Board of Governors Meeting
via Teleconference/Webinar

May 5, 2014
10:15 a.m. - 5:15 p.m. ET
Welcome and Introductions

Grayson Norquist, MD, MSPH
Chair, Board of Governors

Joe Selby, MD, MPH
Executive Director
<table>
<thead>
<tr>
<th>Time</th>
<th>Agenda Item</th>
</tr>
</thead>
<tbody>
<tr>
<td>10:15 – 10:20 a.m.</td>
<td>Call to Order and Welcome</td>
</tr>
<tr>
<td>10:20 – 11:00 a.m.</td>
<td>Executive Director’s Report</td>
</tr>
<tr>
<td>11:00 – 12:00 p.m.</td>
<td>Update on Science Processes</td>
</tr>
<tr>
<td>12:00 – 1:00 p.m.</td>
<td>Lunch</td>
</tr>
<tr>
<td>1:00 – 1:45 p.m.</td>
<td>Overview of Addressing Disparities Program</td>
</tr>
<tr>
<td>1:45 – 2:15 p.m.</td>
<td>Methodology Committee Update</td>
</tr>
<tr>
<td>2:15 – 2:30 p.m.</td>
<td>Engagement Update</td>
</tr>
<tr>
<td>2:30 – 3:00 p.m.</td>
<td>Evaluation Framework and Plan</td>
</tr>
<tr>
<td>3:00 – 3:15 p.m.</td>
<td>Break</td>
</tr>
<tr>
<td>3:15 – 4:00 p.m.</td>
<td>Mid-year Dashboard Review</td>
</tr>
<tr>
<td>4:00 – 4:30 p.m.</td>
<td>Consider for Approval: Resolution for a cash-secured letter of credit for additional office space lease. Financial Review as of 2/28/2014</td>
</tr>
<tr>
<td>4:30 – 5:00 p.m.</td>
<td>Public Comment</td>
</tr>
<tr>
<td>5:00 – 5:15 p.m.</td>
<td>Wrap Up and Adjourn Meeting</td>
</tr>
</tbody>
</table>
Call for a Motion to:

- Approve April 22, 2014 Board Meeting Minutes

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/Roll Call Vote:

- Vote to Approve the Final Motion
- Ask for votes in favor, opposed, and abstentions
Executive Director’s Report

Joe Selby, MD, MPH, Executive Director
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
PCORnet Structure and Milestones

PCORnet Update

Pragmatic Clinical Studies Update

New Advisory Panels Report

Welcome Jean Slutsky

Review of Today’s Agenda
PCORnet Organizational Structure

PCORnet STEERING COMMITTEE

Members represent:
- Each Clinical Data Research Network
- Each Patient-Powered Research Network
- Patients
- HHS agencies:
  - NIH
  - FDA
  - AHRQ
  - CDC
  - CMS
  - ONC
  - ASPE
- Medical product and device manufacturers
- PCORI and Coordinating Center

PCORnet Executive Committee

11 CLINICAL DATA RESEARCH NETWORKS

18 PATIENT-POWERED RESEARCH NETWORKS

TASK FORCES
- GOVERNANCE
- DATA PRIVACY
- ETHICS & REGULATORY
- DATA STANDARDS & SECURITY
- HEALTH SYSTEMS INTERACTIONS
- PROJECT MANAGEMENT OFFICE
- PATIENT & CONSUMER ENGAGEMENT
- PATIENT GENERATED OUTCOMES
- CLINICAL TRIALS
- RARE DISEASES
- BIOREPOSITORIES
- OBESITY
PCORnet Timeline

3/1/14 Kickoff Meeting

9/30/14 Successful Query of CDM

12/30/14 Phase 2 Announcement

9/30/15 End of Phase 1

7/31/15 Cohorts Completed

12/30/14 Governance Policies Approved

9/30/15 Engagement Plans Implemented
Pragmatic Clinical Studies – Letters of Interest Invited:

Reducing Asthma Morbidity in Highly Impacted Populations
HCV Antiviral Treatments in a Diverse Population with Chronic Hepatitis C
Treatments to Improve Outcome for Opioid Dependence
Novel approaches to reducing CVD risk in vulnerable populations
Smoking Referral/Cessation Interventions in High Prevalence Populations
Personalized pain management in Sickle Cell disease
CE of population-based approaches for preventing diabetes mellitus
CE of approaches to use of colony stimulating factor in cancer
Inpatient vs. outpatient workup of low risk chest pain
Financial and social incentives to enhance medication adherence
CE of approaches to improving functional outcomes after stroke
Management of chronic illness medications during cancer therapy
Lithium vs other therapies for bipolar disorder in adolescents and young adults
Pragmatic Clinical Studies – Letters of Interest Invited:

School-based **caries prevention** to reduce disparities in children’s oral health
Community-based **Depression/Anxiety interventions** to Reduce Disparities

**Radiotherapy alternatives** for common cancers
Monitoring strategies for **follow-up of lung nodules found at screening**
Endovascular repair vs. optimal medical **management of Type B aortic dissection**
Primary care approaches to **preventing progression of acute low back pain**

**Prevention of C-section** in low risk patients
**Drugs Used in treating a very rare disorder**

**Personalized vs. annual mammography screening** for breast cancer
**Statins** for primary prevention of coronary artery disease **after Age 75**
Management strategies for **ductal carcinoma in situ (DCIS)**
**Gene expression testing** vs. traditional risk stratification in **suspected CAD**
Managing **pain and maintaining physical function in osteoarthritis**
**Pulmonary embolism prevention** after hip and knee replacement
**Pragmatic Clinical Studies Timeline**

- **4/7/14** Letter of Interest Invitations
- **8/8/14** Application Deadline
- **11/14** Merit Review
- **1/15** Awards Announced
- **4/15** Earliest Start Date
Initial Focus: Foundational Issues

- Minimal datasets, data standards for rare diseases registries
- Other issues of importance to registries, e.g., ownership of data, legacy issues, sustainability, access to data, Institutional Review Board issues
- Landscape review on rare diseases research issues
- Addressing Institutional Review Board issues related to rare disease research
- Advise on appropriate CER methods and questions for rare diseases

Coordinate with PCORI’s research prioritization Advisory Panels on issues related to rare disease research

Coordinate with PCORnet Task Force on Rare Diseases
Advisory Panel on Clinical Trials–May 1st Recap and Summary

Review PCORI methodology standards related to clinical trials and advise on need for revisions and new standards

Provide guidance for applicants on innovative methods (i.e. adaptive trials and/or Bayesian methods)

In collaboration with the Methodology Committee, provide PCORI with methodologic consultation on the Letter of Interest and solicitation process for Targeted PFAs and Pragmatic Clinical Studies

In collaboration with the Methodology Committee, provide methodological consultation to PCORI and to applicants to enhance the scientific rigor and pragmatic utility of proposed studies

Advise PCORI on developing policies for Data and Safety Monitoring Board oversight and other human subjects issues for Clinical Trials (safety, efficacy, effectiveness)

Discuss ethical assessment of low-risk pragmatic trials (with particular focus on PCORnet settings)
The First GAO 5-Year Review of PCORI Has Begun

- Notification letter from GAO in March
- It identifies two primary objectives for the review, to determine:
  - To what extent has PCORI established research priorities and funded research in accordance with its legislative requirements?
  - To what extent has PCORI established plans and undertaken efforts to evaluate the effectiveness of its work?
- Entrance Conference with the GAO Team in April
- GAO will return for a series of meetings with PCORI staff in May
- GAO’s report to Congress is due in March of 2015
Jean Slutsky, PA, MPH

- Chief Engagement and Dissemination Officer
- Program Director, Communications and Dissemination Research
Preview of Today’s Agenda

**Produce Useful Research**

Monitor Performance:
- 2nd Quarter Dashboard
- Report on Addressing Disparities Portfolio
- 5-month Finance Review

Advise:
- Ensuring Adherence to Methodology Standards
- Selecting Awards
- Selecting Priority Topics
- Methodology Update

Strategy:
- PCORI Evaluation Plan
- Engagement Update

Board of Governors Meeting, May 5, 2014
Science Process Update:
Implementing Methodology Standards
Award Selection
Topic Selection

Bryan Luce, PhD, Chief Science Officer
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Meeting Agenda and Topics
What is being discussed today?

