Board of Governors Meeting
via Teleconference/Webinar

May 23, 2016
10:00 a.m. -5:45 p.m. ET
Welcome and Introductions

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

Joe Selby, MD, MPH
Executive Director
## Agenda

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<td>Welcome, Call to Order and Roll Call</td>
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<tr>
<td>Q2 Dashboard Review</td>
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<td>Mid-Year Financial Review</td>
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<td>Methodology Committee (MC) Update</td>
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<td><strong>Consider for Approval:</strong> PCORnet Cross-PPRN Research Project Award</td>
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<td>Report on Application Enhancement Efforts</td>
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<td>Portfolio Analysis – Depression, Pain, Sleep, and Fatigue Outcomes</td>
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Consent Agenda Items

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors
Motion for Consent Agenda Items

That the Board approve:

• Minutes from April 26, 2016 Board meeting
• Nomination of Dr. Andrew Bindman to serve on the Engagement, Dissemination and Implementation Committee (EDIC) and the Science Oversight Committee (SOC)
• The Updated Research and Infrastructure Project Budget Increase Policy
Board Vote

Call for a Motion to:

- Approve each of the Motions on the Consent Agenda

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
Executive Director’s Report

Joe Selby, MD, MPH
Executive Director
Welcome, Dr. Andrew Bindman!

- Andrew Bindman, MD, is the newest member of the PCORI Board of Governors
- Recently named Director of the Agency for Healthcare Research and Quality (AHRQ)
- His career as a primary care physician and health services researcher shows great commitment to the care of vulnerable populations and the translation of research into policy
- Dr. Bindman has served as Professor of Medicine, Epidemiology and Biostatistics at the University of California, San Francisco; Robert Wood Johnson Health Policy Fellow; and Senior Advisor to the Centers for Medicare & Medicaid Services, among others. He was elected to the National Academy of Medicine in 2015
BMJ: Partnering with Patients

- In June 2014, the British Medical Journal launched an initiative to incorporate the patient perspective
  - The level of patient involvement is now a stated field in all research papers
  - Patients have also been incorporated into the peer review process and the BMJ editorial board
- BMJ cited PCORI as an organization sharing this patient-centered mission

Discussion groups of patients, carers, and clinicians led by ... the Patient Centered Outcomes Research Institute in the United States, are *shedding light on the mismatch between the questions that patients and doctors want answers to and the ones that researchers are investigating.*

‘Let the patient revolution begin’

*BMJ 2015*
Stakeholder Workshop: Hepatitis C

- A Stakeholder Workshop on Hepatitis C took place on May 18th as a follow-up to the October 2014 meeting on the topic.
- Participation was invite-only and the public was able to virtually attend.
- 33 stakeholders participated, in addition to six PCORI Board members and 17 public audience members.

Next Steps:
- The Board of Governors will discuss the feedback from this workshop and determine whether to pursue a targeted PFA.
2016 Annual Meeting – Overview and Goals

- Nov. 17-19, Gaylord National Harbor Hotel
- ~1,000 members of PCORI community; “Patients Included”
- Plenaries/breakouts, summits, networking opportunities; guided by stakeholder steering committee/staff program committee
- Goals
  - Spotlight PCORI’s leadership role in CER/PCOR
  - Focus on emerging results of funded studies as well as projects in progress
  - Convene awardees/stakeholders to advance discussion of topics of interest, provide new partnership opportunities, inform future work
  - Highlight our emerging dissemination work and efforts to advance knowledge sharing
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Dashboard Review
Second Quarter of FY-2016

Joe Selby, MD, MPH
Executive Director
Enrolling patients in surgical trials is challenging, so in a PCORI-funded study of patient activation that compares surgery to antibiotics to treat pediatric appendicitis, stakeholders provided suggestions to improve enrollment and retention rates. They made the enrollment script more patient- and family-centered and offered an online option for follow-up, which increased enrollment in the trial from 65% to 95%, and increased retention from 58% to 85%.
Specialty Physicians Engaging with PCORI to drive a useful portfolio and facilitate uptake of results

Radiology
The Association of University Radiologists Radiology Research Alliance Task Force reviewed all PCORI-funded radiology projects, and described how the PCORI Methodology Standards apply to medical imaging. The Task Force identified opportunities for future projects in the field, and developed a National Agenda for PCOR.

As radiologists, we must embrace PCOR or risk missing a key opportunity to demonstrate value and improve the care provided to our patients.

Nephrology
In an interview on becoming Editor in Chief of the American Journal of Kidney Diseases, Harold Feldman, MD, MSCE, Director of the Center for Clinical Epidemiology and Biostatistics at the University of Pennsylvania, highlighted the major studies related to Nephrology that PCORI is funding, and said the journal will focus on translating findings from PCOR into clinical practice.
Goal 3 Results: Influence Research

Meharry-Vanderbilt Alliance
Consuelo H. Wilkins, MD

PCORI is credited with being a catalyst for:

- Inclusion of community members and stakeholders in Scientific Review Process for the Vanderbilt Clinical and Translational Science Award Pilot Program
  - Observing PCORI’s review process encouraged them to think more broadly about inclusion of stakeholders in the scientific review and their ability to contribute to proposal review
  - Developing training curriculum based on PCORI’s Mentor Program
- Post-Doctoral Research Fellow Program in Community Engaged Research
  - 2-year program for training researchers in community engagement
  - 2 full-time fellows are supported each year by the NIH CTSA
- Community Scholars Program for pre-doctoral students
  - 1-year immersion experience in community engaged research
  - Includes research partnership and mentoring
  - $5,000 to support the research project and $500 stipend to the community organization

The motivation of researchers because of PCORI funding has been a big stimulus for the work that we do... the availability of PCORI funding and the interest was a catalyst for us to be able to expand our reach.

Consuelo Wilkins, MD
Results of Engagement in Research: Stakeholder Involvement Led to Improved Enrollment


• Awarded 2013, Assessment of Prevention, Diagnosis, and Treatment Options project
• Principal Investigator: Katherine Deans, MD, Nationwide Children’s Hospital

In this PCORI-funded study of a patient activation tool (part of a larger comparison of surgery vs. antibiotics to treat pediatric appendicitis), stakeholders provided suggestions help improve enrollment and retention rates, including making the enrollment script more patient- and family-centered and offering an online option for follow-up.

These changes increased enrollment in the trial from 65% to 95% and increased retention from 58% to 85%.

These are tangible statistics that show that this process of involving the stakeholders can improve the study.

Dr. Minneci, Co-Investigator

This is why we have our stakeholder group, so that we can incorporate their input into all phases of the study. In this situation, it was critical to the success of our project.

Dr. Deans, Principal Investigator

In the Top 5% of research outputs scored by Altmetric
## Progress of Pragmatic Clinical Studies

### Table 1. Active Research Project Details

<table>
<thead>
<tr>
<th></th>
<th>Q3-15</th>
<th>Q4-15</th>
<th>Q1-16</th>
<th>Q2-16</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Number of Active Research Projects</strong>&lt;br&gt;(Contracts Executed, have passed the Start Date)</td>
<td>0</td>
<td>7</td>
<td>10</td>
<td>14</td>
</tr>
<tr>
<td><strong>Number of Projects Eligible for First Evaluation</strong>&lt;br&gt;(Active projects far enough along to be categorized based on progress)</td>
<td>-</td>
<td>0</td>
<td>8</td>
<td>12</td>
</tr>
<tr>
<td>Percent of Projects on Track (in the Green Zone)</td>
<td>-</td>
<td>-</td>
<td>100%</td>
<td>83%</td>
</tr>
<tr>
<td><strong>Number of Projects with Recruitment Milestones in Quarter</strong></td>
<td>-</td>
<td>-</td>
<td>0</td>
<td>4</td>
</tr>
<tr>
<td>Percent of Projects Meeting <strong>100%</strong> of Recruitment Milestones</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>50%</td>
</tr>
</tbody>
</table>

### Distribution of Pragmatic Clinical Studies Project Status by Quarter

<table>
<thead>
<tr>
<th>Quarter</th>
<th>Green Zone</th>
<th>Yellow Zone</th>
<th>Orange Zone</th>
<th>Red Zone</th>
</tr>
</thead>
<tbody>
<tr>
<td>Q1 2016</td>
<td>8</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Q2 2016</td>
<td>10</td>
<td>2</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Number of Projects*
Progress of PCORnet Phase II

Research Projects Under Way in PCORnet

<table>
<thead>
<tr>
<th>Target</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
<th>Q4</th>
</tr>
</thead>
<tbody>
<tr>
<td>Actual</td>
<td>3</td>
<td>10</td>
<td>6</td>
<td>2</td>
</tr>
</tbody>
</table>

Networks Engaged in Research Projects

<table>
<thead>
<tr>
<th>Target</th>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
<th>Q4</th>
</tr>
</thead>
<tbody>
<tr>
<td>Actual</td>
<td>12</td>
<td>13</td>
<td>5</td>
<td>0</td>
</tr>
</tbody>
</table>
PCORnet Network Collaboration

There are currently 8 collaborative Research Demonstration projects taking place in PCORnet involving 25 of 33 networks.

