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?
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.
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
First Steps in Developing New Comparative Effectiveness Research

- 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
New Comparative Effectiveness Research 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.
Engage Stakeholders/Patients to Help

- Formulate research questions
- Design the study to
  - integrate with routine clinic/office operations
  - minimize disruption to participant’s daily routine
- Refine recruitment strategies and proactively deal with recruitment issues
- Participate in data monitoring and safety activities
- Capitalize on existing resources (e.g., electronic health records, claims databases, networks)
  - to collect study outcomes information
- Disseminate the study findings
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.
- Conditions having important evidence gaps
- Chronic or multiple chronic conditions
- Rare and understudied conditions
- Conditions for which outcomes vary across subpopulations
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
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
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.
  - List of priority clinical and health systems topics.
  - Competitive LOIs.
  - Larger budgets and longer project durations.
  - Up to $90 million per cycle.
We Target Specific, High-Priority Topics

Assessment of Prevention, Diagnosis and Treatment Options
- PCOR Treatment Options in Uterine Fibroids*

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
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**

By primary health topic:
- Cardiovascular Disease 18.5%
- Cancer 20%
- Nervous System Disorders 11%
- Mental Disorders 9%
- Other 41.5%
Clinical Focus of Previously Funded Projects
PCORI Assessment of Options Program

- Treatment: 73%
- Prevention: 12%
- Diagnosis: 15%
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/comparisons 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.
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
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
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
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.
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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