General Session

Advisory Panel Meeting
January 13–14, 2014
Fairmont Georgetown, 2401 M Street, NW
Washington, DC, 20037
Welcome

Joe Selby, MD, MPH
Executive Director, PCORI

Patient-Centered Outcomes Research Institute
Advisory Panels Updates

- Progress report on PCORI’s research funding and on Advisory Panel recommendations from 2013
- Prioritized topics’ life course
- Coordinating your continued work and the Pragmatic Trials Announcement
- Creation of two new panels: Rare Disease and Clinical Trials (and application numbers)
- Reauthorization of the charters for PCORI’s four standing panels and reappointments
PCORI’s 2013 Accomplishments

As of 2013, more than 270 projects to advance patient-centered comparative effectiveness research

Funded 30 pilot “Pipeline to Proposal” Engagement Awards

Enhancing National Infrastructure for Conducting Comparative Effectiveness Research (PCORnet)

Issued revised PCORI Methodology Report

Established six multi-stakeholder advisory panels to guide funding process
Our Growing Research Portfolio

Total number of research projects awarded to date: 279
Total funds committed to date: $464.4M
Where we are funding research: 40 states (plus the District of Columbia and Quebec)
Minimum research commitment in 2013: $400 million

PCORI - Patient-Centered Outcomes Research Institute
How We Pick Research Questions to Study

1. Gather Suggestions
   - Patient Stakeholder Community

2. Evaluate Suggestions
   - PCORI Staff

3. Prepare Topic Briefs
   - AHRQ and Academic Centers

4. Prioritize Topics
   - Advisory Panels

5. Select Suggestions
   - PCORI Board

6. Release Funding Announcements
   - PCORI Staff

PCORI Staff

Patient-Centered Outcomes Research Institute
### Targeted Funding Announcements

<table>
<thead>
<tr>
<th>Funding Announcements from Board-Prioritized Topics</th>
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<tbody>
<tr>
<td>Clinical Trial of a Multifactorial Fall Injury Prevention Strategy <em>(with NIA)</em> <em>(Improving Healthcare Systems)</em></td>
<td>July 16, 2013</td>
<td>One award $30 million</td>
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<td>Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma <em>(Addressing Disparities)</em></td>
<td>June 18, 2013</td>
<td>Eight awards $23.2M</td>
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<td>Treatment Options for Uterine Fibroids <em>(with AHRQ)</em> <em>(Addressing Options for Prevention, Diagnosis and Treatment)</em></td>
<td>September 30, 2013</td>
<td>One award $20M</td>
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<tr>
<th>In Development <em>(including Advisory Panel Topics)</em></th>
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<tr>
<td>Obesity Treatment Options in Diverse Populations <em>(Addressing Disparities)</em></td>
<td>Anticipated February 2014</td>
<td>$20M</td>
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<td>Transitions in Care <em>(Improving Healthcare Systems)</em></td>
<td>Anticipated February 2014</td>
<td>$15M</td>
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<tr>
<td>Pragmatic Trials <em>(Multiple Advisory Panel Topics)</em></td>
<td>Anticipated February 2014</td>
<td>$90M</td>
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Topics Prioritized Highly by Advisory Panels

**Addressing Disparities**
- Hypertension in minorities
- Heart attacks among racial and ethnic minorities
- Interventions for improving perinatal outcomes
- Reduce lower extremity amputations in minorities

**Clinical Effectiveness Research**
- Management strategies for ductal carcinoma in situ
- Medication treatment options for bipolar disorder
- Treatment strategies for symptoms of osteoarthritis
- Treatment strategies for adults with migraines

**Improving Healthcare Systems**
- Hospital to home transitional care
- Patient empowering care management for patients with chronic conditions
- Interventions for improving perinatal outcomes
# Life Courses of Advisory Panel Prioritized Topics

