

Accelerating Patient-Centered Outcomes Research and Methodological Research

Emily Evans, PhD, MPH
Program Officer, Science

Andrea Heckert, PhD, MPH
Program Officer, Science

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Welcome!



Emily Evans, PhD, MPH
Program Officer, Science



Andrea Heckert, PhD, MPH
Program Officer, Science

In This Session

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



PCORI Comparative Effectiveness Research (CER) Methods Program



PCORI CER Methods Program and Contracts Staff

CER Methods Program Staff



Rachael Fleurence
PhD
Program Director Methods
& Infrastructure



Jason Gerson
PhD
Assoc. Director
Methods



Emily Evans
PhD, MPH
Program Officer
Methods



Maryan Zirkle
MD, MS, MA
Program Officer Methods
& Infrastructure



Andrea Heckert
PhD, MPH
Program Officer
Methods



Jason Rotter
MHS
Program Associate
Methods



Kate McQuestion
MPH
Program Associate
Methods



Kathryn Smith
MSc
Program Assistant
Methods

Post-Award Contracts Staff



Stephen Jones
Post-Award Contracts
Administrator, Contracts



Shayan Hobbi
Contracts Coordinator
Contracts



Improving Patient-Centered Outcomes Research (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.



Improving PCOR Methods: Program Goals

Identify Methods' Gaps

- **Identify** methodological gaps relevant to the conduct of PCOR

Fund Research

- **Fund high-impact studies** which address gaps in methodological research

Disseminate Promising/Best Practices

- **Disseminate** and facilitate the adoption of **new methods** to improve the conduct of PCOR.



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 and 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

** Improving Methods for Conducting PCOR PFA – Cycle 2 2015 (LOI Deadline – July 29, 2015)*



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PCORI CER Methods Portfolio



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CER Methods Program Portfolio

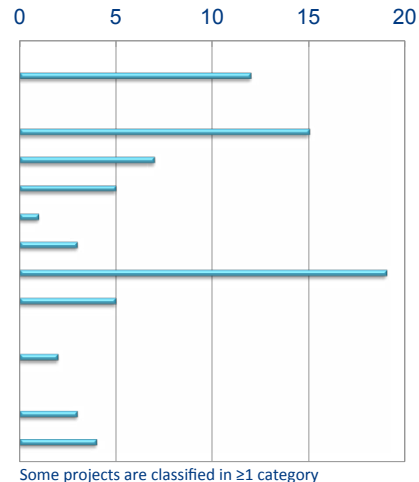
(67 projects as of 4/21/2015)

RESEARCH AREAS OF INTEREST

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

PREVIOUS AREAS OF INTEREST

Evidence synthesis
 Validity of data sources



Some projects are classified in ≥1 category



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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



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Presenting Patient-Reported Outcomes (PRO) Data to Improve Patient and Clinician Understanding and Use

Methods

- Patient and clinician interviews to evaluate their comprehension of PRO data, followed by evaluation of novel approaches for presenting PRO data through online surveys and additional interviews

Engagement

- Stakeholder advisory board of patients, caregivers, clinicians, and PRO developers and researchers to inform study design, conduct, and results dissemination

PCOR Impact Areas

- Usefulness of patient-reported data in clinical care
- PRO score interpretability, with a focus on clinical meaningfulness
- Integrating PROs into clinical practice, including clinician use and acceptance of PRO data



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Develops best practices for presenting PRO data to patients and clinicians to improve their understanding of and ability to use PRO data to promote patient-centered care.

*Claire Snyder, PhD,
Johns Hopkins University
Baltimore, MD*

Improving Methods for Conducting PCOR: Research Funding Opportunities



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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 to PCOR of the proposed project.
- 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



Non-Responsive LOIs: Programmatic Fit

- Overlap with currently funded or concurrent proposed projects
- Other areas of research explicitly identified as non-responsive for the PFA cycle

Merit Review Criteria

BROAD PFA



Criterion #1: *Impact of the condition on the health of individuals and populations*



Criterion #2: *Potential for the study to improve health care and outcomes*



Criterion #3: *Technical merit*



Criterion #4: *Patient-centeredness*



Criterion #5: *Patient and stakeholder engagement*

METHODS PFA



Criterion #1: *Study addresses evidence gaps identified in PCORI Methodology Committee Report or other comparable sources*



Criterion #2: *Potential for the study to improve PCOR methods*



Criterion #3: *Technical merit*



Criterion #4: *Patient-centeredness*



Criterion #5: *Patient and stakeholder engagement*

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 that is the case.
- Stakeholder examples include: data architects, clinicians, domain experts, health services researchers with different expertise than members of the research team, policy makers, etc.



Engagement as a Path to Useful, High-Quality Research



PCOR Engagement Principles

Reciprocal Relationships

- Roles and decision-making authority understood by all

Co-learning

- Patient and stakeholder engagement and research process understood by all

Partnerships

- Time and contributions of patient and stakeholders are valued and demonstrated

Trust, Transparency, Honesty

- Inclusive decision-making, information sharing, open and honest communication, and the sharing of useful findings to communities



Questions?

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Thank You!

Emily Evans, PhD, MPH
Program Officer, Science
eevans@pcori.org

Andrea Heckert, PhD, MPH
Program Officer, Science
aheckert@pcori.org



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