Board of Governors Meeting via Teleconference/Webinar

June 16, 2015
12:00-1:30 p.m. ET
Welcome and Introductions

Grayson Norquist, MD, MSPH
Chair, Board of Governors

Joe Selby, MD, MPH
Executive Director
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<th>Time</th>
<th>Agenda Item</th>
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<tr>
<td>12:00-12:05</td>
<td>Call to Order, Roll Call, and Welcome Consent Agenda</td>
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<tr>
<td>12:05-12:25</td>
<td>Consider for Approval: PCORnet Health Plans PFA Development</td>
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<td>12:25-12:55</td>
<td>Methods Program Overview</td>
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<td>12:55-1:10</td>
<td>Evaluation Update: PCORI Stakeholder Surveys</td>
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<td>1:10-1:20</td>
<td>Methodology Curriculum Update</td>
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<td>1:20-1:30</td>
<td>Annual Meeting Update</td>
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<td>Wrap Up and Adjournment</td>
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Consent Agenda Items

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

Joe Selby, MD, MPH
Executive Director
Motion for Consent Agenda Items

• Approve minutes from May 4, 2015 Board meeting
• Approve the Proposed “Resolutions on Signature Authority on Behalf of PCORI”
  • Under the proposed Resolutions, in addition to Executive Director and Chief Operating Officer, these executive staff would be authorized signatories, subject to limits of Board-approved budget, applicable controls and required approvals:
    • Chief Science Officer
    • Chief Engagement and Dissemination Officer
    • General Counsel
Board Vote

Call for a Motion to:

- Approve each of the Motions on the Consent Agenda, as reflected

Call for the Motion to Be Seconded:

- Second the Motion
  - If further discussion, may propose an Amendment to the Motion or an Alternative Motion

Voice Vote:

- Vote to Approve the Final Motion
  - Ask for votes in favor, opposed, and abstentions
Initiative on Health Plan Data and PCORnet

Rachael Fleurence, PhD
Program Director, CER Methods and Infrastructure
Critical element for intended uses of PCORnet is availability of complete longitudinal data on populations receiving health care within specified CDRNs.

During Phase I, CDRNs worked independently to advance their ability to develop longitudinal data sets.

- Regional efforts included CDRN outreach to health plans and to CMS.

During Phase I, PCORI funded a pilot to explore data linkages between CMS and two CDRNs.

As a supplemental approach, PCORI is seeking to directly fund major US health plans that cover significant numbers of patients in one or more of the PCORnet CDRNs.

In May, a majority of the Research Transformation Committee voted to recommend that the Board approve the development of the PFA.

PCORI staff is proposing that initial PCORI funding focus on up to two health plans.
PCORI would award funds to **up to two major US health plans** that cover significant numbers of patients in one or more of the PCORnet CDRNs.

Each awardee would receive **up to $3 million in direct funds**.

A competitive LOI will allow PCORI to survey the landscape of interested parties.

Intent is for the award(s) to begin in late 2015, aligning closely with the start of Phase II funding for CDRNs and PPRNs, with a **3 year duration**.
Key Funded Activities

- Identifying the extent of overlap between patients in CDRNs (with EHR data) and members in the health plans (with enrollment and claims data)
- Organizing the data on these patients into PCORnet’s Common Data Model
- Developing framework for performing use-case linkages to CDRN data and PPRN data in compliance with applicable privacy, security, and human subject research laws
- Involving health plan leadership in discussions with CDRNs, PPRNs, and funders about legal and organizational options for collaborations
- Participation in specific research studies would not be required or funded. Health plans retain the ability to decide voluntarily about participation in research studies, which would be subject to separate agreements and separate funding
Next Steps

- Today: Request approval from the Board of Governors to develop the PFA
- Summer 2015: PFA released
- Board considers approving up to two awards
Board Vote

Call for a Motion to:
- Approve the development of a PFA to fund up to two Health Plans to collaborate with CDRNs and PPRNs to facilitate access to complete longitudinal data through PCORnet with direct costs not to exceed $6 million.

Call for the Motion to Be Seconded:
- Second the Motion
  - If further discussion, may propose an Amendment to the Motion or an Alternative Motion

Voice Vote:
- Vote to Approve the Final Motion
  - Ask for votes in favor, opposed, and abstentions
Accelerating Patient-Centered Outcomes Research and Methodological Research

Rachael Fleurence, PhD
Program Director, CER Methods and Infrastructure

Jason Gerson, PhD
Associate Director, CER Methods and Infrastructure
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

**Post-Award Contracts Staff**

- Stephen Jones
  Post Award Contracts Administrator, Contracts
- Shayan Hobbi
  Contracts Coordinator

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Andrea Heckert PhD, MPH
Program Officer Methods

Jason Rotter MHS
Program Associate Methods

Kate McQueston MPH
Program Associate Methods

Kathryn Smith MSc
Program Assistant Methods
Improving PCOR Methods

**PCORI’s Mission**
- PCORI helps people make informed healthcare decisions, and improves healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, 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.”

