Overview of PCORI

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National Drug Abuse Treatment
Clinical Trials Network
Forging partnerships to improve the quality of drug abuse treatment throughout the nation.

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PCORI’s Vision for Patient Centered Research

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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
- 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.

Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns.
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?

PCORI Community

- Patient/Consumer
- Caregiver/Family Member of Patient
- Clinician
- Payer
- Industry
- Policy Maker
- Hospital/Health System
- Training Institution
- Purchaser
- Patient/Caregiver Advocacy Org

Patient-Centered Outcomes Research Institute
Engagement as a Path To Useful, High-Quality Research

- Proposal Review; Design and Conduct of Research
- Dissemination and Implementation of Results
- Topic Selection and Research Prioritization
- Evaluation
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.
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

- Understand the choices made by patients and clinicians
- Define the important patient sub-groups
- Define the outcomes (benefits and harms) that are important to patients
- 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.
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
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
New Initiatives Derived from Stakeholder-Based Clinical Priorities

- Opportunity to identify important evidence gaps
  - Nomination of clinical topics
  - Advisory panels

Pragmatic Studies Announcement
  - 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.
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.

53 PROJECTS
$105.3M AWARDED

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

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.
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.
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