Network Collaborations
in Research Demonstration Projects

Number of Networks

Study Design

- Observational Study
- Randomized Trial

Key
- ADAPTABLE Study
- Obesity Studies
- PPRN Demonstration Projects
PCORnet Front Door Policy

Internal Launch of PCORnet Front Door

Front Door Policy Approved

Public Opening of PCORnet Front Door

Inquiry Types
(as of April 15, 2016)
• 13 Trials
• 4 Observational Studies
• 2 Participating Sites Inquiries

Requester Types
(as of April 15, 2016)
• 8 Academic
• 6 Industry
• 2 Non-profit/Foundation
• 1 National Association
• 1 Federal
• 1 Research Center
Research-Ready PCORnet

Data Characterization Progress

Number of DataMarts

DataMart Totals:
71 as of March 31st
75 as of April 15th
80 as of May 5th
83 as of May 19th

Date
Mar 31
Apr 15
May 5
May 19

Time

Phases:
- Approved for Research
- Data Characterization Review
- Prep-to-Research Ready Phase
- Data Characterization Phase
- Diagnostic Query Phase
Discussion Questions for Q2-16:

- Do our FY-2016 Dashboard and associated background materials cover the topics that are most important for your review?
- Do you have questions or comments about our progress or performance on any of our Dashboard indicators?
- Does the in-depth focus this quarter on the progress of PCORnet tell you what you need to know?
Results of Engagement in Research

Enrolling patients in surgical trials is challenging, so in a PCORI-funded study of patient activation that compares surgery to antibiotics to treat pediatric appendicitis, stakeholders provided suggestions to improve enrollment and retention rates. They made the enrollment script more patient- and family-centered and offered an online option for follow-up, which increased enrollment in the trial from 65% to 95%, and increased retention from 58% to 85%.

Highlight: Specialty Physicians & PCOR

Two clinical specialties, Nephrology and Radiology, are working in their fields to implement PCORI Methodology Standards, drive a useful portfolio, and promote uptake of PCORI research results.

Results: Influencing Research

Catalyzed by PCORI, the Meharry-Vanderbilt Alliance developed pre- and post-doctoral programs in community engaged research, and began including stakeholders in their grant review process.
FY2016 Mid-Year Financial Review (As of 3/31/2016)

Larry Becker
Chair, Finance and Administration Committee

Regina Yan, MA
Chief Operating Officer
Overview

• Summary
  • PCORI Revenue and Cash Balance
  • Research and Other Programmatic Funding Commitments
• Budget vs. Actuals Review (as of 3/31/2016)
  • FY2016 Budget vs. Actual by Broad Categories
  • FY2016 Budget vs. Actual Percentages
  • Top Three Factors in Variance
• Funding Commitment Plan: FY2012 - FY2019
• PCORI Estimated Revenue and Expenditures
## Summary: PCORI Revenue and Cash Balance

<table>
<thead>
<tr>
<th></th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cash Balance at 9/30/2015</strong> ($ in millions)</td>
<td>$816.5</td>
</tr>
<tr>
<td>Revenue from 10/1/2015 - 3/31/2016</td>
<td>214.4</td>
</tr>
<tr>
<td>Federal Appropriation</td>
<td>120.0</td>
</tr>
<tr>
<td>CMS Transfers</td>
<td>98.7</td>
</tr>
<tr>
<td>PCOR Fee</td>
<td>(5.0)</td>
</tr>
<tr>
<td>Interest Income</td>
<td>0.7</td>
</tr>
<tr>
<td><strong>Cash Disbursements</strong></td>
<td>(159.4)</td>
</tr>
<tr>
<td><strong>Cash Balance at 3/31/2016 in PCOR Trust Fund and bank account</strong></td>
<td>$871.5</td>
</tr>
</tbody>
</table>

Note: As of March 31, 2016, there were outstanding award obligations of $936 million that will become due and payable as research progresses over time.
### Summary: Research and Other Programmatic Funding Commitments

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cumulative Funding Commitments*</td>
<td>$1,327 million</td>
</tr>
<tr>
<td>(as of 3/31/2016)</td>
<td></td>
</tr>
<tr>
<td>Outstanding Award Obligations**</td>
<td>$936 million</td>
</tr>
<tr>
<td>(as of 3/31/2016)</td>
<td></td>
</tr>
</tbody>
</table>

* Includes Research, Infrastructure, and Engagement funding commitments.

** Outstanding award obligations are amounts of contracts awarded that will require payments during a future period. These amounts will become due and payable as research progresses over time.
## FY2016 Budget vs. Actual by Broad Categories
(As of 3/31/2016)

<table>
<thead>
<tr>
<th></th>
<th>Annual Budget FY2016</th>
<th>Budget thru 3/31/16</th>
<th>Actual thru 3/31/16</th>
<th>Variance thru 3/31/16 ($)</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Award Expense</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research, Infrastructure, and Engagement Awards</td>
<td>$331,526,300</td>
<td>$129,769,402</td>
<td>$111,817,452</td>
<td>$17,939,074</td>
<td>14%</td>
</tr>
<tr>
<td><strong>Program Support</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Methodology Committee</td>
<td>1,636,000</td>
<td>818,000</td>
<td>448,104</td>
<td>369,896</td>
<td></td>
</tr>
<tr>
<td>Science</td>
<td>30,110,200</td>
<td>14,664,880</td>
<td>8,740,785</td>
<td>5,924,095</td>
<td></td>
</tr>
<tr>
<td>Evaluation &amp; Analysis</td>
<td>75,000</td>
<td>37,500</td>
<td>43,163</td>
<td>(5,663)</td>
<td></td>
</tr>
<tr>
<td>Research Infrastructure</td>
<td>2,407,450</td>
<td>1,246,948</td>
<td>1,522,763</td>
<td>(275,815)</td>
<td></td>
</tr>
<tr>
<td>Engagement &amp; Dissemination</td>
<td>12,148,203</td>
<td>5,155,604</td>
<td>3,583,659</td>
<td>1,571,945</td>
<td></td>
</tr>
<tr>
<td>Contracts Management &amp; Administration</td>
<td>6,674,025</td>
<td>3,217,004</td>
<td>2,173,939</td>
<td>1,043,065</td>
<td></td>
</tr>
<tr>
<td><strong>Total Program Support</strong></td>
<td>53,050,878</td>
<td>25,139,936</td>
<td>16,512,413</td>
<td>8,627,523</td>
<td>34%</td>
</tr>
<tr>
<td><strong>Administrative Support</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Board of Governors</td>
<td>1,085,000</td>
<td>521,667</td>
<td>551,897</td>
<td>(30,230)</td>
<td></td>
</tr>
<tr>
<td>Management and General</td>
<td>37,819,122</td>
<td>19,011,948</td>
<td>14,786,710</td>
<td>4,225,238</td>
<td></td>
</tr>
<tr>
<td><strong>Total Administrative Support</strong></td>
<td>38,904,122</td>
<td>19,533,615</td>
<td>15,338,607</td>
<td>4,195,008</td>
<td>21%</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td>$423,481,300</td>
<td>$174,442,953</td>
<td>$143,681,349</td>
<td>$30,761,064</td>
<td>18%</td>
</tr>
</tbody>
</table>

The variance for the same period in FY2015 was $41.4 million or 28%.
# FY2016 Budget vs. Actual Percentages
(As of 3/31/2016)

<table>
<thead>
<tr>
<th></th>
<th>2016 Budget</th>
<th>% of Total Budget</th>
<th>Actual thru 3/31/16</th>
<th>% of Total Actual</th>
</tr>
</thead>
<tbody>
<tr>
<td>Award Expense</td>
<td>$ 331,526,300</td>
<td>78%</td>
<td>$ 111,830,328</td>
<td>78%</td>
</tr>
<tr>
<td>Program Support</td>
<td>53,050,878</td>
<td>13%</td>
<td>16,512,413</td>
<td>11%</td>
</tr>
<tr>
<td>Administrative Support</td>
<td>38,904,122</td>
<td>9%</td>
<td>15,338,607</td>
<td>11%</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>$ 423,481,300</strong></td>
<td><strong>100%</strong></td>
<td><strong>$ 143,681,349</strong></td>
<td><strong>100%</strong></td>
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## Budget vs. Actual Review: Top Three Factors in Variance

<table>
<thead>
<tr>
<th>Key Factors in Variance</th>
<th>Amount ($)</th>
<th>% of Total Variance</th>
</tr>
</thead>
<tbody>
<tr>
<td>Award Expense</td>
<td>$17.9 million</td>
<td>58%</td>
</tr>
<tr>
<td>Salaries and Benefits</td>
<td>$3.4 million</td>
<td>11%</td>
</tr>
<tr>
<td>Evidence to Action Networks</td>
<td>$1.7 million</td>
<td>6%</td>
</tr>
</tbody>
</table>
### Funding Commitment Plan: FY2012 – FY2019

**FUNDING COMMITMENT PLAN** ($ in millions)

<table>
<thead>
<tr>
<th>FISCAL PERIOD</th>
<th>RESEARCH*</th>
<th>INFRASTRUCTURE (PCORnet)**</th>
<th>ENGAGEMENT</th>
<th>TOTAL</th>
</tr>
</thead>
<tbody>
<tr>
<td>Inception to FY2013</td>
<td>$272</td>
<td>$ -</td>
<td>$ -</td>
<td>$272</td>
</tr>
<tr>
<td>FY2014</td>
<td>305</td>
<td>103</td>
<td>3</td>
<td>411</td>
</tr>
<tr>
<td>FY2015</td>
<td>370</td>
<td>149</td>
<td>16</td>
<td>535</td>
</tr>
<tr>
<td>FY2016</td>
<td>415</td>
<td>43</td>
<td>24</td>
<td>482</td>
</tr>
<tr>
<td>FY2017</td>
<td>345</td>
<td>-</td>
<td>28</td>
<td>373</td>
</tr>
<tr>
<td>FY2018</td>
<td>345</td>
<td>-</td>
<td>27</td>
<td>372</td>
</tr>
<tr>
<td>FY2019</td>
<td>100</td>
<td>-</td>
<td>23</td>
<td>123</td>
</tr>
<tr>
<td></td>
<td><strong>$ 2,152</strong></td>
<td><strong>$ 295</strong></td>
<td><strong>$ 120</strong></td>
<td><strong>$ 2,567</strong></td>
</tr>
<tr>
<td></td>
<td>84%</td>
<td>11%</td>
<td>5%</td>
<td>100%</td>
</tr>
</tbody>
</table>

* Research funding commitments include $60 million in projects conducted within PCORnet.

