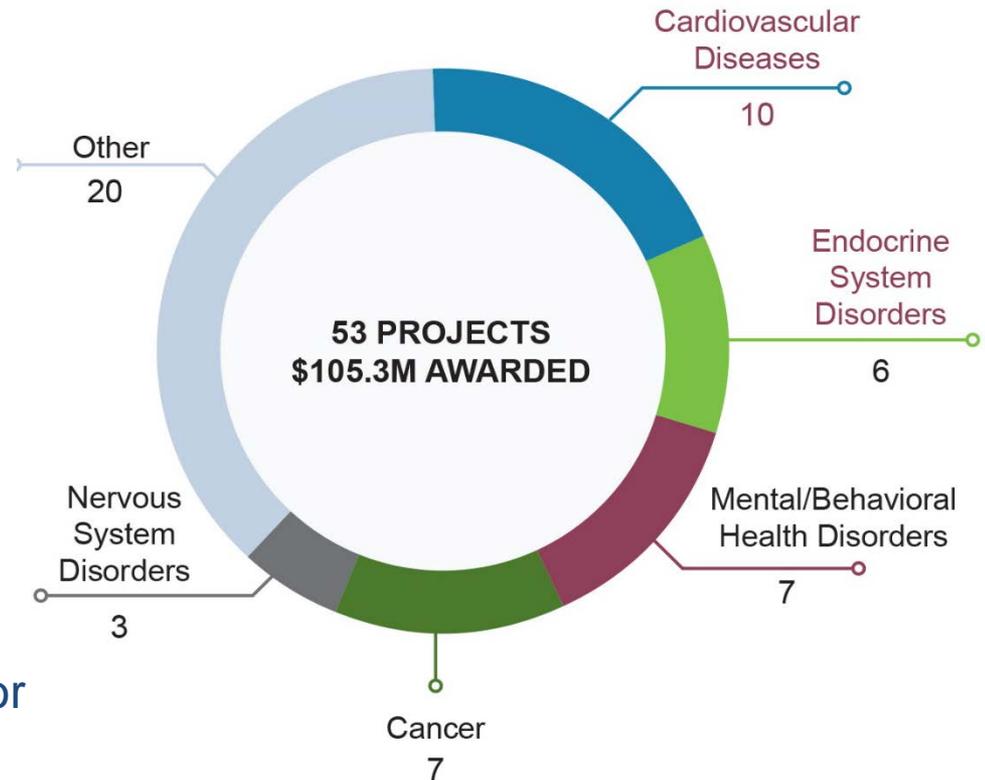


*By primary health topic*

# Improving Healthcare Systems

Seeks to fund investigator-initiated research on effects of system changes on :

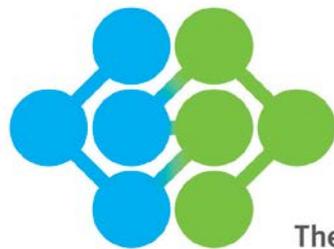
- Patients' access to high quality, support for self-care, and coordination across healthcare settings.
- Overall health, functional ability, quality of life, stress, and survival.
- The efficiency of healthcare delivery, as measured by the amount of ineffective, duplicative, or wasteful care provided to patients.



*By primary health topic*

# The National Patient-Centered Clinical Research Network (PCORnet)

- Improve the nation's capacity to conduct clinical research more efficiently, by creating a large, highly representative, national patient-centered clinical research network with a focus on conducting comparative studies – both randomized and observational.
- Support a learning US healthcare system, which would allow for **large-scale research** to be conducted with **enhanced accuracy and efficiency** within real-world care delivery systems.



