

PCORI Funding Announcements (PFAs), Methodology Standards, Approach to Open Science and National Priorities

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Welcome!



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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
 - Open science initiatives
- Which projects have the best chance of receiving PCORI funding?



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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 two or more medical treatments, services, and items...**”



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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



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Perspectives on Comparative Effectiveness Research

- Comparative Effectiveness Research (CER) 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



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First Steps in Developing New CER

- Understand the choices made by patients and clinicians
 - Which clinical options are realistically available to patients?
- Define the important patient sub-groups
 - Recognize disparities and their sources
- Define the outcomes (benefits and harms) that are important to patients
 - Benefits
 - Harms



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New CER Must Address Important Evidence Gaps

- Assess the available evidence about important outcomes
 - Systematic reviews
 - Evidence gaps that are important to
 - Decision-makers
- Design a study that can feasibly close the evidence gap
 - If the gap is not important, the research will not be useful.



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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



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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
 - Conditions having important evidence gaps
 - Chronic or multiple chronic conditions
 - Rare and understudied conditions
 - Conditions for which outcomes vary across subpopulations



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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)



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Our National Priorities for Research



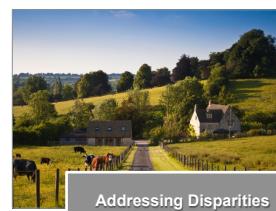
Assessment of Prevention,
Diagnosis and Treatment Options



Improving Healthcare
Systems



Communication &
Dissemination Research



Addressing Disparities



Accelerating PCOR and
Methodological Research



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A Simple Guide to 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
 - Pragmatic Studies
- Improving the infrastructure for CER
 - Data Infrastructure (PCORNet)
 - Methods



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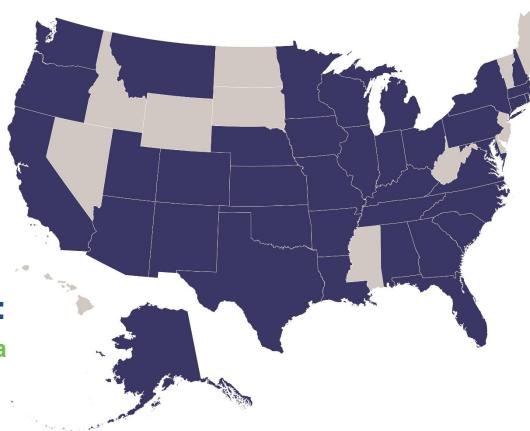
Snapshot of Funded Projects

Number of projects:
360

Amount awarded:
\$671 million

**Number of states where
we are funding research:**
**39 (plus the District of Columbia
and Quebec)**

As of September 30, 2014



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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
 - List of priority clinical and health systems topics
 - Competitive LOIs
 - Larger budgets and longer project durations
 - Up to \$90 million per cycle



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We Target Specific, High-Priority Topics

- **Assessment of Prevention, Diagnosis and Treatment Options**
 - PCOR Treatment Options in Uterine Fibroids*
 - Treatments for Hepatitis C Infection
- **Improving Healthcare Systems**
 - Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons**
 - Effectiveness of Transitional Care
- **Addressing Disparities**
 - Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma
 - Obesity Treatment Options Set in Primary Care for Underserved Populations
 - Clinical Interventions to Address Hypertension Disparities

* Administered by AHRQ

** Administered by the National Institute on Aging



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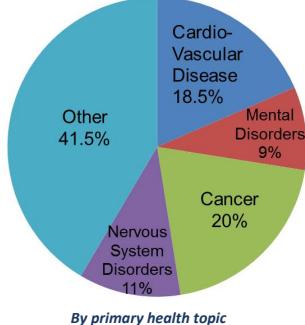
Assessment of Prevention, Diagnosis, and Treatment Options

Seeks to fund investigator-initiated research that:

- Compares the effectiveness of two or more options that are known to be effective but have not been adequately compared in previous studies
- Focuses on topics relating to individual decisions made by patients and their providers
- Investigates factors that account for variation in treatment outcomes across patient groups

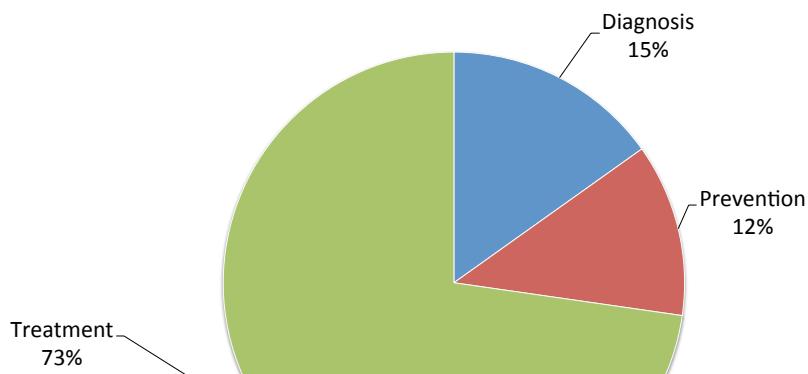
Portfolio Snapshot

- 83 Projects
- \$149 Million Awarded



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Clinical Focus of Previously Funded Projects PCORI Assessment of Options Program