- Review timeline, progress, and plans
- Update on process improvement review
- Present final award selection process
- Update on process improvement review

Report on implementing Methodology Standards
Award Selection
Report on progress of topic selection
Implementing the PCORI Methodology Standards

Bryan Luce, PhD, Chief Science Officer
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Session Topics and Objectives
What are we going to cover today?

Overview of Timeline
• Review the timeline for past and future activities related to implementing the Methodology Standards

Adherence to the Standards
• Review the process for monitoring adherence to the Methodology Standards

Training
• Discuss key training initiatives in place or underway
Timeline for Implementing Methodology Standards

- **Prior to July 2013**: Methodology Standards *recommended* for all projects (Cycles I-III)
  - Application Guidelines, Funding Announcements, Merit Reviewer Guidance

- **August 2013 Cycle**: Adherence *required* for all projects
  - Application Guidelines, Funding Announcements, Contract Language

- **December 2013**: Instituted process for pre/post-award adherence monitoring

- **April 2014**: Developed Pre-/Post-Award Adherence process for staff
Pre/Post Award Requirements

**Pre-Award:** Adherence reviewed and required prior to contract activation
- Checklist used by staff to track adherence
- Research template modified to highlight standards through the application

**Post-Award:** Adherence monitored through active portfolio management
- Milestone schedule at six-month or year-one interim report and final report includes progress on methodologic adherence
## Methodology Standards Checklist (excerpt)

<table>
<thead>
<tr>
<th>Standard Category</th>
<th>Abbrev.</th>
<th>Standard</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>RQ-1</td>
<td>Identify Gaps in Evidence</td>
</tr>
<tr>
<td></td>
<td>RQ-2</td>
<td>Develop a Formal Study Protocol</td>
</tr>
<tr>
<td></td>
<td>RQ-3</td>
<td>Identify Specific Populations and Health Decision(s) Affected by the Research</td>
</tr>
<tr>
<td></td>
<td>RQ-4</td>
<td>Identify and Assess Participant Subgroups</td>
</tr>
<tr>
<td></td>
<td>RQ-5</td>
<td>Select Appropriate Interventions and Comparators</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Yes</td>
<td>No (but plan to comply should have been provided in application)</td>
<td>Address in Milestones Interim Report</td>
<td>Could address the development of a protocol, but would not be complete at time of application.</td>
</tr>
</tbody>
</table>
A. Background
- Describe the impact of the condition on the health of individuals and populations. (Criterion 1)
- Identify gaps in evidence. (RQ-1)

B. Significance
- Describe how the specific aims (see below) relate to the significance of the proposed research study.
- Describe the potential for the study to improve healthcare and outcomes. (Criterion 2)
- Describe the study’s patient-centeredness. (Criterion 4)

C. Study Design or Approach
- Describe the research strategy or methodological approach.
- State the specific aims.
- Demonstrate the study’s technical merit. (Criterion 3)
  - Within this section, describe adherence to methodology standards and:
    1. Describe the plan for developing a formal study protocol. (RQ-2)
    2. Describe how you will identify, select, recruit, and retain study participants representative of the spectrum of the population of interest and ensure that data are collected thoroughly and systematically from all study participants. (PC-2)
    3. Describe how you will or have selected appropriate interventions and comparators. (RQ-5)
    4. Describe the outcomes that people representing the population of interest notice and care about. (RQ-6)
    5. Describe your use of Patient-Reported Outcomes when patients or people at risk of a condition are the best source of information. (PC-3)
    6. Describe a priori specific plans for data analysis that correspond to major aims. (IR-3)
Adherence to Methodology Standards

August 2014 Cycle: all 53 projects (awarded in December 2013) were reviewed using this adherence review process.

All methodological issues requiring modification were shared with principal investigator, resolved, and added to milestone schedules.

Next Steps:
- Staff developing a plan for reviewing projects funded in Cycles I-III
- Training program being instituted
Methodology Standards Training

- Working with scientific expert on developing content and curriculum

Schedule for Training Modules:
- Patient/Stakeholder Reviewers (training: March 2014)
- Staff (May 2014)
- Technical Reviewers (July 2014)
- Prospective applicants (September 2014)
- Future in-person training conferences (planned)
Questions?
Award Selection Process

Bryan Luce, PhD, Chief Science Officer
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
PCORI PFA and Award Cycle

Board of Governors Funding Slate Review
- Recommended funding slates and rationale presented to the Board for review and approval
- Funded awards announced to public

PFA Development
- Research Topic Database reviewed by Programs
- Program staff prioritize topics
- Additional evidence collected, shared with Advisory Panels
- Advisory Panels rank topics
- Program staff provide Board with rationale for topic funding
- Board reviews research topics
- Approved topics incorporated in funding announcement

Letter of Intent Receipt
- Review of budget requests
- May have a screen/invite process during which program staff evaluate LOIs for programmatic fit

Full Application Receipt
- PFA responsiveness review
- Referral of applications to review panels
- Reviewer recruitment
- COI/Expertise indication by reviewers

Application Assignment to Reviewers
- Reviewer training

Application Review
- Evaluation of adherence to methodology standards
- Evaluation using merit review criteria
- Critiques submitted
- Score reports sent to programs

Programs Set Discussion Line
- Identifies applications that will move forward to be discussed at the in-person meeting

In-Person Meeting
- Applications discussed and scored
- Chair records discussion summaries
- SROs take discussion notes

Selection Committee Meeting
- Programs present funding slates to the Selection Committee
- Selection Committee reviews slates, merit review scores, and recommends slates for approval

Summary Statement Production
- SROs use Chair summaries, notes to write discussion summaries
- Programs review discussion notes
- Contracts produces summary statements

Funding Slates Set for Programs
- Score reports and portfolio balance part of consideration when proposing the funding slates

Overview

Board of Governors Meeting, May 5, 2014 29
Establishing Funding Slate

- Top scoring applications reviewed by Program Directors.
- Initial funding line set based on available program funding and application budgets.
- Applications reviewed for possible budget adjustments.
- Staff recommend slate based on merit scores and exception criteria.
- Slate submitted to Selection Committee for review and approval.
- Selection Committee brings slate to Board for final approval.
Exceptions Criteria

- Fit with programmatic vision as outlined in each Program Strategic Framework
- Potential for synergy within the portfolio
- Avoiding duplication in project aims within the portfolio
- Possible adjustment to address between-panel scoring differences
Guidelines for Recommending Projects Using Other Considerations

In addition to Merit Review Scores, projects are discussed based on exception criteria.

Selection Committee members will review abstracts and summary statements of all recommended projects based on considerations other than merit review scores alone and may request entire application.