pcornet

The National Patient-Centered Clinical Research Network



Patient-Centered Outcomes Research Institute

# PCORnet



## **11 Clinical Data Research Networks (CDRNs)**

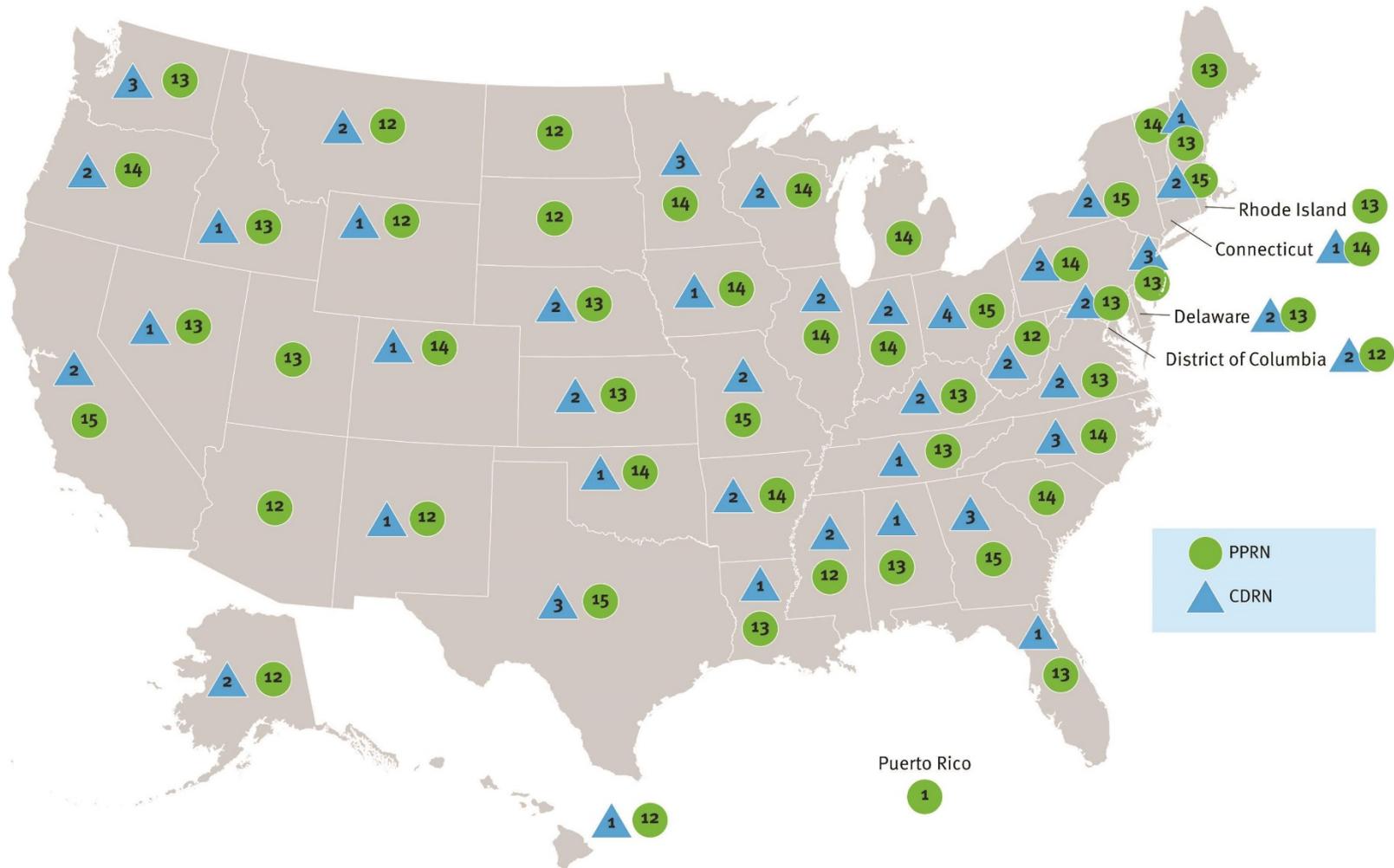
System-based networks, such as hospital systems  
\$76.8 million awarded



## **18 Patient-Powered Research Networks (PPRNs)**

Patients with a single condition form a research network  
\$16.8 million awarded

# Geographic Coverage of PPRNs and CDRNs



# Some Projects are Outside of PCORI's Priorities

- Cost-effectiveness analyses or studies that primarily address costs of care as an outcome.
- Studies of the efficacy of unproven treatments.
- Natural history studies.

# We Work to Improve Research Methodology

In any study, methods matter. That's why we've developed methodology standards that all research should follow, at a minimum.

## Methodology Standards: 11 Broad Categories

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects
- Data Networks
- Data Registries
- Adaptive and Bayesian Trial Designs
- Causal Inference
- Studies of Diagnostic Tests
- Systematic Reviews

# Characteristics of the Methodology Standards

- Are minimal standards for performing comparative effectiveness research.
- Are intended to provide helpful guidance to researchers and those who use research results.
- Reflect generally accepted best practices.
- Provide guidance for both project protocols and reporting of results.
- Are used to assess the scientific rigor of funding applications.
- Context of the research should drive use of the standards.

# Improving Methods for Conducting Patient-Centered Outcomes Research

**Seeks to fund investigator-initiated research that:**

- Addresses gaps in methodological research relevant to conducting PCOR. Results of these projects will inform future iterations of PCORI's Methodology Report.
- Focuses on Patient-Reported Outcome Measurement Information System (PROMIS)-related research.

- **58 Projects**
- **\$54.8 Million Awarded**



# Which Strategies are Likely to be Successful when Seeking Funding?

- PCOR should compare clear clinical options.
  - Be cautious with a “usual care” comparator.
  - The clinical interventions should be easy to replicate: path to dissemination.
- Make sure that the outcomes are meaningful (both benefits and harms).
- Cover all of the Methodology Standards.
- Engagement, engagement, engagement.

# Strategies for Preparing a Successful Funding Application

- Your proposal should tell the story of why the research study is important to all reviewers.
- Don't assume reviewers know something about your project that you don't address.
- Be clear on what is to be gained if your study is funded.

# Conclusions

- Patient-centered outcomes research (PCOR) provides relevant information to help patients and providers choose among alternative clinical strategies.
- Applicants for funding should engage patients and stakeholders in identifying questions and defining important outcomes.
- Projects should directly address important clinical comparisons and examine meaningful outcomes.
- The PCORI Methodology Standards guide best practices for planning, conducting and reporting research.

# Join Us at PCORI.org