<table>
<thead>
<tr>
<th>Prioritized Topics</th>
<th>Landscape Review</th>
<th>Workgroups</th>
<th>Targeted PFA Announcement</th>
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<tr>
<td>Bipolar Disorder</td>
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<td>Ductal Carcinoma in situ</td>
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<td>Migraine Headache</td>
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<td>Osteoarthritis</td>
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<td>Transitional Care</td>
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<td>Perinatal Care Management</td>
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<td>Patient-Empowering Care Management</td>
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<td>Integration of Mental Health &amp; Primary Care</td>
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<td>Features of Health Insurance</td>
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<td>Health Communication Models</td>
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<td>Major Vascular Events</td>
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<tr>
<td>Hypertension in Minorities</td>
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<tr>
<td>Interventions for Improving Perinatal Outcomes</td>
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<tr>
<td>Reduce Lower-Extremities Amputations in Minorities</td>
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**Assessment of Options**

**Improving Healthcare Systems**

**Addressing Disparities**

*Feb 2014*
Priority Topics Within Pragmatic Trials

- Bipolar Disorder (CER)
- Ductal Carcinoma In Situ (CER)
- Migraine Headache (CER)
- Osteoarthritis (CER)
- Hypertension in minorities (AD)
PCORI’s Pragmatic Clinical Trials Initiative

- Broad, standing funding announcements devoted solely to pragmatic, head-to-head comparison studies
- Feature increased funding levels, longer studies (up to $15 million, 5 years)
- Emphasize high-priority questions, strong engagement with relevant stakeholder groups
- Target date for release for first announcement: mid-February 2014
- With multiple announcements and 6-8 awards per cycle, potential to fund 12 – 18 large studies of high priority per year
Post-Prioritization Process

Prioritized research topics

From Advisory Panel process*

(PNL recommends Targeted PFA)

Landscape review

Topic-specific workgroup

Additional topic assessment and refinement as needed (staff with Board and PDC)

Final Disposition

Proceed to tPFA

Topics place in broad PFA for PCT/LST's

PCT/LST "worthy"

*Advisory Panel or PDC may also elect to not forward the topic
Distinguishing Topics for Targeted Approach from Topics for “Broad” PCT Announcements

High Priority Topics from Advisory Panel*

1. Specific comparison, study population, and outcomes in mind
2. Desire to set aside money specifically this topic

Proceed to targeted PFA

1. High priority topic, but multiple questions seem important, or specific question is not clear
2. Consider adding to list of high priority questions for PCT/LST announcement

Topics place in broad PFA for PCT/LST’s

*Advisory Panel also elects NOT to forward a topic
PCORnet: the National Patient-Centered Clinical Research Network

A “network of networks” to conduct large-scale, efficient system-based CER.

- **11 Clinical Data Research Networks** - System-based networks, such as hospital systems, health plans - $77 Mil

- **18 Patient-Powered Research Networks** - Patients with a single condition form a research network and participate in research – 17M

- **A Coordinating Center** - Provides technical and logistical assistance under the direction of the Steering Committee and PCORI Staff – 9M
The 4th Panel - PEAP

Assure meaningful patient engagement in:
- Identifying research priorities and topics
- Monitoring research activities
- Conducting our research
- Disseminating research findings

Developed the Patient and Family Engagement Rubric to evaluate and monitor research for its engagement

Work closely with PCORnet to support the PPRN’s in their “disruptive” work
Questions?
Advisory Panel Update: Launching Two New Panels and Planning for New Advisory Panel Members

Kara Odom Walker, MD, MPH, MSHS
Deputy Chief Science Officer, PCORI

Patient-Centered Outcomes Research Institute
Our Advisory Panels

Leverage knowledge and experience of clinicians, researchers, patients, and other experts to help PCORI achieve its goals

Assure meaningful patient engagement in:
- Identifying research priorities and topics
- Monitoring research activities
- Conducting randomized clinical trials
Our Two New Advisory Panels