- Selection Committee members include members of the Board and Methodology Committee.

Note: Award selection process was reviewed by the Science Oversight Committee and all recommendations were incorporated.
Questions?
Topic Selection Update

Bryan Luce, PhD, Chief Science Officer
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Brief Background and Status

- Board has indicated interest in reviewing the topic selection process and its role
- Science Oversight Committee (SOC): “engage a process improvement expert”
  - Completed in April
  - SOC has been briefed
  - Staff has vetted recommendations, revised processes
- Revised draft processes submitted to SOC
  - To be discussed at tomorrow’s Committee meeting
Process Improvement Steps by Expert Consultant Group

- Reviewed all existing policies and procedures
- Interviewed Board members and Science staff
- Depicted detailed map of entire topic capture and prioritization process
- Special consideration of Board role in the process
- Recommended improvements
Eight Topic Capture and Research Prioritization Processes

1. Engaging stakeholders
2. Monitoring for new opportunities (i.e., gap analysis, discussion with other funders)
3. Developing individual Program strategic frameworks
4. Capturing topics and database management
5. Vetting and prioritization topics
6. Developing topic briefs/landscape reviews
7. Prioritizing topics by Program advisory panels
8. Refining research questions and developing funding announcements
Two Key Issues to Discuss with SOC

- Timing and nature of SOC/Board involvement
- Extent of information (e.g., topic brief, full landscape review, systematic evidence review) appropriate for SOC/Board review
Questions?
LUNCH

Join the conversation on Twitter via #PCORI
Addressing Disparities
Program Portfolio

Romana Hasnain-Wynia, PhD
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Addressing Disparities Program Background
- Program mission and goals
- Program progress to date

Conceptual Framework and Driver Model
- Addressing Disparities program portfolio
- Disparities projects across all PCORI programs

Next Steps

Discussion
Addressing Disparities Program Staff

Romana Hasnain-Wynia, MS, PhD  
Program Director

Cathy Gurgol, MS  
Program Officer

Ayodola Anise, MHS  
Program Officer

Katie Lewis, MPH  
Program Associate

Mychal Weinert  
Program Associate

Tomica Singleton  
Senior Administrative Assistant
The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis, .... and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services.....

-- from Patient Protection and Affordable Care Act
IDENTIFYING RESEARCH PRIORITIES.

The Institute shall identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care…

-- from Patient Protection and Affordable Care Act
Program’s Mission Statement

To reduce disparities in healthcare outcomes and advance equity in health and healthcare

Program’s Guiding Principle

To support comparative effectiveness research that will identify best options for eliminating disparities
Addressing Disparities: Program Goals

Identify Research Questions
- Identify high-priority research questions relevant to reducing and eliminating disparities in healthcare outcomes

Fund Research
- Fund comparative effectiveness research with the highest potential to reduce and eliminate healthcare disparities

Disseminate Promising/Best Practices
- Disseminate and facilitate the adoption of promising/best practices to reduce and eliminate healthcare disparities
Progress toward Goal (2012-2015)

**Broad PFAs**
- 4 cycles
- 31 comparative effectiveness research projects totaling $52.8M

**Targeted PFAs**
- 1 cycle
- Treatment Options for Uncontrolled Asthma: 8 comparative effectiveness research projects totaling $23.2M

**Pipeline for Targeted PFAs**
- Obesity treatment options in primary care, awards in August 2014 (Will fund up to 2 awards totaling $20 M)
- Pragmatic clinical trials, awards in January 2015
- In Development Stage (Hypertension, Perinatal, Lower Limb Amputations)
Addressing Disparities Comparative Effectiveness Research Portfolio Snapshot

Research Areas

Chronic conditions 36%
Mental Health 18%
Cancer 8%
Reproductive Health 5%
Care Systems 5%
Infectious Disease 5%
Disabilities 2%
Injury/trauma 2%
Neurologic 3%
Alcohol/drug 3%
Infectious Disease 5%
Disabilities 2%
Injury/trauma 2%
Neurologic 3%
Alcohol/drug 3%

Chronic Conditions Portfolio

Chronic pain, 2
CVD, 2
Asthma, 8
Multiple conditions, 4
Diabetes, 1
COPD, 1
Respiratory illness, 1

AD Portfolio

Patient-Centered Outcomes Research Institute
Addressing Disparities Comparative Effectiveness Research Portfolio Snapshot

Disparities Population (not mutually exclusive)

<table>
<thead>
<tr>
<th>Population</th>
<th># of Projects</th>
</tr>
</thead>
<tbody>
<tr>
<td>Racial/ethnic...</td>
<td>31</td>
</tr>
<tr>
<td>Low income</td>
<td>25</td>
</tr>
<tr>
<td>Rural</td>
<td>8</td>
</tr>
<tr>
<td>Low literacy</td>
<td>8</td>
</tr>
<tr>
<td>Disabilities</td>
<td>2</td>
</tr>
<tr>
<td>LGBT</td>
<td>2</td>
</tr>
</tbody>
</table>

AD  PCORI-wide (excluding AD)

Board of Governors Meeting, May 5, 2014
Addressing Disparities Comparative Effectiveness Research Portfolio Snapshot

Research Methods: Study Design

### Study Design

<table>
<thead>
<tr>
<th>Study Design</th>
<th># of Projects</th>
</tr>
</thead>
<tbody>
<tr>
<td>RCT</td>
<td>35</td>
</tr>
<tr>
<td>Non-RCT</td>
<td>5</td>
</tr>
</tbody>
</table>

Quasi-experimental and Observational studies are not shown in the graph.
Long-Term Outcomes of Community Engagement to Address Depression Outcomes Disparities

Engagement

- Community agencies collaborate to tailor depression toolkits to the needs and strengths of community

Potential Impact

- Could change practice by providing information about how depressed patients prioritize outcomes and make decisions, and could affect practice by showing how clinicians respond to patients’ preferences

Methods

- Mixed methods approach and a randomized controlled trial

Looks at long-term patient outcomes of community engagement intervention vs. a technical assistance model, identifies patient preferences and priorities for outcomes, and assesses community capacity to respond to these priorities.

Kenneth Wells, MD, MPH, University of California, Los Angeles
Los Angeles, CA

Addressing Disparities Research Project, awarded December 2012
Reducing Health Disparities in Appalachians with Multiple Cardiovascular Disease Risk Factors

Engagement

• Patient focus groups will inform intervention design, and community members will recruit participants and educate patients

Potential Impact

• Could change practice by providing needed patient-centered cardiovascular disease (CVD) risk reduction to a major at-risk population living in an environment where CVD risk reduction is difficult

Methods

• Randomized controlled trial

Compares two approaches to reducing cardiovascular Disease risks among at-risk adults in Appalachian Kentucky. Study will look at referral to primary care physician) vs. HeartHealth, a patient-centered, self-care intervention.

Debra Kay Moser, DNSc, RN, University of Kentucky
Lexington, KY

Addressing Disparities Research Project, awarded December 2012
Rural Options at Discharge Model of Active Planning

Engagement

- Patient and provider surveys, interviews, and forums will inform design, evaluation, and dissemination of discharge tool, and Innovation Design Team, composed of all stakeholder groups, will guide study

Potential Impact

- Could change practice by reducing re-hospitalization by as much as 30%, and improving patient recovery and return to active life

Methods

- Quasi-experimental mixed methods

Designs rural options for a discharge model that improves patient outcomes and reduces disparities for rural patients. Rural Options at Discharge Model of Active Planning (ROADMAP) will be tested against the current standard discharge methods.