Robin Newhouse, PhD, RN, Chair, Methodology Committee
Perspective

Getting the Methods Right — The Foundation of Patient-Centered Outcomes Research

Sherine E. Gabriel, M.D., and Sharon-Lise T. Normand, Ph.D.

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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 to Date:
- 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 in the Methods PFA

- Methods for Patient and Stakeholder Engagement
- Methods for Patient-Centered Outcomes (PCOs) and Patient-Reported Outcomes (PROs)
- Methods to Improve Study Design (registries, complex interventions, pragmatic trials, adaptive designs)
- Methods to Improve Validity and Efficiency of Analyses
- Methods to Support Data Research Networks
- Methods of Recruitment and Retention of participants into PCOR/CER including randomized trials, observational studies, and registries
- Research on Issues Related to Human Subjects Protections in PCOR/CER
PCORI Methods Program Portfolio
Methods Program Portfolio
(67 projects as of 4/21/2015)

- 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 methods
  - Evidence synthesis
  - Validity of data sources

Some projects are classified in ≥1 category
Sensitivity-Analysis Tools for Clinical Trials with Missing Data

Methods

- Analyze existing randomized controlled trial data that include patient-centered outcomes to demonstrate the utility of the methods for sensitivity analysis.

Potential Impact

- Could change PCOR conduct by improving ability to conduct and report the results of sensitivity analyses of clinical trials with missing data, allowing patients, caregivers, regulators, and policy makers to better judge the robustness of the inferences from these trials.

Engagement

- An advisory panel of relevant stakeholders meets twice a year to provide advice and feedback.

Creates unified and coherent methods for sensitivity analysis of clinical trials with monotone and nonmonotone missing data and develops free, open-source, well-documented, and reproducible software in SAS and R to implement those methods.

Daniel O. Scharfstein, ScD,
Johns Hopkins Bloomberg School of Public Health
Baltimore, MD

Awarded September 2013
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 meets five times per year to provide feedback.

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

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 promoting the utility, performance, timeliness, and efficiency of large clinical and patient-powered research networks for supporting PCOR

Engagement

- Stakeholders and patients are 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, ScD, BPHAR, MS
Harvard Pilgrim Healthcare, Inc.
Boston, MA

Awarded September, 2014
Facilitating Patient-Reported Outcome Measurement for Key Conditions

Methods

- Qualitative methods to construct condition-specific surveys for heart failure (HF) or knee osteoarthritis (OA); quantitative methods to evaluate measurement properties; crosswalks “linking” condition-specific scores to generic scores

Potential Impact

- Could change PCOR conduct by producing a generalizable method that can be broadly implemented to incorporate patient-reported, condition-specific assessments alongside patient-reported generic health assessments

Engagement

- An advisory committee of clinicians and patients with either HF or knee OA meets regularly over 3 years

Develops a novel methodology to incorporate condition-specific concerns important to patients in publicly available generic health assessments from the Patient Report Outcomes Measurement Information System (PROMIS).

Elliott S. Fisher, MD, MPH, Dartmouth College Hanover, NH

Awarded September 2013
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.
Other Methods Program Activities

- Methods Consultation
- Revision of Methodology Standards
- Development of Data and Safety Monitoring Plan Policy
- Program Oversight for PCORI Pilot Projects
- Network Research Methods Work Group
Questions
PCORI Stakeholder Surveys
Patients, Caregivers, Clinicians, & Researchers

Lori Frank, PhD,
Program Director, Evaluation & Analysis

Laura Forsythe, PhD, MPH,
Associate Director, Evaluation & Analysis
Presentation Overview

- Background and Rationale
- Survey Methods
- Selected Findings
- Dissemination Plans
Goals of PCORI Survey Program

• Collect data to inform PCORI’s work
• Measure PCORI impact and progress
• Provide authoritative information on CER/PCOR
In late 2014, PCORI conducted surveys of four stakeholder groups:
Survey Objectives

• To understand attitudes toward CER
• To understand the use of health information in decision making
• To understand awareness of and attitudes toward engagement in research
• To evaluate the potential for engagement in research to enhance the uptake of research results in clinical practice
• To inform methods to facilitate use of CER and the engagement of patients and stakeholders in research
Survey Methods
Survey Development

• Partnership with American Institutes for Research (AIR)
• Guided by an overarching multi-stakeholder advisory panel and four survey-specific working groups
• Survey development included literature review and cognitive testing
• Surveys fielded September 2014 – January 2015
## Survey Samples and Recruitment