** Infrastructure (PCORnet) funding commitments are CER capacity building investments that make the data and partnerships with patients, clinicians and researchers available to CER researchers, but does not actually invest in Research that uses this infrastructure, as does the Research funding. Infrastructure awards include funding for CDRN and PPRN networks, PCORnet coordinating centers, health plan infrastructure, and CMS linkage project to supplement CDRN data with Medicare claims.
PCORI Estimated Revenue and Expenditures

<table>
<thead>
<tr>
<th>Description</th>
<th>In Millions</th>
<th>% of Total Expenditures</th>
</tr>
</thead>
<tbody>
<tr>
<td>Revenue (thru FY2019)</td>
<td>$3,258</td>
<td></td>
</tr>
<tr>
<td>Awards (Research/Infrastructure/Engagement)</td>
<td>$2,567</td>
<td>79%</td>
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<tr>
<td>Dissemination</td>
<td>$103</td>
<td>3% (or 4% of Awards)</td>
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<tr>
<td>Program Support</td>
<td>$310</td>
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<tr>
<td>General Admin</td>
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<tr>
<td><strong>Total Expenditures</strong></td>
<td><strong>$3,258</strong></td>
<td><strong>100%</strong></td>
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</table>

* $2.6 billion will be committed by FY2019. Expenses will continue through FY2024 until all research projects are completed.

**Dissemination:** Includes major PCORI dissemination activities, as well as funds provided to awardees to conduct dissemination

**Program Support:** Includes costs related to Methodology Committee, Science, Engagement, and Contract Management

**General Admin:** Includes costs related to the Board, administrative staff, rent, IT system infrastructure, etc
Methodology Committee Update

Robin Newhouse, PhD, RN
Chair, PCORI Methodology Committee
Methodology Committee Members

- Robin Newhouse, Chair
- Steven Goodman, Vice Chair
- Naomi Aronson
- Ethan Basch
- Stephanie Chang
- David Flum
- Cynthia Girman
- Mark Helfand
- Michael Lauer
- David Meltzer
- Brian Mittman
- Sally Morton
- Neil Powe
- Mary Tinetti
- Adam Wilcox

New MC member: Stephanie Chang
Session Topics and Objectives

- Implementation of the PCORI Methodology Standards
- Update on the public comment period for the draft revisions to the PCORI Methodology Standards
- Coordinating with the Clinical Trials Advisory Panel
- Other updates
  - Network Research Methods work group
  - MC advisors
Goals of Implementation of the PCORI Methodology Standards

• Help investigators understand and use the Standards

• Establish a system for using the Standards to ensure methodological integrity of research projects funded by PCORI

• Identify barriers to use of the Standards
Helping Researchers Understand and Use the Standards

• Webinars for applicants (launched in 2013)

• PCORI outreach conferences (launched in 2014)

• Online CME program (launched in 2015)

• Academic curriculum (launched in 2016)
Using the Standards to Ensure Methodological Integrity of Funded Projects
Uptake of Methodology Standards

Public Comments on the Draft Revisions to the Methodology Standards

- Public comment period open between January and April, 2016
- Total comments: 84
- Stakeholder groups represented:
  - Health researchers
  - Industry
  - Caregivers/family members
  - Patients
- The public comments will guide final revisions to the Methodology Standards and will be summarized in the revised PCORI Methodology Report
## Overview of Public Comments

<table>
<thead>
<tr>
<th>Standard Category</th>
<th>Number of comments</th>
</tr>
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<tbody>
<tr>
<td>Formulating Research Questions</td>
<td>22</td>
</tr>
<tr>
<td>Patient-Centeredness</td>
<td>12</td>
</tr>
<tr>
<td>Data Integrity and Rigorous Analysis</td>
<td>9</td>
</tr>
<tr>
<td>Preventing and Handling Missing Data</td>
<td>4</td>
</tr>
<tr>
<td>Heterogeneity of Treatment Effects</td>
<td>5</td>
</tr>
<tr>
<td>Data Registries</td>
<td>3</td>
</tr>
<tr>
<td>Data Networks as Research-Facilitating Structures</td>
<td>5</td>
</tr>
<tr>
<td>Causal Inference Methods</td>
<td>12</td>
</tr>
<tr>
<td>Adaptive Trial Designs</td>
<td>3</td>
</tr>
<tr>
<td>Studies of Diagnostic Tests</td>
<td>4</td>
</tr>
<tr>
<td>Systematic Reviews</td>
<td>0</td>
</tr>
<tr>
<td>Research Designs Using Clusters</td>
<td>5</td>
</tr>
</tbody>
</table>
PCORI’s Clinical Trials Advisory Panel

• Established to advise PCORI and other entities on best practices for clinical trials

• Close collaboration with and oversight by the Methodology Committee

• Types of advice and resources
  • Strategies for development of the PCORI clinical trials portfolio
  • Guidance on the conduct of clinical trials
  • Position papers
Complementary Activities of MC & CTAP

Methodology Committee
- New and updated Methodology Standards
- Dissemination of the Methodology Standards

Advisory Panel on Clinical Trials
- Selection, research design, implementation, technical issues of clinical trials
- Recruitment, Accrual and Retention Subcommittee
- Standardization of Complex Concepts and Their Terminology Subcommittee
Network Research Methods Work Group

• Data Quality and Missing Data expert meeting held on December 10, 2015

• Planned follow up activities include:
  • Potential guidance and standards
  • Webinars and workshops
  • Collaboration with PCORnet
Thank You!

Robin Newhouse, PhD, RN
Chair, PCORI Methodology Committee
Consider for Approval: PCORnet Cross-Patient-Powered Research Networks (PPRN) Demonstration Project

Rachael Fleurence, PhD
Program Director, Research Infrastructure
Purpose

• Patient-Powered Research Networks (PPRNs) have a unique opportunity to broaden the scope of their research to include topics that are meaningful to the larger participant community.

• PCORI sought to fund up a comparative effectiveness research (CER) project that will demonstrate scientific, administrative, and operational capacity to collaborate across PPRNs.

• This project also had to address comparative clinical and/or health care services questions that reflect shared information needs and decisional uncertainties commonly faced by the collaborating PPRN communities.
Project Background

- **Project Title:** Healthy Mind Healthy You

- **Research Question:** What is the comparative effectiveness of two online, evidence-based approaches to using mindfulness to improve well-being?
  - 8 session mindfulness-based cognitive behavioral therapy (MBCT)
  - 3 session “mindfulness light”

- **Study Design:** Prospective Randomized Comparative Effectiveness Trial
  - **Targeted Sample Size:** 8,500

- **Length of follow-up time:** 3 months

- **Total Budget:** $4M

- **All 20 PPRNs are collaborators on the project**

- **Led by MoodNetwork PPRN**
Intervention Background

• **Outcomes:**
  • Well-being (primary), perceived stress, anxiety, depression, psychosocial functioning, quality of life, and mindfulness

• **Specific Aims:**
  • Determine whether a brief 3-session “mindfulness-light” intervention compared to a standard 8-session MBCT intervention will improve well-being in PPRN participants
  • Explore the heterogeneity of treatment effects to both interventions.
  • Contribute to the PCORnet Commons

• **Potential Impact:**
  • Help determine whether a standard MBCT intervention compared to a brief mindfulness approach will have a clinically meaningful effect on individual participant stress and well-being

• **Contributions to the PCORnet Commons:**
  • Web-based intervention tools for managing stress, depression, and anxiety
  • Lessons learned regarding governance, data, engagement, and dissemination for cross-PPRN research
**Slate Overview:**

*Cross-Patient-Powered Research Network (PPRN) Research Demonstration Project*

<table>
<thead>
<tr>
<th>PFA</th>
<th>Allotted</th>
<th>Proposed Total Budget*</th>
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<tbody>
<tr>
<td>Cross-Patient-Powered Research Network</td>
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<td>$4M</td>
</tr>
<tr>
<td>(PPRN) Demonstration Project</td>
<td></td>
<td>$4M</td>
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</table>

*Total budget = direct + indirect costs

*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*
Board Vote

Call for a Motion to:

- Approve funding for the Cross-Patient-Powered Research Networks (PPRN) Demonstration Project

Call for the Motion to Be Seconded:

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

Roll Call Vote:

- Vote to Approve the Final Motion
  - Ask for votes in favor, opposed, and abstentions
Application Enhancement Efforts

Jean Slutsky, PA, MSPH
Chief Engagement and Dissemination Officer
Program Director for Communication and Dissemination Research

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

Regina L. Yan, MA
Chief Operating Officer
Application Enhancement Efforts (2015-2016)

- 4 workgroups/committees and 1 commissioned external review

- Address concerns from funding announcements through application process, merit review, and feedback to applicants