Tom Seekins, PhD, University of Montana Missoula, MT

Addressing Disparities Research Project, awarded May 2013
Engagement

- Patient participation informs the design and specific aims of the study, and qualitative data collection from families and clinicians examines access to care from additional perspectives.

Potential Impact

- Could change practice by providing information to patients with disabilities on how they can best access care and stay healthier.

Methods

- Secondary data analysis complemented by mixed methods.

Investigates why it is more difficult for people with disabilities to get care, with the main goal of developing the Patient-Inspired Surveillance Tool to provide guidance on addressing disparities in care.

Margaret Stineman, MD, University of Pennsylvania Philadelphia, PA

Addressing Disparities Research Project, awarded May 2013
Conceptual Framework and Driver Model
Conceptual Framework: Designing and Evaluating Interventions to Eliminate Disparities in Health Care

- **Barriers**
  - Personal/Family: acceptability, cultural, language/literacy, attitudes, beliefs, preferences, involvement in care, health behavior, education/income
  - Structural: availability, appointments, how organized, transportation
  - Financial: insurance coverage, reimbursement levels, public support

- **Use of Services**
  - Visits: primary care, specialty, emergency
  - Procedures: preventive, diagnostic, therapeutic

- **Mediators**
  - Quality of providers: cultural competence, communication skills, medical knowledge, technical skills, bias/stereotyping
  - Appropriateness of care
  - Efficacy of treatment
  - Patient adherence

- **Outcomes**
  - Health Status: mortality, morbidity, well-being, functioning
  - Equity of Services
  - Patient Views of Care: experiences, satisfaction, effective partnership

Cooper, et al. Journal of General Internal Medicine
Addressing Disparities Program: Comparative Effectiveness Research Driver Model

Tertiary Drivers
- Self-Management
- Community Health Workers
- Cultural/Language Tailoring
- Decision Support
- Family/Caregiver Involvement
- Team-Based Care
- Social Support
- Developmental

Secondary Drivers
- Access to Care
- Training/Education
- Workforce
- Patient Empowerment
- Technology
- Community/Home Environment

Primary Drivers
- Policy
- Organizational
- Point of Care/Communication

Program Goal
Reduce/Eliminate Disparities in Health Care Outcomes

Board of Governors Meeting, May 5, 2014
The model is an *evolving* tool used to

- Evaluate where we are
- Identify where we need to go
Targeted Funding Announcement: Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma

- 8 Awards, $24 million

Projects

- Compare interventions to improve clinician and patient adherence to NHBLI guidelines by
  - Enhancing provider and patient communication (e.g., use of mobile technology)
  - Improving systems of care (e.g., evaluate models that look at data integration) and/or
  - Improving integration of care (e.g., team-based care)

- Include patient-centered outcomes
- Have strong stakeholder engagement
## Addressing Disparities Project: Mapping Example 1

<table>
<thead>
<tr>
<th>Project Title</th>
<th>Guidelines to Practice: Reducing Asthma Health Disparities through Guideline Implementation</th>
</tr>
</thead>
<tbody>
<tr>
<td>PI</td>
<td>James W. Krieger</td>
</tr>
<tr>
<td>Organization</td>
<td>Seattle-King County Public Health</td>
</tr>
<tr>
<td>State</td>
<td>Washington</td>
</tr>
<tr>
<td>Project Description</td>
<td>Compares the relative impact of multi-component, multi-systems interventions that aim to improve asthma outcomes among patients at community health centers in Seattle. Three-arm study includes: 1) health plan intervention plus provider education, 2) CHW home visits + self-management support, and 3) enhanced clinic care with decision support.</td>
</tr>
</tbody>
</table>
Addressing Disparities Driver Model: Mapping Example

Tertiary Drivers
- Self-Management
- Community Health Workers
- Cultural/Language Tailoring
- Decision Support
- Family/Caregiver Involvement
- Team-Based Care
- Social Support
- Developmental

Secondary Drivers
- Access to Care
- Training/Education
- Workforce
- Patient Empowerment
- Technology
- Community/Home Environment

Primary Drivers
- Policy
- Organizational
- Point of Care/Communication

Program Goal
- Reduce/Eliminate Disparities in Health Care Outcomes
Addressing Disparities Driver Model: Mapping Example

Tertiary Drivers
- Self-Management
- Community Health Workers
- Cultural/Language Tailoring
- Decision Support
- Family/Caregiver Involvement
- Team-Based Care
- Social Support
- Developmental

Secondary Drivers
- Access to Care
- Training/Education
- Workforce
- Patient Empowerment
- Technology
- Community/Home Environment

Primary Drivers
- Policy
- Organizational
- Point of Care/Communication

Program Goal
Reduce/Eliminate Disparities in Health Care Outcomes
All Addressing Disparities Projects Mapped to Driver Model
Addressing Disparities Program: Comparative Effectiveness Research Driver Model (n=39*)

**Primary Drivers**
- Patient Empowerment (24)
- Technology (14)
- Community/Home Environment (14)

**Organizational**
- Patient Empowerment (21)

**Point of Care/Communication**
- Access to Care (27)

**Secondary Drivers**
- Training/Education (32)
- Workforce (9)

**Tertiary Drivers**
- Self-Management (19)
- Community Health Workers (17)
- Cultural/Language Tailoring (16)
- Decision Support (9)
- Family/Caregiver Involvement (7)
- Team-Based Care (6)
- Social Support (7)
- Developmental (4)

**Program Goal**
Reduce/Eliminate Disparities in Health Care Outcomes

*Categories are not mutually exclusive. There can be a maximum of 39 projects in each category.
Addressing Disparities Program: Comparative Effectiveness Research Driver Model (n=39*)

**Tertiary Drivers**
- Self-Management
- Community Health Workers
- Cultural/Language Tailoring
- Decision Support
- Family/Caregiver Involvement
- Team-Based Care
- Social Support
- Developmental

**Secondary Drivers**
- Access to Care
- Training/Education
- Workforce
- Patient Empowerment
- Technology
- Community/Home Environment

**Primary Drivers**
- Policy
- Organizational
- Point of Care/Communication

**Program Goal**
Reduce/Eliminate Disparities in Health Care Outcomes

*Categories are not mutually exclusive. There can be a maximum of 39 projects in each category.*
### Number of Projects that Include Underserved Populations across PCORI Portfolio

<table>
<thead>
<tr>
<th>Program</th>
<th># of Disparities Projects</th>
<th>Total # of Projects in Program</th>
<th>% of Portfolio Looking at Disparities</th>
</tr>
</thead>
<tbody>
<tr>
<td>Addressing Disparities</td>
<td>39</td>
<td>39</td>
<td>100%</td>
</tr>
<tr>
<td>Improving Healthcare Systems</td>
<td>31</td>
<td>41</td>
<td>76%</td>
</tr>
<tr>
<td>Assessment of Prevention, Diagnosis and Treatment Options</td>
<td>31</td>
<td>65</td>
<td>48%</td>
</tr>
<tr>
<td>Communication and Dissemination Research</td>
<td>12</td>
<td>25</td>
<td>48%</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>113</strong></td>
<td><strong>170</strong></td>
<td><strong>66%</strong></td>
</tr>
</tbody>
</table>
Engage patients and other end-users from start to finish (i.e., research topic generation → dissemination)