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<th>Sample</th>
<th>Methods for Recruitment</th>
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<td><strong>Patients</strong></td>
<td><strong>Rare Conditions</strong>&lt;br&gt;Convenience samples obtained through Genetic Alliance liaising with relevant advocacy organizations to survey their members <em>(N=560 patients, 609 caregivers)</em></td>
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<tr>
<td><strong>Caregivers</strong></td>
<td><strong>Chronic Conditions</strong>&lt;br&gt;Nationally representative samples obtained via probability-based online panels (GfK Knowledge Panel®) <em>(N=762 patients, 776 caregivers)</em></td>
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<td><strong>Clinicians</strong></td>
<td>Nationally representative sample of primary and specialty physicians, physician assistants, and nurse practitioners drawn from the American Medical Association MasterFile <em>(N=638)</em></td>
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<tr>
<td><strong>Researchers</strong></td>
<td>Convenience sample of clinical researchers invited via 23 professional organizations and the PCORI mailing list <em>(N=508)</em></td>
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Selected Findings

Patients, Caregivers, Clinicians

Findings from the Researcher survey were presented Jan 27, 2015:
http://www.pcori.org/events/2015/board-governors-meeting-1
Few patients, caregivers, and clinicians are familiar with the term CER.
Attitudes toward CER

When the concept is explained, nearly all patients, caregivers, and clinicians believe that CER is useful for making treatment decisions.
The top reasons patients sought health information were to understand treatment options and how to manage their condition.
Some clinicians are dissatisfied with the availability of research evidence to inform clinical decisions.
Attitudes toward Engagement in Research

Patients, caregivers and clinicians expressed the belief that engagement would improve the value of research.

Belief that partnering with researchers would improve the value of research

- Patients: Chronic (N=742) - 86%
- Caregivers: Chronic (N=761) - 79%
- Patients: Rare (N=488) - 97%
- Caregivers: Rare (N=605) - 95%
- Clinicians (N=570) - 97%

No
Yes

Patients, caregivers and clinicians expressed the belief that engagement would improve the value of research.
Interest in Engagement

Interest in engagement in research was highest among rare condition patients and rare condition caregivers.
Top factors in determining whether to partner with researchers:

Patients and Caregivers

- Belief that participation can result in meaningful findings
- Feeling respected by the researchers
- Having an interest in the research

Clinicians*

- Helping my patients receive better care
- Contributing to scientific knowledge
- Making research studies more meaningful to patients

*Item asked of a subset of clinicians (N=337)
Patients, caregivers, and clinicians have limited familiarity with the term CER but expect CER findings to be useful for decision making.

Some clinicians are dissatisfied with the availability of research evidence to inform clinical decisions.

Patients seek health information to help understand treatment options and to help manage their condition.

Familiarity with research engagement is limited but interest is high.

Making research more meaningful to patients is an important motivator for patients, caregivers, and clinicians.

Respect and trust in partnerships are important for patients and caregivers.
Dissemination Plans
Sharing Findings, Using Results

Enhancing PCORI’s Programmatic Work:
• Science Oversight Committee’s Application Enhancement Workgroup
• Engagement, Dissemination, and Implementation Committee
• Staff

Informing Our Stakeholders:
• Presentations
  • PCORI Rare Disease Advisory Panel (RDAP)
  • PCORI Annual Meeting
• Webinars
• Publications
Questions
Methodology Standards Academic Curriculum

Bryan Luce, PhD, MBA
Chief Science Officer
Overview of Methodology Standards Dissemination Activities

• Methodology Standards Academic Curriculum
• Baylor College of Medicine CME/CE initiative
• Learning Labs at the PCORI Annual Meeting October 6-8
Academic Curriculum Project

• We have contracted with Johns Hopkins University to develop the academic curriculum for the PCORI Methodology Standards

• Three key deliverables over 6 months:
  1. **Textual Curriculum (month 2-3)**  
     a. Overview -- purpose and overarching themes of Methodology Standards  
     b. Learning objectives for each module (11 standards categories and 2 others)  
     c. Lecture outlines for each module  
  2. **Tool Kit (month 4-5)**  
     Slide sets and audio/video presentations for each module, including learning activities associated with each objective (such as case studies and exercises)  
  3. **Final Report by Fall 2015 (month 6)**  
     Recommended set of strategies and potential venues for the distribution and use of the curriculum and training materials
Target Audiences

- Master/Doctoral level graduate students who are learning to conduct CER/PCOR
- Faculty who teach CER/PCOR
- Researchers and potential PCORI research funding applicants
- Stakeholders who are interested in applying evidence from CER/PCOR
Textual Curriculum: Overview