- ~45 recommendations from across workgroups and external review

- Three overarching principles from the workgroup/committee reports:
  - Ensure PCORI culture supports applicant success
  - Implement change management process
  - Improve and increase communication with external stakeholders
Broad Recommendation Categories

- Letter of Intent (LOI) Review Process
- Application Format
- Application Process
- Merit Review (details to be covered in the future)
- Feedback to Applicants
- Strategic Issues
- Engagement
- Systems Issues
- Training
- Benchmarking
What We’ve Done So Far on Recommendations

• Organized a staff steering committee of internal stakeholders and decision-makers

• Categorized recommendations for implementation
  • Immediate (Cycle 3 2016 – PFAs post August 2016)
  • Short-term (Cycle 1 2017 – PFAs post February 2017)

• Developing RFP for change management process
Application Enhancement Steering Committee

Goals of the Application Enhancement Steering Committee are to improve:

• Applicant Experience
• Application Quality
• Process Efficiency

Executive Team Sponsors: Evelyn Whitlock, Regina Yan, and Jean Slutsky

Committee Members: Shevonne Polastre, Suzanne Schrandt, Bill Silberg, Scott Solomon, Tsahai Tafari, Dan Tisch, and Kara Walker

- Immediately complete a full review of all resource and guidance materials to ensure consistency and reduce duplication
- Enhance the ease of use of resource materials on our website
- Standardize all language and templates
- Move to a single Broad PFA with sections for specific programs
- Improve quality of feedback to applicants
- Develop change management process to review and reduce unnecessary changes
Application Enhancement: Short-term by Cycle 1, 2017 – PFAs post February 2017

- Shorten research plan template
- Review PFA cycle timeline
- Include reviewers and applicants in testing of our online system to improve the user experience
- Implement change management process
Next Steps

• Will continue to work through Science Oversight Committee
• Will provide additional details with future updates
Break

We will return at 1:00 pm ET

Join the conversation on Twitter via #PCORI
Stakeholder Panel: Specialty Physicians

Neil M. Kirschner, Ph.D.

Richard L. Schilsky, M.D., FACP, FASCO

Christopher Ethan Cox, MD
Plans for Dissemination & Implementation at PCORI

Debra Barksdale, PhD, RN  
Co-Chair, Engagement, Dissemination, and Implementation Committee

Jean Slutsky, PA, MSPH  
Chief Engagement and Dissemination Officer  
Program Director for Communication and Dissemination Research

Joanna Siegel, ScD  
Director, Dissemination and Implementation
Dissemination and Implementation

• Background

• Initial dissemination and implementation activities for PCORI findings

• Dissemination and implementation activities for selected high-impact studies

Board Discussion to Date:

• Initial presentation to EDIC March 1, 2016
• Updates and discussion at EDIC meeting April 5, 2016
Program Goals

Translation, dissemination, and implementation to improve the usability and uptake of research findings, to improve healthcare delivery and health outcomes

• **Translation** ... *presentation of research findings in language and format* that improves their accessibility to and comprehension by the target audience

• **Dissemination** ... *intentional, active process of identifying target audiences and tailoring communication strategies to increase awareness and understanding of evidence, and to motivate its use in policy, practice, and individual choices* (Mathematica Framework 2015)

• **Implementation** ... *deliberate, iterative process of integrating evidence into policy and practice through adapting evidence to different contexts and facilitating behavior change and decision making* (Brownson et al. 2012)
“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 PCORI’s authorizing legislation
“(1) DISSEMINATION.— ...the Agency for Healthcare Research and Quality, in consultation with the NIH shall broadly disseminate the research findings that are published by the Patient Centered Outcomes Research Institute .... And other government-funded research relevant to comparative clinical effectiveness research. The Office shall create informational tools that organize and disseminate research findings for physicians, health care providers, patients, payers, and policy makers.”

-- from PCORI’s authorizing legislation
PCORI Dissemination & Implementation Program

**Dissemination Planning (Research in Process)**
- Dissemination Framework
- Capacity building
  - Engagement Awards
  - Communication & Dissemination Research
  - Stakeholder Roundtables
- Develop and adopt PCORI policy and processes
- Summarize evidence

**Initial Dissemination**
- Peer review
- Lay and medical professional abstracts
- Journal publications
- Release of findings to study participants
- Final reports on pcori.org
- Limited Competition D&I Awards
- Nomination of findings to AHRQ

**Dissemination for Selected Findings**
- Limited Competition D&I Awards
- Smaller dissemination activities through PCOR-TC (eg, grand rounds)
- Larger PCORI D&I projects (TBD)

AHRQ-PCORI collaborative projects

*Bold type shows activities currently underway*
Capacity Building for Dissemination & Implementation

Engagement Roundtables

• Linkages with critical intermediaries for dissemination
• Convening groups of physicians, nurses, purchasers, pharmacy benefit managers
• Highlight opportunities for involvement; identify motivated individuals/ orgs. for future input

Engagement Awards

• Build organizational capacity for dissemination
• Develop and demonstrate the processes, collaborations, and approaches that will facilitate the dissemination to organization’s membership or target audience

Communication and Dissemination Research

• Comparative effectiveness of approaches
Initial Dissemination Activities for Findings from PCORI-Funded Studies
Peer Review and Release: PCORI’s Obligations

Conduct Peer Review of Primary Research

• Assess scientific integrity
• Assess adherence to PCORI’s Methodology Standards

Release Research Findings

• No later than 90 days after “conduct or receipt”
• Make available to clinicians, patients, and general public
• Make comprehensible and useful to patients and providers for healthcare decisions
• Include considerations specific to certain sub-populations, risk factors, and comorbidities
• Describe process and methods, including conflicts of interest; include limitations and further research needed

-- from PCORI’s authorizing legislation
Lay and Clinician Abstracts

• “PCORI will create a standardized summary of the study’s results for patients and general public....

• Creating and posting the 500-word (lay) abstract ... addresses the following specific provision of the law’s section “Release of Research Findings”: “...(i) convey the findings of research in a manner that is comprehensible and useful ...”

• “...no longer than 90 days after PCORI’s acceptance of the final research report...PCORI will post on its website the 500-word public-facing summary, the 500-word abstract for medical professionals....”

-- PCORI's Process for Peer Review of Primary Research and Public Release of Research Findings; adopted by the Board of Governors February 24, 2015
Timeline: Primary Completion to Results Posted

**Primary completion date**—Date of last data collection for the primary outcome

*Awardee completes data analysis and prepares draft final research report*

**Within 13 months**, awardee submits draft final research report to PCORI. PCORI initiates peer review. *Note: PCORI strongly encourages awardees to submit their reports promptly*

**Within 2 months**, PCORI provides peer review comments to awardee

**Within 1.5 months**, awardee responds with disposition of comments and submits final version of research report. PCORI accepts final research report

**Within 3 months**, results (clinician and lay-language abstracts) are posted on PCORI.org

Note: PCORI may allow additional time for response to peer review comments.

-- Adopted by the Board of Governors February 24, 2015
Dissemination & Implementation Activities: All funded studies

Translation, Communication

- Lay and Clinician Abstracts; Peer Review Summary
- Investigator journal publications
  - Public Access provisions
- Return of research results to study participants
  - “PCORI will supply the Awardee Institution with a copy of the 500-word summary ... for distribution to study participants and partners. ...”
  -- PCORI's Process for Peer Review of Primary Research and Public Release of Research Findings
- Other - academic presentations, CE/CME, blogs, materials targeting specific audiences
Responsibilities to include:

- Translation
  - Writing Lay and Clinician Abstracts
  - Summarizing Peer Review comments
  - Revising PCORI website Project Summaries
  - Developing consistent formats for these products

- Dissemination activities for selected findings
  - Grand Rounds, Author in the Room
  - Review of PCORI awards to identify high-impact findings

Projected start date: July 2016
Public Access to Published PCORI Research Findings

• Policy to improve public access to findings in peer-reviewed literature
• PCORI Awardees will deposit manuscripts in PubMed Central
  • Final, peer-reviewed version of accepted manuscript
  • PubMed Central makes available 6-12 months after publication depending on journal policy
• To facilitate immediate access, PCORI will pay up to $3500 per project, directly to journal, to cover fees for providing free public access upon publication.
  • Journal article must present primary research findings
  • Awardees retain discretion in journal choice
Limited Competition Dissemination and Implementation Awards

<table>
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<tr>
<th>Key Information</th>
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</thead>
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<tr>
<td><strong>Cycle:</strong> Cycle 1 2016</td>
</tr>
<tr>
<td><strong>Full Announcement:</strong> Dissemination and Implementation of Patient-Centered Outcomes Research Institute (PCORI) funded Patient-Centered Outcomes Research (PCOR) Results and Products in Real-World Settings</td>
</tr>
<tr>
<td><strong>Purpose:</strong> Offer PCORI awardee teams an opportunity to propose <em>investigator initiated</em> strategies for disseminating and implementing their research results and products.</td>
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<tr>
<td><strong>Letter of Intent (LOI) Deadline:</strong> March 2, 2016</td>
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<tr>
<td><strong>Eligibility:</strong> Current Awardee; draft final research report submitted</td>
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<td><strong>Application Deadline:</strong> June 6, 2016</td>
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<td><strong>Total Direct Costs:</strong> $300,000</td>
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<td><strong>Merit Review:</strong> September 2016</td>
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<td><strong>Funds available up to:</strong> $2,000,000 per cycle</td>
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<td><strong>Awards Announced:</strong> November 2016</td>
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<td><strong>Maximum Project Period:</strong> 2 years</td>
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<td><strong>Earliest Start Date:</strong> January 2017</td>
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<td><strong>Cycles per year:</strong> 3</td>
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</table>
Dissemination and Implementation Activities for Selected High-Impact Studies
Identify priority candidates for dissemination and implementation efforts