Establish *learning communities* among PCORI-funded projects

- Mechanism to foster cross-learning among project teams
- Bring together the research community and end-users including patients, payers (e.g., AHIP), employers and purchasers (e.g., National Business Group on Health), clinicians, professional societies, policy makers, and training institutions
Where Do We Go From Here?
Learning, Disseminating, and Implementing

- Continue to develop a more sophisticated understanding of the practices that are effective in reducing/eliminating disparities in care

- Produce a strong evidence base of promising/best practices for disparities reduction strategies

- Disseminate promising/best practices in partnership with key stakeholders
Addressing Disparities Program

Questions and Discussion
Methodology Committee Update

Robin Newhouse, PhD, RN, NEA-BC, FAAN, Chair of the Methodology Committee
David Hickam, MD, MPH, Director of Clinical Effectiveness Research
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
### Session Topics and Objectives

**What are we going to cover today?**

| Methodology Standards | • Update on new standards development  
<table>
<thead>
<tr>
<th></th>
<th>• Public input process for new standards</th>
</tr>
</thead>
<tbody>
<tr>
<td>PCORnet Methods Task Force</td>
<td>• New task force to consider methods questions for PCORnet</td>
</tr>
<tr>
<td>Open Science Work Group</td>
<td>• Update on development of an open science framework</td>
</tr>
<tr>
<td>Decision Sciences Initiative</td>
<td>• Update on planning for a workshop to discuss research opportunities in the field</td>
</tr>
</tbody>
</table>
| Member Updates | • Update on GAO Solicitation for new members  
|                       | • Recognition of Al Berg and John Ioannidis |
Methodology Standards

The Methodology Committee is developing two sets of new standards in the areas of Research Designs Using Clusters and Complex Interventions.

- Draft standards will be made available for public comment for a period of 45-60 days, per the requirements of the legislation.

The Committee has also added a public solicitation feature to the website for recommendations for new standards.

A task group has formed to address methods issues and gaps for PCORnet.

Membership includes Methodology Committee members, members of the PCORnet Coordinating Center, and PCORI staff.

- Methodology Committee member Sebastian Schneeweiss is the liaison.
Open Science Work Group

- A work group, composed of Board and Methodology Committee members, has formed to develop a PCORI policy for open science and reproducible research.

- The group will work under the oversight of the Research Transformation Committee and in collaboration with the Methodology Committee.

- Steve Goodman, Methodology Committee vice-chair, and Harlan Krumholz are the leaders of the group.
Methodology Committee members and PCORI staff are working on several activities to explore research opportunities in the field of decision sciences.

The group has developed a framework that encompasses the domains to be considered.

Currently, the group is in the planning stages for a convening of experts to gather information.
Member Updates

The GAO opened a request for nominations to fill four seats on the Methodology Committee
- The Methodology Committee submitted a formal response and recommendation for new members to the GAO
- The request was live from March 14th – April 11th, 2014

On behalf of the Methodology Committee, the Board, and PCORI staff, we recognize the work and contributions of Committee members
- Al Berg
- John Ioannidis
Update on Engagement

Jean Slutsky, PA, MSPH, Chief Engagement and Dissemination Officer
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Quick Update on Engagement

Jean named Chief Engagement and Dissemination Officer March 17, 2014

Working in collaboration with PCORI’s Chief Science Officer, planning and activities have started to integrate Science, Engagement, Communications, and Dissemination more closely

The first Engagement Officer has been hired

Offer made for the Director of the Eugene Washington PCORI Engagement Awards Program

Initiated development of instructions for small to large conference award applications under the Engagement Awards Program

Patient and Family Engagement Rubric has been completed and plans are underway to incorporate it into future funding announcements and provide training for Merit Reviewers and potential PCORI applicants
Evaluation at PCORI

Michele Orza, ScD, Senior Advisor to the Executive Director
PCORI Board of Governors Meeting
Alexandria, VA
May 2014
Objectives

The objectives of this presentation are to provide:

- An introduction to Evaluation at PCORI
- A progress report on some core activities
- A guide to where to find further information and opportunities to provide input
Our Current Tasks

Building our *Evaluation Framework*, that is, delineating, organizing, and prioritizing the questions that staff and stakeholders have about PCORI’s work and about PCOR in general

Determining how we can measure some of the elements that we know already will be central to answering many of the questions, especially our *three goals and Engagement in Research*

Undertaking many evaluation activities that have clearly emerged as high priorities, such as surveys and *assessing the impact of Engagement on the early phases of research*
What Is Our Evaluation Framework?

Our framework organizes our evaluation questions and describes how we will answer them

<table>
<thead>
<tr>
<th>Evaluation Questions</th>
<th>Metrics/Indicators</th>
<th>Methods</th>
<th>Sources</th>
</tr>
</thead>
<tbody>
<tr>
<td>What do PCORI staff and stakeholders want/need to know about PCOR and PCORI?</td>
<td>For each evaluation question, what are we measuring and how will we measure it?</td>
<td>What approach will we take to answering each evaluation question?</td>
<td>From where will we get the data to answer each evaluation question?</td>
</tr>
</tbody>
</table>
Our Evaluation Framework Includes Three Kinds of Questions

1. What are we doing?
   Are we doing it efficiently and effectively?
   Are we on track?

2. Are we accomplishing our goals?
   Producing useful information?
   Speeding its uptake?
   Influencing research?

3. How do the various components of PCORI’s approach contribute to reaching its goals and achieving its mission?
1: Questions about Our Day-to-Day Work

1. What are we doing?
   - Are we doing it efficiently and effectively?
   - Are we on track?

2. Are we accomplishing our goals?
   - Producing useful information?
   - Speeding its uptake?
   - Influencing research?

3. How do the various components of PCORI's approach contribute to reaching its goals and achieving its mission?
2: Questions about Our Goals

1. What are we doing?
   Are we doing it efficiently and effectively?
   Are we on track?

2. Are we accomplishing our goals?
   Producing useful information?
   Speeding its uptake?
   Influencing research?

3. How do the various components of PCORI’s approach contribute to reaching its goals and achieving its mission?
First Goal: Useful Information

Substantially increase the quantity, quality, and timeliness of *useful, trustworthy* information available to support health decisions.
We are developing criteria to assess the potential usefulness of the information we produce:

**Rationale/User-Driven**
- People who would use the information have been identified
- Specific uses for the information have been identified
- People who would use the information are asking the question

**Research Question/User-Focused**
- Study compares options that are relevant for the people who would use the information
- Study assesses the outcome(s) that matter for the people who would use the information

**Real-world Use**
- Results could provide a clear answer to the question
- Results could be tailored to individuals or subgroups
- Results could be scaled/spread beyond the study setting

We are refining these criteria, cross-walking them with our other criteria (such as for Merit Review), and pilot testing them, and will soon apply them to our portfolio of funded studies.
Usefulness: What could we say about it in 2014?

- How our portfolio stacks up against our criteria for potential usefulness and
  - How applications we funded compare to those we didn’t
  - How studies funded in earlier cycles compare to later ones
  - How studies funded under Broad PFAs compare to Targeted
  - How our portfolio compares to others on these criteria

- What patients and stakeholders think about the potential usefulness of our portfolio
  - For example, what concerned patients and relevant stakeholders think about the cluster of asthma, pediatric, or mental health studies we are funding
Assessing Potential and Actual Usefulness

How do the studies we fund measure on usefulness criteria?

Do people find information from PCORI studies useful?

Is the information from PCORI studies being used? By whom? How?