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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



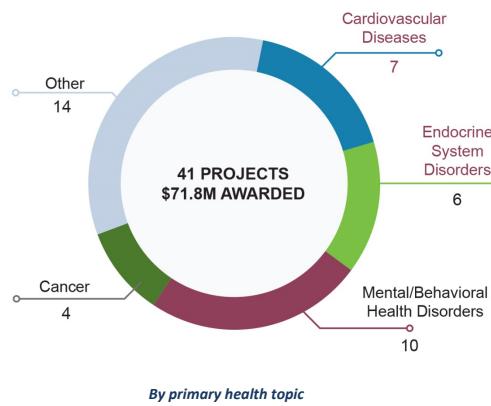
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Addressing Disparities

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



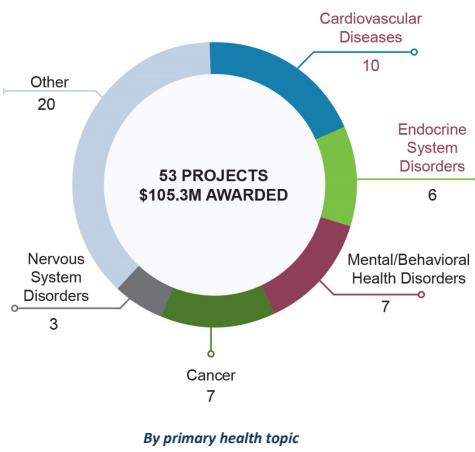
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Improving Healthcare Systems

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



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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.



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PCORnet



11 Clinical Data Research Networks (CDRNs)

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



18 Patient-Powered Research Networks (PPRNs)

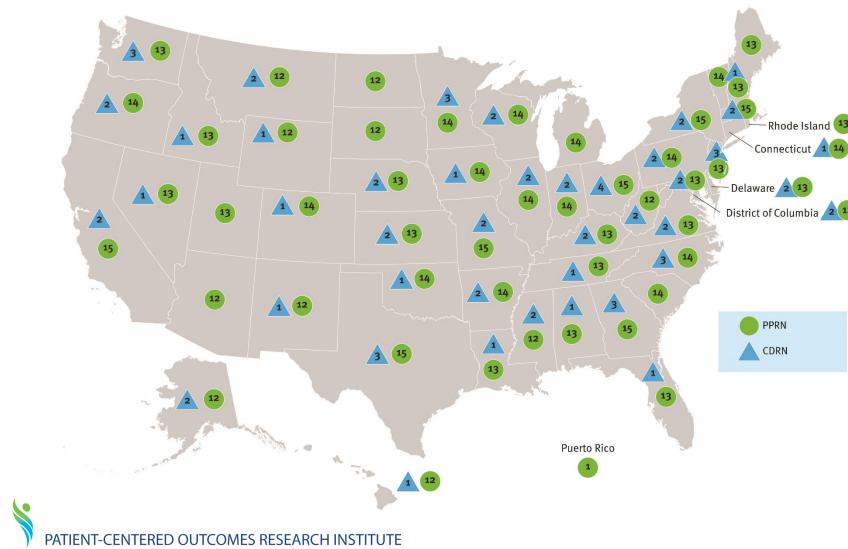
**Patients with a single condition form a research network
\$16.8 million awarded**



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Geographic Coverage of PPRNs and CDRNs



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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



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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



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Characteristics of the Methodology Standards

- Are minimal standards for performing CER
- 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



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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**



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PCORI has a Mandate to Promote Open Science and Research Transparency

From the PCORI Legislation:

- Conduct Peer Review of Primary Research
 - Assess scientific integrity
 - Assess adherence to PCORI's Methodology Standards
- Ensure Timely Release of Research Findings
 - No later than 90 days after "conduct or receipt"
 - Provide comprehensible and useful information to patients and providers for healthcare decisions
 - Address subpopulations, risk factors, and comorbidities
 - Disclose conflicts of interest
 - Include limitations and further research needed



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PCORI's Open Science Plan

- Registration of all funded studies
 - Register on clinicaltrials.gov
 - Submission of full study protocol
- Scrutiny of final reports
 - Required submission within 90 days of completion of data analyses for main outcomes
 - External peer review of draft final reports
 - Public posting of final reports/publications
- Development of translational products
 - For clinicians
 - For patients



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Summing Up: 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



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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



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Conclusions

- PCORI 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.



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Join Us at PCORI.org

The screenshot shows the PCORI website with a white header containing the PCORI logo and navigation links: About Us, What We Do, Funding Opportunities, Meetings & Events, Get Involved, News Room, and Employment. Below the header is a large banner with a red envelope icon and the text "Keep Informed about PCORI Funding Announcements and Opportunities to Get Involved" followed by a "Join our Mailing List" button. To the right of the banner is a sidebar titled "More News From PCORI" with three news items: "Two Leading Scientists to Direct Core Research..." (with a "more" link), "Submit a Statement of Interest..." (with a "more" link), and "PCORI Outlines Vision in Televised Interview..." (with a "more" link). At the bottom of the page are four social media links: Slideshare, YouTube, and Twitter, along with the text "PCORI INSTITUTE".

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Questions?

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Thank You!

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