- Targeted PFA topics; Pragmatic Clinical Studies (PCS)
- Broad Awards: Developing processes for peer reviewers, PCOR-TC, others to flag promising results
Summarize body of evidence

• Evidence summaries available for many topics

• Evidence Mapping
Set strategy for dissemination and implementation activities

- AHRQ Dissemination & Implementation of PCOR
  - AHRQ is developing nomination process
  - Will select findings based on strength of evidence, implementation feasibility, other criteria
  - PCORI will submit findings through AHRQ process
  - Multiple opportunities for collaboration in dissemination and implementation initiatives
Future Dissemination & Implementation Efforts

- In collaboration with / complementing AHRQ efforts
- Tailored to specific high-impact findings; topic-specific activities
- Stakeholder-informed as to best approaches
- Awardee teams with strengths and experience in dissemination and implementation
- Multi-pronged dissemination and implementation, large investments for blockbuster findings
Questions, Comments?
Break

We will return at 3:15 pm ET

Join the conversation on Twitter via #PCORI
Targeted PCORI Funding Announcement Recommendations for Development

Robert Zwolak, MD, PhD
Science Oversight Committee Chair

Evelyn P. Whitlock, MD, MPH
Chief Science Officer
PCORI Topic Prioritization Pathway

List 1
Nominated Topics

List 2
Approved for Topic Brief Development

List 3
Approved for Advisory Panel Review

List 4
Reviewed by Advisory Panels

List 5
Approved for Refinement

List 6
Approved for a Targeted Funding Announcement

List 7
Listed as a Priority in a Pragmatic Clinical Studies Funding Announcement

Lists 6 & 7
### 8 Awarded Targeted PFAs to Date

<table>
<thead>
<tr>
<th>Title</th>
<th>Date Awarded</th>
<th># of Projects</th>
<th>$ Awarded</th>
<th>Approximate Completion Date</th>
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<tbody>
<tr>
<td>Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma</td>
<td>December 17, 2013</td>
<td>8</td>
<td>$23</td>
<td>Q2—2017</td>
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<tr>
<td>Treatment Options in Uterine Fibroids (Administered by AHRQ)</td>
<td>September 30, 2014</td>
<td>1</td>
<td>$20</td>
<td>Q4—2019</td>
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<tr>
<td>The Effectiveness of Transitional Care</td>
<td>September 30, 2014</td>
<td>1</td>
<td>$15</td>
<td>Q1—2017</td>
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<td>Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons (Administered by NIA)</td>
<td>June 4, 2014</td>
<td>1</td>
<td>$30</td>
<td>Q4—2018</td>
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<tr>
<td>Obesity Treatment Options Set in Primary Care for Underserved Populations</td>
<td>September 30, 2014</td>
<td>2</td>
<td>$20</td>
<td>Q2—2018</td>
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<tr>
<td>Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease</td>
<td>May 4, 2015</td>
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<td>Q4—2018</td>
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<td>Testing Multi-Level Interventions to Improve Blood Pressure Control in High-risk Populations (Administered by NHBLI)</td>
<td>September 4, 2015</td>
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<td>$25</td>
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<td>Clinical Management of Hepatitis C Infection</td>
<td>September 28, 2015</td>
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5 Approved Targeted PFAs

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<tr>
<th>Title</th>
<th>Expected Award Date</th>
<th># of Projects</th>
<th>Budget</th>
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</thead>
<tbody>
<tr>
<td>Treatment-Resistant Depression</td>
<td>Summer 2016</td>
<td>Up to 3</td>
<td>Up to $30M</td>
</tr>
<tr>
<td>New Oral Anticoagulants</td>
<td>Summer 2016</td>
<td>Up to 3</td>
<td>Up to $30M</td>
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<tr>
<td>Treatment Strategies for Managing and Reducing Long-Term Opioid Treatment for Chronic Pain</td>
<td>Summer 2016</td>
<td>Up to 4</td>
<td>Up to $40M</td>
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<tr>
<td>Treatment of Multiple Sclerosis</td>
<td>Summer 2016</td>
<td>Up to 8</td>
<td>Up to $50M</td>
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<tr>
<td>Management of Chronic Low Back Pain</td>
<td>Winter 2017</td>
<td>Up to 2</td>
<td>Up to $22M</td>
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Targeted PFA Topics for Development Pending Approval

- For Board Vote Today:
  - Management of Sickle Cell Disease
  - Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-cancer Pain
  - Community-based Palliative Care Delivery for Adult Patients with Advanced Illnesses and their Caregivers

<table>
<thead>
<tr>
<th>Action</th>
<th>Date</th>
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<tbody>
<tr>
<td>Board of Governors Vote on PFA Development</td>
<td>May 23, 2016</td>
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<tr>
<td>Targeted PFA Announced</td>
<td>August 15, 2016</td>
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<tr>
<td>Letter of Intent Due</td>
<td>September 14, 2016</td>
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<tr>
<td>Application Deadline</td>
<td>December 19, 2016</td>
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<tr>
<td>Merit Review</td>
<td>March 27, 2017</td>
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<tr>
<td>Board of Governors Vote to Approve Awards</td>
<td>May 2017</td>
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Management of Sickle Cell Disease
Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

- Support care transitions from pediatric to adult health care in emerging adults with sickle cell disease (SCD)
Overview: Sickle Cell Disease (SCD)

• SCD is a chronic genetic disorder affecting the body’s red blood cells and induces a series of disease-related complications, such as acute chest syndrome, pain crises, and stroke

• Between 70,000-100,000 Americans, predominantly African Americans, have SCD (concentrated in the South and East)
  • Early onset disease (5-6 months of age)
  • Average lifespan ranges between 36 and 56 years
  • The emerging adult population (ages 16-25) is particularly vulnerable to worsened health outcomes during the time of transition from pediatric to adult care

• By age 45, SCD patients average ~150 hospital visits, and will have accrued almost $1 million in medical expenses
Care Transitions in Emerging Adults

• For emerging adults with SCD, transition in care is a life-changing and continuous process
  • Very different from traditional transition models (e.g., from hospital to home)
  • Children with SCD are now living into adulthood, thus the burden of SCD-related morbidity and mortality has shifted to emerging adults
    • High rates of comorbid conditions (e.g., asthma, restrictive lung disease, cardiac dysfunction and renal dysfunction)
    • Cumulative disease effects
Care Transitions in Emerging Adults (cont.)

• Quality of care decreases from pediatrics to adult care
  • Challenges with access to specialists (e.g., hematologists)
    • ~60% on Medicaid; limits access to specialists
  • Adult care clinicians report dissatisfaction with the quality of care they can provide
  • Patients report dissatisfaction with quality of care they receive

• Emerging adults become disengaged from the healthcare system
  • Loss of usual source of care
    • Decrease in routine preventative and screening visits (for chronic blood transfusions, hydroxyurea treatments, vaccines)
    • More likely to seek care for acute medical events in emergency department
      • 5.0 emergency department visits per year vs. 3.3 in other SCD age groups
Potential to Leverage NHLBI and PCORnet

• Targeted PFA can be actively distributed within soon-to-be-funded NHLBI SCD research consortia

• Applicants may potentially collaborate with, and access data from, PCORnet (CDRNs) across the SCD cohorts
  • Three CDRNs have already collected data on 3000+ SCD patients

**Collaboration with NHLBI or PCORnet CDRNs would be encouraged, but not required. All are welcome to apply.**
Evidence Gaps: Sickle Cell Disease

• Current guidelines are based on weak evidence and/or consensus-based opinion

• SCD-related complications are highest among emerging adults, but there is a lack of evidence about how to improve the care transition process and outcomes

• Further research is needed to help to fill gaps to improve care processes and outcomes for individuals with SCD
  • There are no current CER trials for care transitions for individuals with SCD
    • Necessary to improve healthcare and health outcomes for vulnerable population when evidence base is weak
Summary of Workgroup

- 38 stakeholders submitted 59 questions prior to workgroup meeting

- Staff refined and consolidated the questions into two topic areas: Care Transitions and Pain Management

- By consensus, each breakout group (care transitions and pain management) identified three potential comparative effectiveness questions, for a total of six potential questions. This PFA focuses on the most important one.
Proposed Research Question & Study Details

• **Research Question:** What is the comparative effectiveness of established transition coordination models for emerging adults with SCD transitioning from pediatric to adult care?