Refine criteria and incorporate into funding decisions
Second Goal: Use of PCORI Information

Speed the implementation and use of patient-centered outcomes research evidence
Draft Measures of Use

**Dissemination**
(Measure for all PCORI funded studies)

- Results reported back to study participants
- Access to PCORI study report
- Presentations:
  - Scientific/professional audiences
  - Lay audiences
- Bibliometrics:
  - # of Publications
  - Time to publication
  - Impact factor
  - Citations
- Alternative metrics for key groups (patients, clinicians, payers, etc.):
  - # manuscript downloads
  - # manuscript bookmarks
  - Media coverage
  - Social media coverage

**Uptake and Use**
(Measure for a subset of PCORI funded studies)

- Adoption of study findings in the study setting
- Incorporation into:
  - Systematic reviews
  - Patient and consumer education materials
  - Graduate Medical Education (GME) or Continuing Medical Education (CME)
  - Practice guidelines
  - Decision making infrastructure (e.g. electronic decision aids, clinical reference tools)
  - Payer policies
  - Institutional, local, state, and national policy

**Impact: Changes in Health Decisions or Care**
(Measure for small set of exemplar studies)

- Change in health decisions or health care among key groups (patients, clinicians, payers, etc.)

**Note:** Most of these metrics are typically not measurable until after study completion, and in many cases, are typically not measurable until several years after study completion.
Third Goal: Influence Research

Influence clinical and health care research funded by others to be more *patient-centered*
Draft Measures of Influence

We are now or soon will be measuring:

- Endorsement, Promotion, and Dissemination of PCORI work
- Use of PCORI Methodology Standards for Patient-Centeredness
- Use of PCORI approaches:
  - Topic Generation and Research Prioritization
  - Merit Review
  - Engagement
  - Communication and Dissemination
- Use of PCORI guidance re: Patient-Centered CER
- Use of PCORI-supported curricula or training
- Collaborations/Co-funding with other funders

We will have to wait a few more years to measure:

- Use of PCOR Methods evidence
- Use and support of PCORnet
3: Questions about Our Approach

What Difference Does “Research Done Differently” Make?

1. What are we doing?
   - Are we doing it efficiently and effectively?
   - Are we on track?

2. Are we accomplishing our goals?
   - Producing useful information?
   - Speeding its uptake?
   - Influencing research?

3. How do the various components of PCORI’s approach contribute to reaching its goals and achieving its mission?
Engagement in Research is the focus of an entire section, and every section also has questions about Engagement, the main difference in PCORI’s approach.
What Difference Does Engagement Make?

What are we looking at now? Some examples:

- **Merit Review**
  - What is the effect of the following on our portfolio of funded studies?
    - Stakeholder *engagement* in review
    - Our Merit review *criteria*
    - Our review *processes*
  - What is the *experience of patients and stakeholders* who participate?

- **Engagement in Research**
  - What is the effect of patient and stakeholder engagement:
    - On the functioning of the *study team*?
    - On study *design*?
    - On *recruitment and retention*?
Impact of Engagement ENgagement ACTivity Inventory (ENACT)

We have developed a self report tool to measure and describe **Engagement** in our funded research studies.

We have developed versions for:
- PCORI Pilot Projects
- CER studies
- PCORnet projects (to describe engagement of patients and other stakeholders in network development)
## Impact of Engagement: Development of the ENgagement ACTivity (ENACT) Inventory

<table>
<thead>
<tr>
<th>Spring 2013</th>
<th>July 2013</th>
<th>Fall 2013</th>
<th>Jan 2014</th>
<th>Fall 2013 to present</th>
</tr>
</thead>
</table>
| • Initial engagement measurement tool developed by PCORI & Academy Health | • Tool fielded with Pilot Project principal investigators | • Patient and Family Engagement Rubric developed | • Webinar with Pilot Project awardees re: initial findings | • Revisions to tool based on:  
  - Engagement Rubric  
  - Pilot project data collection and principal investigator input  
  - PCORI Evaluation Group  
  - Advisory Panel on Patient Engagement  
  - PCORnet  
  - Pipeline to Proposal program office |

---

*Board of Governors Meeting, May 5, 2014*
Impact of Engagement

ENACT Captures:

- Who is engaged
- Partnership characteristics – how formed, length, frequency of engagement, etc.
- Level of engagement
- When in research process are they engaged
- Perceived level of influence of partners
- Perceived effects of engagement on research questions, study design, study implementation, and dissemination of results
- Challenges, facilitators
- Lessons learned for engagement
- PCOR principles – respect, co-learning, etc.
Impact of Engagement

Using ENACT, we are assessing all studies that have reached their 12-month point.

For these studies, we expect to be able to describe the impact of *Engagement* on:

- Formation of the Research Questions
- Study Design
- Functioning of the Study Team
- Early Phase of Recruitment
### An Example From Our Evaluation Framework

<table>
<thead>
<tr>
<th>Evaluation Question</th>
<th>Metrics/Indicators</th>
<th>Methods</th>
<th>Sources</th>
</tr>
</thead>
<tbody>
<tr>
<td>What is the effect of patient and stakeholder engagement on the functioning of the study team and on study design?</td>
<td>Engagement Activity Inventory (ENACT): Elicits detailed descriptions of the nature of engagement and its effects</td>
<td>Qualitative and Quantitative Use of ENACT as well as structured interviews</td>
<td>ENACT Progress Reports Project and Engagement Officers</td>
</tr>
</tbody>
</table>
Welcome to Evaluation at PCORI!

Follow our blog series – Evaluating the PCORI Way

- These blogs link to materials from our PCORI Evaluation Group meetings and also ask for your input

Review the questions in our Draft Evaluation Framework – let us know if yours is reflected


Comment on our draft Usefulness Criteria (others coming soon)

- [http://www.pcori.org/public-feedback-on-proposed-usefulness-criteria/](http://www.pcori.org/public-feedback-on-proposed-usefulness-criteria/)

Comment on any or all of it!

- info@pcori.org
BREAK

Join the conversation on Twitter via #PCORI
Our Vision: Patients and the public have information they can use to make decisions that reflect their desired health outcomes.

Our Goals: Increase Information, Speed Implementation, and Influence Research

Methodology Standards Dissemination & Implementation

<table>
<thead>
<tr>
<th>Metric</th>
<th>Q4</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Application Adherence (%)</td>
<td>NA</td>
<td>NA</td>
<td>74%</td>
<td></td>
</tr>
<tr>
<td>Downloads from Web (#)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Citations (#)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Endorsements (#)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Adoption by Others (#)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Pipeline to Proposal Awards

<table>
<thead>
<tr>
<th>Type of Project</th>
<th>FY 2012</th>
<th>FY 2013</th>
<th>FY 2014</th>
</tr>
</thead>
<tbody>
<tr>
<td>Targeted</td>
<td>151</td>
<td>134</td>
<td>154</td>
</tr>
<tr>
<td>PCORnet Pilots</td>
<td>142</td>
<td>150</td>
<td>143</td>
</tr>
<tr>
<td>Pragmatic Studies</td>
<td>8</td>
<td>12</td>
<td>10</td>
</tr>
<tr>
<td>Targeted PFA</td>
<td>450</td>
<td>333</td>
<td>422</td>
</tr>
</tbody>
</table>