Two new panels to be launched in 2014
- Advisory Panel on Clinical Trials will support our methodological work
- Advisory Panel on Rare Disease will provide recommendations in research and engagement with the rare-disease research community
- Other panels are planned, including Communication and Dissemination Research
PCORI’s Two New Advisory Panels

Both mandated by PCORI’s authorizing legislation

“(ii) EXPERT ADVISORY PANELS FOR CLINICAL TRIALS.—The Institute shall appoint expert advisory panels in carrying out randomized clinical trials under the research project agenda under paragraph (2)(A)(ii). Such expert advisory panels shall advise the Institute and the agency, instrumentality, or entity conducting the research on the research question involved and the research design or protocol, including important patient subgroups and other parameters of the research. Such panels shall be available as a resource for technical questions that may arise during the conduct of such research.

“(iii) EXPERT ADVISORY PANEL FOR RARE DISEASE.—In the case of a research study for rare disease, the Institute shall appoint an expert advisory panel for purposes of assisting in the design of the research study and determining the relative value and feasibility of conducting the research study.
PCORI’s Two New Advisory Panels

Advisory Panel on Clinical Trials will support our methodological work by providing expertise throughout the selection, design, and implementation of trials.

- 10 to 14 members
  - At least two members will be patients, caregivers, or representatives of patient advocacy organizations.
  - One member will have special expertise in the ethical dimensions of clinical trials.
  - At least half the panel will be selected from technical experts in the conduct of clinical trials.
PCORI’s Two New Advisory Panels

Advisory Panel on Rare Disease will provide recommendations in two broad areas: the conduct of patient-centered comparative clinical effectiveness research in rare diseases and coordination and engagement with the rare-disease research community.

- 12 to 15 members
  - One-third will be patients, caregivers, or representatives of rare disease advocacy organizations.
  - The rest will be clinicians, researchers, policymakers, or representatives of industry or payers.
Application Period: 12/09 – 01/10

pcori
RECEIVED
429 APPLICATIONS
- 238 CLINICAL TRIALS
- 136 RARE DISEASE
- 191 ASSESSMENT OF OPTIONS
- 141 IMPROVING HEALTHCARE SYSTEMS

10 THIRD-PARTY NOMINATIONS

APPLIED FROM
37 US STATES
AND 8 COUNTRIES

APPLICATIONS
220 MEN
206 WOMEN

pcori
Patient-Centered Outcomes Research Institute
Your Service

• The charters for these four advisory panels were approved by the BOG in April 2013, and each was authorized for one year.

• All four established panels have same governance structure relating to authorization, size, terms for members, and term limits.

• All established panels met several times this year and have been working successfully.
## Recent Changes to Advisory Panel Charters

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<tr>
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<th>Initial Structure (Current)</th>
<th>Proposed Future Structure</th>
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<tr>
<td>Authorization for Charters</td>
<td>One year (from the date of the first meeting)</td>
<td>Five years (until December 31, 2018)</td>
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<tr>
<td>Number of Members</td>
<td>12 to 21 members</td>
<td>Same</td>
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<tr>
<td>Term for Members</td>
<td>One-year terms</td>
<td>Three-year terms, staggered</td>
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<tr>
<td>Term Limits</td>
<td>Two terms of one year</td>
<td>One full term of three years</td>
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Note: Since each advisory panel is still new, all members of these advisory panels will be eligible for re-appointment, if interested. Voluntary drop-offs to be replaced (BOG appointed)
Advisory Panelists’ Reappointments

- Less than 6 percent of panelists rescinded their reappointment
- Staggered terms for continuity
- Reappointment lengths will vary among one, two or three years
- PCORI staff and committees will set up a strategy to assign reappointment lengths
Thank you for your continued service to PCORI!
Questions?
Assessment of Prevention, Diagnosis, and Treatment Options

Alvin I. Mushlin, MD, ScM —Panel Chair
Chairman, Department of Public Health, Weill Cornell Medical College; Public Health Physician-in-Chief, New York

Margaret F. Clayton, RN, PhD—Panel Co-Chair
Associate Professor, College of Nursing and Co-Director of the PhD Program, University of Utah
High Priority Research Topics Identified at April 2013 Meeting

- **Bipolar disorder**—Compare the effectiveness of medication regimens for adolescents and young adults with bipolar disorder.