• **Population:** Emerging adults (e.g., 16-25 years of age) with SCD
  • SCD patients typically transition from pediatric to adult care between 16-18 years of age (timing varies based on needs and readiness)
  • Pediatricians may continue to see patients through college
  • By 26 years of age, emerging adults are no longer covered by their parents’ insurance
    • Interest in older age (up to 30 years of age) range to assess issues related to insurance transitions for emerging adults
Interventions and Comparators:

- Interventions must incorporate patients, care givers, and clinicians
- Interventions should be patient-facing, with robust patient engagement
- Direct comparisons of efficacious or commonly used transition coordination interventions

  - Examples could include (but are not limited to):
    - Co-located pediatric and adult care providers;
    - Clinic-based transition coordinator;
    - Virtual consultation (telehealth) with provider or specialist;
    - Use of mHealth (e.g., mobile apps, text messaging)

  - An appropriate comparator may be usual care or standard of care

- Evidence of efficacy in other diseases (e.g., diabetes, cystic fibrosis, congenital heart disease) and transition models may be used
• **Outcomes:**
  - Health related quality of life (e.g., physical and mental health), depression, patient activation/self-management, patient satisfaction and experiences of care, social functioning (e.g., missed days from work and school)
  - Number of hospitalizations and number of days hospitalized due to complications (e.g., pain crises, strokes, comorbid conditions), measures of emergency department use

• **Study Design:** Cluster RCT with sufficient sample size and/or clusters to power study

• **Setting(s):** Outpatient settings including primary care practices, patient-centered medical homes, specialty SCD clinics

• **Timing:** Maximum 5 year study

• **Proposed Research Commitment:** Up to 3 studies, $25M (total costs)
Call for a Motion to:
• Approve $25M (total costs) for Management of Sickle Cell Disease targeted PFA development

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

Roll Call Vote:
• Vote to Approve the Final Motion
  • Ask for votes in favor, opposed, and abstentions
Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-cancer Pain
Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

• Prevent unsafe opioid prescribing while ensuring adequate pain management using either of two related intervention strategies:
  • payer or health system strategies
  • patient and provider communication interventions addressing benefits and harms of various treatments
Background—related PFA, October, 2015

- Clinical Strategies for Managing and Reducing Long-term Opioid Use for Chronic Non-Cancer Pain Targeted PFA
  - Among patients with chronic non-cancer pain on moderate/high-dose long-term opioid therapy, what is the comparative effectiveness of strategies for reducing/eliminating opioid use while managing pain?
  - Among patients with chronic non-cancer pain on moderate/low-dose long-term opioid therapy, what is comparative effectiveness and harms of strategies used to limit dose escalation?
  - $40 million for up to 4 awards
  - Awards anticipated July 2016
  - This proposed announcement is complementary
Abundance of Evidence Gaps

- Wide variation among states in opioid prescribing rates; indicating a lack of consensus about when to prescribe opioids (CDC, 2016)

- Little evidence exists on how to prevent unsafe prescribing of opioids; research focus largely on patients on chronic opioid therapy (Dy et al, 2016)
  - No systematic reviews, RCTs, or controlled observational studies addressing the effects of opioid prescribing policies on clinical outcomes (Chou et al., 2009)
  - A number of strategies targeted to providers and/or patients to promote safe opioid prescribing have been developed but not rigorously evaluated (HHS, 2014)
  - Strategies that have proven successful in managing chronic pain and reducing the risk of opioid misuse for chronic pain have not been tested to promote safer initiation of opioids (Chang, et al. 2015)

- Guidelines recommend use only when alternatives are ineffective (CDC, 2016; Dy et al., 2016)
Two Research Questions for Targeted PFA

**Question 1:** What is the comparative effectiveness of different payer or health system strategies that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?

**Question 2:** What is the comparative effectiveness of different patient and provider facing interventions that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?
Research Question 1: Payer/Health System Strategies

- **Research Question:** What is the comparative effectiveness of different payer or health system strategies that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?

- **Population:** Potential new users of opioids or patients who have used opioids for < 3 months with either acute or chronic pain. Outside of end-of-life care. Does not include treatment for active cancer.
  - Patients with risk factors for dependence, abuse, and harm
  - Conditions where safer alternatives may be as or more effective
  - Conditions at risk of becoming chronic (e.g., nonstructural low back pain)

- **Interventions:** Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management. Interventions must be evidence based or in widespread use.
Research Question 1 (cont.)

- **Outcomes:**
  - **Primary:** Pain, quality of life, functional outcomes, reduction in unsafe prescribing
  - **Secondary:** Anxiety/depression, sleep, disability, harms (tolerance, dependence, addiction/opioid use disorder, overdose, death), provider satisfaction, provider self-efficacy, emergency department utilization

- **Study Design:** Cluster RCT (encourage two active comparators plus usual care arm); or large, prospective observational study; encourage mixed methods

- **Setting:** Primary care, broadly defined to include primary care practices, emergency departments, dentists offices, urgent care centers

- **Time:** 3 years

- **Sample Size:** 600+ for cluster RCT

- **Proposed Research Commitment:** Up to 3 studies, up to $15M (total costs)
Research Question 2: Improved Knowledge, Communication and/or Shared Decision Making

• **Research Question:** What is the comparative effectiveness of different patient and provider facing interventions that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?

• **Population:** Potential new users of opioids or patients who have used opioids for < 3 months with either acute or chronic pain. Outside of end-of-life care. Does not include treatment for active cancer.
  • Patients with risk factors for dependence, abuse, and harm
  • Conditions where safer alternatives may be as or more effective
  • Conditions at risk of becoming chronic (e.g., nonstructural low back pain)
Interventions:
- Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management
- Must be evidence based or in widespread use
- May include combinations of patient and provider education, psychological management strategies, and/or self-management strategies
- Encourage two active comparators but dependent on interventions selected

Outcomes:

<table>
<thead>
<tr>
<th>Primary Outcomes</th>
<th>Secondary Outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Patient</strong></td>
<td><strong>Primary Outcomes</strong></td>
</tr>
<tr>
<td>Knowledge</td>
<td>Patient anxiety (from potential health outcomes)</td>
</tr>
<tr>
<td>- Quality of life (including pain control)</td>
<td>Reduction in unsafe prescribing</td>
</tr>
<tr>
<td>- Functional outcomes</td>
<td>Repeat opioid prescriptions</td>
</tr>
<tr>
<td><strong>Provider</strong></td>
<td>Rate of opioid initiation</td>
</tr>
<tr>
<td>- Knowledge</td>
<td>Satisfaction</td>
</tr>
<tr>
<td>- Reduction in unsafe prescribing</td>
<td>Length of visit</td>
</tr>
<tr>
<td>- Repeat opioid prescriptions</td>
<td>Confidence and self-efficacy</td>
</tr>
<tr>
<td>- Knowledge</td>
<td><strong>Provider</strong></td>
</tr>
</tbody>
</table>
Research Question 2 (cont.)

- **Study Design:** RCT or cluster RCT

- **Setting:** Primary care, broadly defined to include primary care practices, emergency departments, dentist offices, urgent care centers

- **Time:** 3 years

- **Sample Size:** 1700 or greater with multiple follow-up data collection points

- **Proposed Research Commitment:** 3-5 studies, up to $15M (total costs)
Board Vote

Call for a Motion to:
- Approve $30M (total costs) for Preventing Unsafe Opioid Prescribing targeted PFA development

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

Roll Call Vote:
- Vote to Approve the Final Motion
  - Ask for votes in favor, opposed, and abstentions
Community-based Palliative Care Delivery for Adult Patients with Advanced Illnesses and their Caregivers
The goal of the proposed targeted PFA is to generate evidence to:

- Support care planning over time that is consistent with the goals and preferences of patients with advanced illnesses and their caregivers, and

- Support the delivery of coordinated, community-based palliative care that effectively implements those care plans
Overview

• **Advanced, serious illnesses result in significant patient and caregiver burden in terms of physical and psychological symptoms and declining quality of life (QOL)** (Kelley and Morrison, 2015; IOM 2014)

• **Palliative care makes a difference**
  - Systematic reviews show that patients with advanced illnesses who receive palliative care services report clinically meaningful improvements in QOL, lower symptom burden, lower caregiver distress, and reduced hospitalizations (Dy et al., 2012; ICER, 2016; Gomez et al., 2013)

• **Core components of palliative care include:** (IOM, 2014; NCP, 2013)
  - Systematic assessment and management of patient symptoms
  - Psychosocial support for patients and caregivers
  - Advance care planning
  - Coordination among different clinicians to facilitate goal concordant care

• **Palliative care is more than Hospice**
  - Hospice is a setting for delivering palliative care to individuals nearing death
Stakeholder Perspectives

- **Patients and caregivers:** Access to palliative care services is typically limited to inpatient hospitals or end-of-life hospice settings; patients and caregivers need palliative care where they live – in their community (CAPC, 2015)

- **Clinicians:** Limited workforce of palliative care specialists results in significant strain on meeting the needs of patients and caregivers in the community; community-based clinicians feel underprepared to communicate about and deliver palliative care (CAPC, 2015, Kamal et al., 2013)

- **Health systems and payers:** Several approaches to delivering community-based palliative care are emerging; decision makers need comparative information on the most **effective and efficient** ways of organizing and delivering palliative care in the community (ICER, 2016)

- **A 2014 WHO resolution** urges member states to integrate evidence-based palliative care services across all levels of care, **with emphasis on primary care, community and home-based care** (WHO, 2014)
Timeliness of the Targeted PFA

• January 1, 2016 approval of Medicare reimbursement for ACP discussions
• ACA payment reforms incentivize delivery of high quality, coordinated care
• Research funders (e.g., NIH, American Cancer Society) and payers (e.g., CMS) consider PCORI funding of large scale head-to-head trials to be the natural evolution of research in palliative care
• Several federally funded networks and consortia for conducting large scale, multi-site palliative care trials currently exist
• PCORI’s multi-stakeholder meeting (82 attendees) identified advance care planning and community-based models of palliative care as important priority areas for CER
• Recent reports call upon PCORI and other funders to support research to facilitate advance care planning (ACP) and identify the optimal mix of providers and settings to deliver coordinated, community-based palliative care (IOM, 2014; Kelley and Morrison, 2015; CAPC, 2015; Schenker and Arnold, 2015; Halpern, 2015)
Evidence Gaps Limit the Implementation of ACP

- Most ACP studies:
  - Have focused on relatively short-term outcomes and not looked at the impact of ACP on goal concordant care
  - Studies have not focused on facilitating ACP over time
  - Most studies have separately looked at either patient-directed interventions or clinician-directed interventions
    - Few studies have evaluated clinician and patient combined interventions
- Systematic reviews suggest:
  - Future studies are needed to understand the effective elements of ACP and the best way to implement structured ACP in standard care (Houben et al., 2014)
  - Randomized studies, in community settings, with a focus on standardized patient and caregiver outcomes are lacking (Brinkmann-Stoppelenburg et al., 2014)
Question 1: Advance Care Planning

- **Research Question:** What is the comparative effectiveness of different patient, caregiver, and clinician-directed and combination approaches to facilitating advance care planning conversations between adult patients living with advanced illnesses, their caregivers, and clinicians on patient-centered and other outcomes over time?