Funds Committed for External Research

<table>
<thead>
<tr>
<th>Year</th>
<th>Targeted</th>
<th>Broad</th>
<th>PCORnet</th>
<th>Pilots</th>
</tr>
</thead>
<tbody>
<tr>
<td>FY 2012</td>
<td>350</td>
<td>250</td>
<td>100</td>
<td>0</td>
</tr>
<tr>
<td>FY 2013</td>
<td>300</td>
<td>200</td>
<td>90</td>
<td>50</td>
</tr>
<tr>
<td>FY 2014</td>
<td>250</td>
<td>150</td>
<td>80</td>
<td>40</td>
</tr>
</tbody>
</table>

Communications & Ops (% of Target or Reference)

<table>
<thead>
<tr>
<th>Communications Category</th>
<th>Q4</th>
<th>Q1</th>
<th>Q2</th>
</tr>
</thead>
<tbody>
<tr>
<td>Unique Web Visitors (Same Q year-ago)</td>
<td>151</td>
<td>134</td>
<td>154</td>
</tr>
<tr>
<td>Email Click-thru Rate (Industry Standard)</td>
<td>142</td>
<td>150</td>
<td>143</td>
</tr>
<tr>
<td>Journal Articles (number, no target)</td>
<td>8</td>
<td>12</td>
<td>10</td>
</tr>
<tr>
<td>Media Mentions (Same Q year-ago)</td>
<td>450</td>
<td>333</td>
<td>422</td>
</tr>
</tbody>
</table>

Engagement Event Survey Results

86% of participants say that they have done something new with PCOR since the workshop

2014 Research Funding Commitment

$528M

2014 Staffing Plan – Number of People

<table>
<thead>
<tr>
<th>Quarter</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total</td>
<td>165</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

2014 Expenditures – $M

<table>
<thead>
<tr>
<th>Quarter</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total</td>
<td>183M</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Increase Information
LOIs, Applications, and Awards

- Letters of Intent
- Applications
- Awards
- Applications that were Resubmissions
- Awards that were Resubmissions

<table>
<thead>
<tr>
<th>Funding Cycle</th>
<th>Letters of Intent</th>
<th>Applications</th>
<th>Awards</th>
<th>Applications that were Resubmissions</th>
<th>Awards that were Resubmissions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cycle I</td>
<td>1301</td>
<td>482</td>
<td>25</td>
<td>22</td>
<td>71</td>
</tr>
<tr>
<td>Cycle II</td>
<td>1099</td>
<td>434</td>
<td>51</td>
<td>22</td>
<td>76</td>
</tr>
<tr>
<td>Cycle III</td>
<td>1417</td>
<td>557</td>
<td>71</td>
<td>13</td>
<td>76</td>
</tr>
<tr>
<td>August 2013</td>
<td>1436</td>
<td>1050</td>
<td>461</td>
<td>13</td>
<td>168</td>
</tr>
<tr>
<td>Winter 2014</td>
<td></td>
<td>355</td>
<td>45</td>
<td>13</td>
<td>89</td>
</tr>
<tr>
<td>Spring 2014</td>
<td></td>
<td></td>
<td>79</td>
<td>13</td>
<td>29</td>
</tr>
<tr>
<td>Infrastructure</td>
<td></td>
<td></td>
<td>168</td>
<td>13</td>
<td>89</td>
</tr>
<tr>
<td>Asthma</td>
<td></td>
<td></td>
<td>108</td>
<td>8</td>
<td>29</td>
</tr>
</tbody>
</table>

Board of Governors Meeting, May 5, 2014
N = 169
(Cycles I-III and August 2013, not including Methods)

Projects included in “Other” overlap 2 or more categories
Increase Information
Comparison of Funding Level Announced with Funding Level Awarded

Board of Governors Meeting, May 5, 2014
Operational Excellence

Time from Award to Contract Execution

- **Target = 90 Days**
- **Included in the 82 awards we made from the last cycle were 29 for new initiatives – the CDRNS and PPRNs.**
- **Despite the complexities in negotiating these novel contracts, we executed 41% of them within 90 days. To date, all but one which is pending signature have been executed.**
- **Once we started working on the other awards – 8 from the Targeted Asthma PFA and 45 from the Broad PFA – we executed 70% of them within 90 days.**
Operational Excellence

Science Response Time

<table>
<thead>
<tr>
<th>Fiscal Quarter</th>
<th>Percent within Target</th>
</tr>
</thead>
<tbody>
<tr>
<td>Q4 2013</td>
<td>54%</td>
</tr>
<tr>
<td>Q1 2014</td>
<td>63%</td>
</tr>
<tr>
<td>Q2 2014</td>
<td>81%</td>
</tr>
</tbody>
</table>

Target: within 3 Business Days
Influence Research
Adherence to PCORI Methodology Standards

The August 2013 Cycle, awarded in December of 2013 (N=53), was the first for which adherence to our Methodology Standards was required.

For four sets of standards, most or all of the standards were applicable to most or all of the studies at the time of application.

On average, these four sets of standards were adhered to by 74% of the applications.

<table>
<thead>
<tr>
<th>Set of Standards</th>
<th>Average Percent of Applications Adhering to the Standards in the Set</th>
</tr>
</thead>
<tbody>
<tr>
<td>Formulating Research Questions</td>
<td>93%</td>
</tr>
<tr>
<td>Patient-Centeredness</td>
<td>91%</td>
</tr>
<tr>
<td>Data Integrity and Rigorous Analyses</td>
<td>72%</td>
</tr>
<tr>
<td>Heterogeneity of Treatment Effect</td>
<td>41%</td>
</tr>
</tbody>
</table>
Influence Research
Pipeline to Proposal Awards – Q2 Snapshot

Tier 1
30 Awards

Tier 2
0
Of those, 0 came from Tier 1

Tier 3
0
Of those, 0 came from Tier 2

Research Proposals
None expected from pipeline in FY 2014
Since attending the PCORI Workshop, have you done anything new to conduct, promote, or use patient-centered research?

Among other things, this might include joining research teams that include researchers and patients, finding ways to incorporate patient input into your research, contacting local media outlets about patient-centered research, using patient-centered research to inform your clinical practice, or simply explaining to others what patient-centered research is and how it can benefit patients.

<table>
<thead>
<tr>
<th>Event</th>
<th>Response Rate</th>
<th>Responded YES to Summary Question</th>
</tr>
</thead>
<tbody>
<tr>
<td>October 2012 Engagement Workshop</td>
<td>49%</td>
<td>91%</td>
</tr>
<tr>
<td>December 2012 Engagement Workshop</td>
<td>39%</td>
<td>83%</td>
</tr>
<tr>
<td>Wichita Regional Workshop, 2013</td>
<td>39%</td>
<td>92%</td>
</tr>
<tr>
<td>Memphis Regional Workshop, 2013</td>
<td>40%</td>
<td>79%</td>
</tr>
</tbody>
</table>

Surveys of workshop participants are conducted six months following a workshop to capture new activities in conducting, promoting, or using patient-centered research and new contributions to a proposal for PCORI research funding and are live in the field for 3 weeks.
Since attending the PCORI Workshop, in which of the following ways have you contributed to a proposal for PCORI research funding?

- Acted as a patient or stakeholder partner on a research team
- Acted as a primary or co-investigator on a research team

<table>
<thead>
<tr>
<th>Event</th>
<th>Acted as a Patient or Stakeholder Partner</th>
<th>Acted as a Primary or Co-investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>October 2012</td>
<td>19%</td>
<td>21%</td>
</tr>
<tr>
<td>December 2012</td>
<td>12%</td>
<td>24%</td>
</tr>
<tr>
<td>Wichita 2013</td>
<td>16%</td>
<td>28%</td>
</tr>
<tr>
<td>Memphis 2013</td>
<td>12%</td>
<td>26%</td>
</tr>
</tbody>
</table>
Influence Research
Communications

Website
Our Q2 website unique visitors has surpassed previous quarters and beat our target.

