Accelerating Patient-Centered Outcomes Research and Methodological Research

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In This Session

- PCORI Overview
- Methods Program Overview
- Methods Program Funded Research
- Methods Program Research Funding Opportunities
  - Letter of Intent (LOI)
  - Applications
- Q & A
PCORI CER Methods Program
Improving PCOR Methods

**PCORI’s Mission**

- PCORI was created to support research that provides high-integrity, evidence-based information to patients, clinicians, and the broader healthcare community.

**Why Methods Matter**

- Rigorous research methods are needed to produce relevant, trustworthy findings that can improve patients’ healthcare outcomes.

**Methods for PCOR**

- Methods include systematic processes, designs, tools, and techniques used to generate the evidence needed to answer questions about which healthcare options work best for particular patients.
Methodology Committee Report Creates Standards and Highlights Gaps

- PCORI’s founding legislation created a Methodology Committee charged “to develop and improve the science and methods of comparative clinical effectiveness research” and to produce “methodological standards for research.”

Dr Robin Newhouse, Chair Methodology Committee
### Improving PCOR Methods: Program Goals

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<tr>
<th>Identify Methods Gaps</th>
<th>Fund high impact studies which address gaps in methodological research</th>
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<td>Identify methods gaps relevant to the conduct of PCOR</td>
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<th>Disseminate Promising/Best Practices</th>
<th>Disseminate and facilitate the adoption of new methods to improve the conduct of PCOR</th>
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Improving PCOR Methods: Program Overview

• **Awards:** As of the Spring 2015 Cycle (retroactively known as Cycle 1 2015)
  – 67 funded projects
  – $64.14 million

• **Merit Review:**
  – Typically, we run two panels:
    • Analytic methods
    • Methods of patient & stakeholder engagement
  – Competitive LOI screening process
  – Merit Review criteria for Methods different than other programs

• **Active Portfolio Management:**
  – Ongoing communication with awardees
    • Identify and address challenges
    • Support innovation and opportunities to increase the impact of research
  – Monitoring performance and adherence to the contract
Research Areas of Interest*

1. Methods for Patient and Stakeholder Engagement
2. Methods for Patient-Centered Outcomes (PCOs) and Patient-Reported Outcomes (PROs)
3. Methods for Protection of Human Subjects
4. Methods to Improve Study Design
5. Methods to Improve Validity and Efficiency of Analyses
6. Methods to Support Data Research Networks

PCORI CER Methods Portfolio
CER Methods Program Portfolio
(67 projects as of 4/21/2015)

RESEARCH AREAS OF INTEREST
Patient & stakeholder engagement
General analytic methods
  • Causal inference
  • Heterogeneity of treatment effect
  • Missing data
  • Treatment adherence
Study design-specific methods
Patient-centered & patient-reported outcomes
Human subjects protections
Recruitment & retention in PCOR & CER
Data research networks

PREVIOUS AREAS OF INTEREST
Evidence synthesis
Validity of data sources

Some projects are classified in ≥1 category
A Method for Patient-Centered Enrollment in Comparative Effectiveness Trials: Mathematical Equipoise

Methods

• Creation of a consolidated database from non-RCT sources on knee osteoarthritis (OA) outcomes and creation of predictive models of the outcomes of total knee replacement (TKR) and nonsurgical treatments

Engagement

• A stakeholder panel of patients and providers will meet quarterly and will play a key role in defining situations in which they believe a state of equipoise exists

PCOR Impact Areas

• Heterogeneity of Treatment Effect (HTE)
• Data quality in EHRs and registries
• Research prioritization
• Decision-making process for patients and clinicians

Develops predictive models for determining when a state of mathematical equipoise exists and applies these models to an important clinical treatment question for which there are no previous RCTs: TKR vs. nonsurgical treatment for knee OA. Results will help identify patients for whom, based on their individual characteristics, there is insufficient evidence to favor one treatment.

Harry P. Selker, MD, MPH,
Tufts Medical Center
Boston, MA
Methods for Comparative Effectiveness and Safety Analyses in a High-Dimensional Covariate Space with Few Events

Methods

• Evaluate the analytic strategies through simulation studies and apply analytic methods to three real-world patient-centered outcomes research sample studies

Potential Impact

• Could change PCOR conduct by improving the ability to evaluate treatments soon after they are made available, evaluate treatment effects in patient subgroups, and evaluate treatments for rare diseases

Engagement

• Patients participated in preliminary interviews, and an advisory panel of 10 patients and other stakeholder representatives will meet five times per year to provide feedback

Evaluates and improves analytic strategies for nonrandomized studies with few outcome events and many potential confounders, such as studies of treatment effects in patient subgroups and studies of treatments for rare diseases.