- **Population:** Geographically and racially/ethnically diverse patients living at home with any advanced illness and who experience a high symptom burden and/or functional limitations and their caregivers
  - Example conditions include advanced heart failure, COPD, advanced kidney disease, advanced neurodegenerative diseases, advanced cancers
  - Clinicians providing healthcare to patients with advanced illness

- **Interventions & Comparators:** Efficacious and/or widely used programs and interventions designed to facilitate ACP conversations and documentation of goals of care for patients with advanced illness over time:
  - Approaches directed at patients and caregivers (e.g. question prompt lists)
  - Approaches to support clinicians in delivering ACP (e.g. clinician skills training)
  - Combined approaches (e.g. Respecting Choices program)
Outcomes

**Proximal Outcomes:**

*Process*
- Identification of surrogate decision maker
- Identification of goals of care
- Documentation of ACP discussion
- Discussion of goals of care with provider

*Patient-centered*
- Satisfaction with communication
- Experience with care
- Shared decision making

**Intermediate Outcomes:**

*Process*
- Revised ACP documentation
- Frequency of ACP discussion

*Patient-centered*
- Understanding of prognosis
- Decision satisfaction, decision regret
- Patient and caregiver QOL, distress, burden

**Distal Outcomes:**

- Goal concordant care
- Setting of death
- Bereavement
Timing: Up to 5 years

Setting: Multi-site community-based settings such as hospital-based clinics, solo or group physician practices, and the patient’s home
  • In-patient institutionalized settings such as nursing homes and hospices are not addressed under this announcement

Design: RCT or cluster RCT, mixed methods are of interest

Sample Size: N=750+ patient and caregiver dyads; multiple follow-up data collection points

Proposed Research Commitment: 3-5 studies, up to $18M (total cost)
Models of Care: Evidence Gaps

- Efficacious models exist, but the utility of the available evidence is limited for informing decisions about the organization and delivery of palliative care services in community settings: (Gomez, 2013; Dy et al., 2012; ICER, 2016)
  - Multi-site studies are lacking
  - Study sample typically lacks geographical and racial/ethnic diversity; studies rarely have adequate statistical power for subgroups analyses
  - There is need to standardize outcomes across studies to facilitate comparisons
  - Comparator in almost all studies is usual care
- Head-to-head studies are needed
Research Question: What is the comparative effectiveness of different established models of palliative care in community settings on improving patient-centered and other outcomes among adult patients with advanced illnesses and their caregivers?

Population: Geographically and racial/ethnically diverse patients living at home with any advanced illness and who experience a high symptom burden and/or functional limitations and their caregivers

- Example conditions include advanced heart failure, COPD, advanced kidney disease, advanced neurodegenerative diseases, advanced cancers
- Applications focused on facilitating the delivery of palliative care in rural and other low resources settings are of particular interest

Interventions & Comparators: Palliative care models to be evaluated in a head-to-head comparison may vary on one or more of the following parameters:

- Level of integration between primary/subspecialty clinicians and palliative care specialists (e.g., consultative model, nurse-led case management model, co-management model)
- Site of palliative care delivery (outpatient clinic/office, home, or both)
- Method of care delivery (in person visit, remote/telemedicine, or both)
Question 2: Models of Care (cont.)

- **Outcomes:**
  - **Primary:** Patient and caregiver QOL; patients’ symptom burden; patient and caregiver distress, caregiver burden; receipt of goal concordant care
  - **Secondary:** Patient experiences of and satisfaction with care, perceptions of symptoms management; healthcare utilization (hospitalizations, emergency department visits); out of pocket costs/expenses

- **Timing:** Up to 5 years

- **Setting:** Multi-site, community-based settings such as hospital-based outpatient clinics, solo or group physician practices, or the patient’s home
  - Inpatient, institutionalized settings such as nursing homes and hospices are not of interest

- **Design:** Cluster RCT

- **Sample Size:** N = 1,000+ patients and their caregivers (total N = 2,000)
  - Multiple follow-up data collection points

- **Proposed Research Commitment:** Up to 3 studies, up to $30M (total costs)
# Timeline

<table>
<thead>
<tr>
<th>Action</th>
<th>Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Advisory Panel</td>
<td>May 27-28, 2015</td>
</tr>
<tr>
<td>Multi-stakeholder Workshop</td>
<td>March 7, 2016</td>
</tr>
<tr>
<td>SOC Endorsement</td>
<td>April 26, 2016</td>
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<tr>
<td><strong>Board of Governors Vote on PFA Development</strong></td>
<td><strong>May 23, 2016</strong></td>
</tr>
<tr>
<td>Targeted PFA Announced</td>
<td>August 15, 2016</td>
</tr>
<tr>
<td>Letter of Intent Due</td>
<td>September 14, 2016</td>
</tr>
<tr>
<td>Application Deadline</td>
<td>December 19, 2016</td>
</tr>
<tr>
<td>Merit Review</td>
<td>March 27, 2017</td>
</tr>
<tr>
<td><strong>Board of Governors Vote to Approve Awards</strong></td>
<td><strong>May 2017</strong></td>
</tr>
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</table>
Call for a Motion to:

- Approve $48M (total costs) for Palliative Care targeted PFA development

Call for the Motion to Be Seconded:

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

Roll Call Vote:

- Vote to Approve the Final Motion
  - Ask for votes in favor, opposed, and abstentions
Additional Proposed Studies to 2015 Cycle 1 and Cycle 2 Award Slates

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer
Additional Study to 2015 Cycle 1
Pragmatic Clinical Studies Slate
<table>
<thead>
<tr>
<th>Project Title</th>
</tr>
</thead>
<tbody>
<tr>
<td>Determining the Optimal Treatment Strategy for Patients who have Chronic Migraine with Medication Overuse</td>
</tr>
<tr>
<td>RofLumilast or Azithromycin to preveNt COPD Exacerbations (RELIANCE)</td>
</tr>
<tr>
<td>Dissemination of Effective Smoking Cessation Treatment to Smokers with Serious Mental Illness</td>
</tr>
<tr>
<td>Patient Empowered Strategy to Reduce Asthma Morbidity in Highly Impacted Populations (PESRAMHIP)</td>
</tr>
<tr>
<td>Comparison of Operative versus Medical Endocrine Therapy for low risk DCIS: The COMET Trial</td>
</tr>
<tr>
<td>Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density</td>
</tr>
</tbody>
</table>

*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*
Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

- **Research Question(s):** How can breast cancer screening be personalized to account for women’s differing breast densities? Does MRI imaging enhance the preoperative evaluation of an initial breast cancer in women, overall or by their differing breast densities?
- **Population:** Women, aged 18-64, with all degrees of breast density
- **Intervention/Comparison, Aim 1:** Screening digital mammography alone *versus* with supplemental screening (tomosynthesis, ultrasound, MRI) in women, considering also the extent of breast density
  - **Aim 2:** Preoperative breast imaging with MRI *versus* no preoperative MRI in women with initial breast cancers, considering also breast density
- **Added Substudy:** Does community radiologists’ interpretation of breast tomosynthesis images change as a result of years of experience or cumulative volume of readings?
  - Does any “learning curve” vary by location of radiologists (academic, community) or specialty focus (general, breast-specialized)?
Comparative Effectiveness of Breast Cancer Screening and Diagnostic Evaluation by Extent of Breast Density

• **Outcomes:**
  • Aim 1: Rates of screen-detected early stage cancers, interval or late-stage cancers, patient-reported outcomes, recall rates, and projected long-term clinical outcomes (breast cancer deaths averted, life years gained, and over-diagnosis)
  • Aim 2: Rates of second cancers, additional cancers detected, definitive surgeries

• **Study Design:**
  • Observational study using with women classified on the basis of imaging studies received, supplemented by surveys and modeling
  • **Sample Size:** 2.8M exams (among 1 million women)
  • **Budget:** $8M
Engagement: Includes Patient Advisory Boards, and Stakeholder Panel comprised of clinical, advocacy, health system, payer, and policy members

Potential Impact:

- Limited evidence on how to best incorporate breast density into clinical care, despite the fact that breast density impacts malignancy detection on screening and direct-to-consumer laws notify women of their breast density in many states, raising questions of supplemental screening
- Limited evidence on whether pre-surgical MRI improves breast cancer outcomes in women, overall or by degree of breast density
- Understanding whether there is a learning curve for some or all radiologists in their performance with a new technology (tomosynthesis) will enhance the accuracy of this study findings and could inform quality considerations in community practice
Slate Overview – 2015 Cycle 1
Large Pragmatic Studies PFA

<table>
<thead>
<tr>
<th>Pragmatic Studies PFA</th>
<th>Announced</th>
<th>Previously Approved Budget</th>
<th>Proposed Total Budget*</th>
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<tbody>
<tr>
<td>Large Pragmatic Studies to Evaluate Patient-Centered Outcomes</td>
<td>$90M</td>
<td>$59M</td>
<td>$67M</td>
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</table>

*Total budget = direct + indirect costs

All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.
### Board Vote

**Call for a Motion to:**

- **Approve** funding the recommended additional award from the Spring 2015 Pragmatic Clinical Studies Cycle

**Call for the Motion to Be Seconded:**

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

**Roll Call Vote:**

- **Vote to Approve** the **Final Motion**
  - Ask for votes in favor, opposed, and abstentions
Additional Studies to Broad Cycle 2 2015 Slate
Broad Cycle 2 2015
What Percentage of Applications are We Proposing to Fund?