• Goals and objectives for each standard
  • Cognitive Objectives
  • Attitudinal Objectives
  • Skill Objectives

• Context and rationale for each standard

• Educational strategy to assure that learners meet stated objectives
  • Strategies may be tailored for different types and levels of learners (i.e., emphasis on different learning objectives, etc.)
Textual Curriculum: Case Studies

• Two or more examples from the literature
  • Situations in which authors met the Standard in their research
  • Situations in which the analytical approaches and/or results of the study are difficult to reproduce

• Each case study will have associated guiding questions
Textual Curriculum: Exercises/Self-Assessments

Brief writing tasks to walk the learner through the process of addressing the particular Standard in a research proposal

Box 4. An Exercise

A. Define heterogeneity of treatment effect (HTE)
B. Describe an example, using your clinical knowledge, where HTE is known to be an issue in interpreting the results from trials of a therapy
C. State a hypothesis for a study that explores HTE
D. What is the concern with unspecified post-hoc analyses in explorations of HTE?
Tool Kit

• Each module will have a set of lecture slides with an audio recording of a presentation
• Modules are adaptable to different levels of learners
• Facilitator notes and discussion guide will be included
MC Review Process

- One or two Methodology Committee members will review each module
Timeline

- May – June 2015: Textual Curriculum Resource development
- July – August 2015: Tool Kit development
- September 2015: Final Report
- The curriculum will be updated as existing standards are revised and as new standards are developed
The JHU Team

Figure 1. Investigators committed to this project and their assignments
Questions and Comments?
Update on 2015 PCORI Annual Meeting: Progress in building a Patient-Centered Outcomes Research Community

Bill Silberg,
Director, Communications

Orlando Gonzales, MPA
Chief of Staff, Engagement
The Basics

- Oct. 6-8, 2015 at the Marriott Crystal Gateway, Crystal City, VA.
- Two-and-a-half day conference
- 500 to 700 members of the broad PCORI community
- Mixture of plenary, breakout sessions, “how-to” sessions, networking opportunities and “mini-summits”
- First half-day of joint programming with AHRQ, focused on dissemination and implementation
- Goals include providing a report to the nation on our achievements and plans and hearing from stakeholders on future opportunities
Session Structure

• **Plenaries:**
  - Opening speaker and multi-stakeholder panel on state of CER/PCOR and progress in building a patient-centered research community
  - Lunch speaker/panel focusing on patient engagement

• **Breakout sessions organized by:**
  - Priority/program (up to 16)
  - Cross-cutting themes from portfolio analysis (up to 20)
  - Selected conditions/populations where our work can seek to fill evidence gaps to improve outcomes (up to 20)
Session Structure

- **Learning labs**
  - Short “how-to” sessions offering an opportunity for guiding and instructing awardees and potential awardees and partners to strengthen their proposals

- **Mini-summits**
  - In-depth meetings featuring both “business” sessions and open presentations. Designed to let awardees and others learn about major initiatives areas (e.g. PCORnet, Pilots, Pragmatic Clinical Studies, Engagement, Contracts and Methodology
PCORI/AHRQ/AcademyHealth Joint Session

• **Opening and Welcome:** Joe Selby and Rick Kronick will provide PCORI’s and AHRQ’s roles and collaborative work on D&I

• **Two Moderated Multi-Stakeholder Panels:** Including representatives from journals, health systems, researchers, clinicians, patient/caregiver organizations, and payers
  - **Panel #1:** Landscape of opportunities and challenges in D&I
  - **Panel #2:** Lessons from the field

• **Keynote Presentation** from an expert in behavioral sciences on how to effect change
Opening Plenary: State of PCOR/CER

- Welcome: Gray Norquist, PCORI Board Chairperson
- Opening: Joe Selby, PCORI Executive Director
- Keynote: Victor Montori, MD, Mayo Clinic, Professor of Medicine
- Multi-Stakeholder Panel:
  - Ms. Nancy Brown, CEO, American Heart Association, (patients)
  - Mr. Tony Coelho, Chairman, Partnership to Improve Patient Care (patients)
  - Ms. Helen Darling, Senior Advisor, Past President and CEO, National Business Group on Health (purchasers)
  - Dr. Freda Lewis-Hall, MD, DFAPA, Chief Medical Officer, Pfizer (industry)
  - Dr. Lew Sandy, MD, FACP, Senior Vice President, Clinical Advancement, UnitedHealth Group (payers)
  - Mr. Ken Sharigian, Senior Vice President & Chief Strategy Officer, American Medical Association (clinicians)
  - Dr. Patricia Travis, PhD, RN, CCRP, Board Secretary, American Nurses Association (ANA) (clinicians)
Questions and Discussion
Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Directors