Jessica M. Franklin, PhD, BS, Brigham and Women’s Hospital
Boston, MA

CER Methods and Infrastructure, awarded September 2013
Privacy-preserving analytic and data-sharing methods for clinical and patient-powered data networks

Methods

• Stakeholder focus groups; simulations testing statistical analyses without sharing private data; univariate and multivariate techniques; other privacy-preserving techniques

Potential Impact

• Could help reduce the barriers to conducting multi-center research by promote the utility, performance, timeliness, and efficiency of large clinical and patient-powered research networks for supporting PCOR

Engagement

• Stakeholders and patients will be involved in the design, outcomes, implementation, and dissemination of the project through in-person meetings and as part of the research team

To increase the understanding and utility of privacy-protecting analytic and data-sharing methods in multi-center PCOR studies, and develop a suite of privacy-protecting analytic tools for PCOR networks to perform rigorous analysis without sharing potentially identifiable patient-level information.

Darren Toh
Harvard Pilgrim Healthcare, Inc.
Boston, MA

CER Methods and Infrastructure, awarded September, 2014
Improving Methods for Conducting PCOR: Research Funding Opportunities
Improving Methods for PCOR: Research Funding Overview

**PCORI Funding Announcements (PFA)**
- Three funding cycles for 2015
- Specific to Program Areas, evolving over time

**Letter of Intent**
- Competitive process
  - Cycle 2: LOI due July 29, 2015
  - Cycle 3: LOI due November 12, 2015
- Staff screen LOIs based on the criteria outlined in the Program-specific PFA

**Full Application**
- Only LOIs deemed most responsive to the PFA are invited to submit full applications
- Trained reviewers provide a preliminary online review based on the five Merit Review Criteria
- Subset of applications selected for discussion by in-person review panel based on reviewer scores and critiques
Responsive LOIs

• Significance
  – Identify and explain the specific anticipated contributions and applications of the proposed project to patient-centered outcomes research.

• Programmatic Overlap
  – Differentiate the proposed research from projects already funded by the PCORI Methods Program.

• Study Design or Approach
  – Provide a detailed description of the methodological work that is planned and the specific ways in which it addresses the identified methodological gaps and the specific aims of the proposed project.

• Engagement
  – Describe the plan for engaging patients, caregivers, and/or other relevant stakeholders in the research process, as appropriate.
Non-Responsive LOIs: Types of Research

• Cost-effectiveness Research
  – Conducts a formal cost-effectiveness analysis in the form of dollar-cost per quality adjusted (or nonadjusted) life-year to compare two or more approaches
  – Measures the relative costs of care of two or more alternative approaches as the primary criteria for choosing the preferred alternative

• Non-methodological Research
  – Develops a discrete intervention or healthcare practice
  – Compares the efficacy of two or more health interventions
  – Develops best practices for healthcare delivery

• Narrowly Focused Research
  – Development, refinement, and/or validation of a disease- or condition-specific measure
Non-Responsive LOIs: Methodological Considerations

• Research Question
  – No clear identification of and/or insufficient support for the methodological gap(s) to be addressed

• Clarity and Credibility
  – Lack of detail about the proposed design, data sources or methods of collection, and analytic plan
Competitive Full Applications

• Critical components of strong, high-quality applications
  – Detailed description
    • Specific anticipated contributions of the research
    • Planned methodological work
    • Conduct and execution of the project
  – Identification and examination of key assumptions
  – Plan for evaluation of new methods (including comparison with existing methods, if applicable)
  – Consideration of potential limitations and obstacles
    • Feasibility
    • Implementation
    • Dissemination
Patient and Stakeholder Engagement in Methods Projects

PCORI encourages investigators to engage patients, caregivers, and/or other relevant stakeholders in some or all phases of the project.

• If patient and stakeholder engagement is deemed inappropriate in some or all phases, investigators should justify why.

• Stakeholder examples include: data architects, clinicians, domain experts, health services researchers with different expertise than members of the research team, policy makers, etc.
Questions?
Thank You!

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