Overall Funding Level 28%

<table>
<thead>
<tr>
<th>Category</th>
<th>Submitted Applications</th>
<th>Identified for Funding</th>
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</thead>
<tbody>
<tr>
<td>Addressing Disparities</td>
<td>24</td>
<td>2</td>
</tr>
<tr>
<td>Assessment of Prevention, Diagnosis, &amp; Treatment Options</td>
<td>22</td>
<td>6</td>
</tr>
<tr>
<td>Communication &amp; Dissemination Research</td>
<td>4</td>
<td>3</td>
</tr>
<tr>
<td>Improving Healthcare Systems</td>
<td>31</td>
<td>5</td>
</tr>
<tr>
<td>Improving Methods for PCOR</td>
<td>50</td>
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</tr>
<tr>
<td>Project Title</td>
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<td>---------------</td>
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<tr>
<td>Continued Anticonvulsants after Resolution of Neonatal Seizures: a Patient-centered Comparative Effectiveness Study</td>
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<tr>
<td>Comparative Effectiveness Analyses among Conservative Treatment Strategies For Ductal Carcinoma In Situ</td>
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<tr>
<td>Multi-institutional Trial of Non-operative Management of Uncomplicated Pediatric Appendicitis</td>
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<tr>
<td>Improving Care for Veterans with Post-Traumatic Stress Disorder (PTSD): Comparative Effectiveness of Medications to Augment First-line Pharmacotherapy</td>
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<tr>
<td>Longitudinal Comparative Effectiveness of Bipolar Disorder Therapies</td>
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<tr>
<td>High Intense Periodic vs. Every Week Therapy in Children with Cerebral Palsy (CP)</td>
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*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract. Resubmissions in bold.*
### Improving Healthcare Systems

#### 1 Additional Recommended Project*

<table>
<thead>
<tr>
<th>Project Title</th>
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<tbody>
<tr>
<td>Healing through Education, Advocacy and Law (HEAL) in Response to Violence</td>
</tr>
<tr>
<td>Patient Osteoarthritis Careplan to Inform Optimal Treatment</td>
</tr>
<tr>
<td>Comparing Interventions to Increase Colorectal Cancer Screening in Low-Income and Minority Patients</td>
</tr>
<tr>
<td>Pragmatic Trial Comparing Telehealth Care and Optimized Clinic-Based Care for Uncontrolled High Blood Pressure</td>
</tr>
<tr>
<td><strong>Patient-Centered Hepatitis C Care via Telemedicine for Individuals on Opiate Substitution Therapy: A Stepped Wedge Cluster Randomized Control Trial</strong></td>
</tr>
</tbody>
</table>

*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract. Resubmissions in bold.*
## Slate Overview – Cycle 2 2015

### Broad PFAs

<table>
<thead>
<tr>
<th>Broad PFA</th>
<th>Allotted</th>
<th>Previously Approved Award Budget</th>
<th>Proposed Total Budget*</th>
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<tbody>
<tr>
<td>Addressing Disparities</td>
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<tr>
<td>Assessment of Prevention, Diagnosis, and Treatment Options</td>
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<td>Communications and Dissemination Research</td>
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<td>Improving Methods for Conducting PCOR</td>
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<td><strong>TOTAL:</strong></td>
<td><strong>$76M</strong></td>
<td><strong>$45M</strong></td>
<td><strong>$55M</strong></td>
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</tbody>
</table>

*Total budget = direct + indirect costs. All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.
Board Vote

Call for a Motion to:

• Approve funding for the recommended two additional awards from the 2015 Cycle 2 Broad PFAs

Call for the Motion to Be Seconded:

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

Roll Call Vote:

• Vote to Approve the Final Motion
  • Ask for votes in favor, opposed, and abstentions
PCORI Projects with Key Outcomes: Depression, Pain, Sleep, and Fatigue

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

Heather Edwards, PhD
Program Officer, Evaluation & Analysis
1. Searched portfolio data to identify projects that were coded as having one of the following outcomes:
   • Pain assessments or control – defined as a reduction in perceived pain
   • Level of anxiety, depression, mood or well-being – defined as the level of anxiety, depression, mood, wellbeing of the patient or caregiver (henceforth “Depression”)
   • Coders’ notes for insomnia or sleep (henceforth “Sleep Outcomes”), and fatigue

2. Supplemented portfolio data by searching research plans for pain, insomnia, sleep, fatigue, and depression keywords

3. Noted whether an outcome in one of the four areas appeared to be a primary or secondary outcome of the project, based on a single-coder’s assessment

4. Recorded the instrument or tool proposed to measure the outcome
Results

• The search excluded Methods, Pilots, MOUs, PPRN, and CDRN projects, and included 285 other research projects.*

• The process identified 136 research projects that planned to report one or more outcome related to pain, depression or anxiety, sleep issues, or fatigue. Many of these 136 projects included more than one of the outcomes and outcome categories. Specifically, we identified 240 mentions of these four outcomes in the 136 projects.

*Through the Spring 2015 PCS Cycle, awarded September 2015
Number of Projects with Depression, Pain, Sleep, and Fatigue Outcomes

Cycle I-Spring 2015 PCS Cycle (N=136)*

- Depression or anxiety: 105 projects
- Pain: 60 projects
- Sleep: 19 projects
- Fatigue: 16 projects

*Excludes Methods, Pilots, PCORnet, MOUs. Outcomes were inclusively coded.
Projects by Primary and Secondary Outcomes

Initial review of 136 projects for outcomes of Pain, Depression or Anxiety, Sleep, and Fatigue: primary outcome for 82 projects; secondary outcomes for 117 projects.

*Excludes Methods, Pilots, PCORnet, MOUs. Outcomes were inclusively coded.
Conditions

- For each outcome, we counted the primary conditions represented

- Each project is counted once, even if coded as representing more than one condition (i.e. cancer and mental health)

Measures

- Measures associated with each outcome are shown as word clouds

- The larger the word in the word cloud, the more projects use that measure of the outcome

- The purpose of this set of exploratory slides was to consider how commonly measurement approaches are being used across projects
Conditions with Depression or Anxiety as an Outcome (N=105 projects)
The size of the rectangles correspond to the number of projects with these conditions.
Depression & Anxiety Measures

promis patient health questionnaire-9 (phq-9)

promis patient health questionnaire-8 (phq-8)

hospital anxiety and depression scale (hads)
Conditions with Pain as an Outcome (N=60)

The size of the rectangles correspond to the number of projects with these conditions.
Pain Measures

tool not specified
(evaluated by pain frequency, pain severity and non-pain symptoms) promis-29
memorial symptom assessment scale

brief pain inventory (bpi)
8 item scales for fatigue and sleep disturbance
pain visual analogue scale nrs sf-12
6 item pain scale
back pain-related dysfunction (roland)

project-designed tool
swiss spinal stenosis questionnaire breast cancer pain questionnaire (bcpq)
bothersomeness of back pain (0-10 scale) haq disability index
patient global assessment pain (mcgill) symptoms (dsi)
pain score (11-point scale)
pain interference

promis sf-36
global health scale
oswetry low back pain disability questionnaire
pain and side effects of regimen womac pain subscale
chronic pain self efficacy scale (cpss)

patient-centered outcomes research institute
Conditions with Fatigue as an Outcome (N=16)

The size of the rectangles correspond to the number of projects with these conditions.
Fatigue Measures

- memorial symptom assessment scale
- fatigue severity scale (fss)
- sf-12
- sf-36
- promis
- fatigue severity scale
- hrqol
- multidimensional fatigue inventory-short form (mfsi-sf)
- tool not specified
- fatigue visual analogue scale
- multidimensional fatigue scale
Conditions with Sleep as an Outcome (N=19)

The size of the rectangles correspond to the number of projects with these conditions.
Sleep Measures

- alliance sleep questionnaire
- pittsburgh sleep quality index (PSQI)
- insomnia/sleep MSAS 1 item
- tool not specified
- NIH Toolbox
- MOS-12
- PROMIS
- PROMIS sleep disturbance 4-8 items
- functional outcomes in sleep questionnaire (FOSQ)

the top clinical scales (top-cs)
Conclusions

- Patient-important outcomes are commonly measured in PCORI’s portfolio and across different types of conditions or disease categories.
- Measurement approaches are inconsistent, limiting the synthesis of findings across topics.
- For some outcomes (pain), the most common tool was either not specified or project defined.
- Judicious use of core outcome sets could improve the coherence of PCORI’s research portfolio, and even its uptake, if focused on measures applicable to clinical practice.
Public Comment Period

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Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chairperson, Board of Directors