E-mail
We continue to exceed industry standards for open and click-through rates by a wide margin.

Social Media
Because our early Twitter stats were low, year-to-year comparisons against targets aren’t useful. We’re tracking follower growth and impressions and working on a more sophisticated reach analysis.

Media Coverage & Journal Articles
We continue to grow the number of mentions of our work in general/trade media and journal articles,

Because our early Twitter stats were low, year-to-year comparisons against targets aren’t useful. We’re tracking follower growth and impressions and working on a more sophisticated reach analysis.

Note: Media mentions and journal articles tallied here include pieces about PCORI’s work by staff, Board and Methodology Committee members, or PCORI-funded PIs, as well as articles by others that mention our work prominently.
Resolution to Authorize a Letter of Credit

Regina Yan, Chief Operating Officer
Funds to lease additional office space are in the approved FY2014 budget.

The lease of 1919 M Street 2\textsuperscript{nd} floor space requires a letter of credit of $150,000 as a form of security deposit.

The letter of credit requires authorization from the Board.
Board Vote: Authorize a Letter of Credit

Call for a Motion to:
- Approve each resolution in “A Resolution of the Board of Governors of the Patient-Centered Outcomes Research Institute to Authorize a Letter of Credit”

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

Vote:
- Vote to Accept the Final Motion
  - Ask for votes in favor
  - Ask for votes opposed
  - Ask for abstentions
FY2014 Financial Review
(As of 2/28/2014)

Regina Yan, MA
Chief Operating Officer

Board of Governors Meeting
Alexandria, VA
May 2014
Overview

1. Key Accomplishments
2. Summary
   - Revenue and Cash Balance
   - FY2014 Funding Commitments
   - Research Contract Obligations
3. Budget vs Actuals Review (as of 2/28/14)
   - Variance Analysis
   - Cost Savings
   - Delayed expenses
4. Next Steps
5. Strategic Question
Key Accomplishments (as of 2/28/2014)

- $191 million funding committed in December 2013 - PCORnet contracts executed
- Spring cycle PFAs, including Pragmatic Trials and 2 Targeted PFAs, issued
- Two new Advisory Panels established
- 30 Pipeline to Proposal Awards were selected for funding in the amount of $448,069
- Successful onboarding of 38 employees
- Performance review and staff training (management, compliance, etc.) rolled out
## Summary: Revenue and Cash Balance (as of 2/28/14)

<table>
<thead>
<tr>
<th>Revenue Source</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Revenue from PCOR Trust Fund</td>
<td>$206 million</td>
</tr>
<tr>
<td>Federal Appropriation</td>
<td>$120 million</td>
</tr>
<tr>
<td>CMS Transfers</td>
<td>$86 million</td>
</tr>
<tr>
<td>Cash Balance</td>
<td>$488 million</td>
</tr>
</tbody>
</table>
Summary: FY2014 Funding Commitments

<table>
<thead>
<tr>
<th>FY2014 Funding Commitments already approved by Board as of 2/28/14</th>
<th>$191 million</th>
</tr>
</thead>
<tbody>
<tr>
<td>Approved Total Funding Commitments for FY2014 (inclusive of $191M)</td>
<td>$528 million</td>
</tr>
</tbody>
</table>
## Summary: Research Contract Obligations
(Includes all PCORnet awards)

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cumulative Obligation (Funding Commitment Made from inception to 2/28/2014)</td>
<td>$525 million</td>
</tr>
<tr>
<td>Outstanding Obligations (Pending Payments)</td>
<td>$468 million</td>
</tr>
</tbody>
</table>
FY2014 Budget vs Actuals (as of 2/28/2014)

- **FY2014 Approved Budget**: $182.5 million
- **Budget Through 2/28/2014**: $68.6 million
- **Actual Through 2/28/2014**: $26.5 million
<table>
<thead>
<tr>
<th>Department</th>
<th>2014 Budget</th>
<th>Budget thru 2/28/14</th>
<th>Actual thru 2/28/14</th>
<th>Variance thru 2/28/14 ($)</th>
<th>Variance thru 2/28/14 (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Program Expense</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research and Engagement Awards</td>
<td>106,206,944</td>
<td>39,966,667</td>
<td>11,826,309</td>
<td>28,140,358</td>
<td>70%</td>
</tr>
<tr>
<td>Program Support</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Methodology Committee</td>
<td>2,745,000</td>
<td>643,750</td>
<td>550,496</td>
<td>93,254</td>
<td>14%</td>
</tr>
<tr>
<td>Science/Program Development &amp; Evaluation</td>
<td>23,259,838</td>
<td>7,625,022</td>
<td>3,471,456</td>
<td>4,153,566</td>
<td>54%</td>
</tr>
<tr>
<td>Engagement</td>
<td>7,634,436</td>
<td>2,745,031</td>
<td>1,422,358</td>
<td>1,322,673</td>
<td>48%</td>
</tr>
<tr>
<td>Contracts Management</td>
<td>12,594,812</td>
<td>5,824,276</td>
<td>2,718,551</td>
<td>3,105,725</td>
<td>53%</td>
</tr>
<tr>
<td>Board of Governors</td>
<td>1,840,500</td>
<td>791,875</td>
<td>340,335</td>
<td>451,540</td>
<td>57%</td>
</tr>
<tr>
<td>Management and General</td>
<td>28,260,087</td>
<td>11,033,421</td>
<td>6,186,714</td>
<td>4,846,707</td>
<td>44%</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>182,541,617</strong></td>
<td><strong>68,630,042</strong></td>
<td><strong>26,516,220</strong></td>
<td><strong>42,113,822</strong></td>
<td><strong>61%</strong></td>
</tr>
</tbody>
</table>
Key Factors for the Variance

$69 million budgeted through 2/28/2014

Cost savings

Delayed expenses

$27 million actual through 2/28/2014
Underspending: Delayed Expenses

Extramural Research Expense: $27.6 million
- Majority of projects meet program milestones
- Invoices and spending reports come in later than the estimated 90 days after contract execution
- Analysis of financial reports and invoices under way to better forecast timing and pace of research spending

Engagement: $1.3 million
- Engagement awards timeline moved back
- Economies realized in program activities
Underspending: Hiring Timeline

- Staff Headcount as of 10/1/2013: 77
- Headcount as of 2/28/2014: 115
- Anticipated Headcount on 9/30/2014: 165
- Q2 Target: 142
Science: $2.0 million
- The decision to fund pragmatic trials greatly reduces PFA development costs needed (e.g., workgroup meetings, landscape review, etc.)
- More PFA development work was done in-house by staff rather than outsourced to consultants

Contracts Management and Administration: $3.1 million
- Reduced department support from external contractors
- Reduced cost of merit review
- Reduced negotiation costs, streamlined contract template and finalization
Next Steps and Revised Spending Forecast

We are updating our spending forecast based on latest cost assumptions and activity timelines.

The revised FY2014 spending forecast will be presented to the Board in June for review and discussion.
In addition to outlining in our revised spending forecast any major activities that might be shifted into FY2015, what other issues do you want to ensure that we address?
PUBLIC COMMENT

Join the conversation on Twitter via #PCORI
Wrap-up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Governors