- **Ductal Carcinoma in situ**—Compare the effectiveness of management strategies for ductal carcinoma in situ among women who had this diagnosis made after undergoing screening mammography.

- **Migraine headache**—Compare the effectiveness of treatment strategies for adults with episodic and chronic migraine headaches.

- **Osteoarthritis**—Compare the effectiveness of alternative strategies for stabilizing symptoms in people with osteoarthritis.
Bipolar Disorder and Antipsychotic Use in Children, Adolescents and Young Adults

- December 20th workgroup consisting of multiple perspectives (clinicians, patients, advocacy groups, Medicaid directors, and NIH) recommended:
  1. Research on diagnosis of bipolar disorder
  2. Long-term studies comparing treatments and treatment strategies
Ductal Carcinoma in situ (DCIS)
- Additional feedback from breast cancer stakeholders and federal partners was obtained

Migraine Headache and Osteoarthritis
- Duke Evidence Synthesis Group developed landscape reviews for these two topics
- Panel will discuss important research gaps for and prioritize high priority research questions for both topics at the meeting today
On Tuesday the panel will prioritize 14 new research topics and select a subset of topics for further consideration:

- Treatments options for hypercholesterolemia
- Treatment option for psoriasis
- Management of arrhythmogenic right ventricular dysplasia (ARVD)
- Treatment options for pemphigus vulgaris
- Treatment options involving mesh for the management of inguinal and abdominal hernia
Research Topic Prioritization, continued

- Assessment of benefits and harms of pelvic floor mesh implants
- Robotic surgery for urologic and gynecologic cancers
- Treatments for hearing loss
- Identifying lung cancer in people with lung nodules
- Treatment options for opioid substance abuse
- Biomarker testing for patients with malignancy
- Treatment options for multiple sclerosis
- Proton beam therapy for breast, lung, and prostate cancer
- Treatment options for autism
Improving Healthcare Systems

Trent Haywood, MD, JD—Panel Chair
Senior Vice President, Office of Clinical Affairs and Chief Medical Officer, BCBS Association

Doris Lotz, MD, MPH—Panel Co-Chair
Medicaid Chief Medical Officer, New Hampshire Department of Health and Human Services Office of Medicaid Business and Policy
Accomplishments to Date

- Redefined and prioritized topics
  - Initial prioritization at the April 2013 meeting
  - Further discussion and refinement at the Sept 2013 webinar

- Provided guidance on priority topics
  - Connected staff with experts on specific topics
  - Reviewed topic direction and literature base
  - Participated in patient and stakeholder workgroups as moderators and panelists

- Self-governed
  - Continuously review charter to ensure fulfillment of requirements
  - Suggest other ways to get involved
Moving Forward

- Plan to review and better understand the IHS portfolio as a whole
- Continue to serve as advisors on various fronts
- Hope to interact more with the other PCORI Advisory Panels
  - Learn from each other
  - Network
  - Collaborate
Improving Healthcare Systems
Advisory Panel Members
Addressing Disparities Program’s Mission Statement

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

Program’s Guiding Principle

PCORI is not interested in studies that describe disparities; instead, we want studies that will identify best options for eliminating disparities.
Addressing Disparities Program

Program Goals:
- Identify high-priority research questions
- Fund research that will have an impact
- Disseminate/Implement best/promising practices

Impact/Outcomes:
- Tell a comprehensive story about our effort and impact
- Identify best/promising practices for research and implementation
- Identify research outcomes that translate to improved health outcomes

PCORI Goals:
- Increase information
- Speed implementation
- Influence research

Ongoing Internal and External Programmatic Evaluation
To date, the Addressing Disparities Advisory Panel had two meetings and prioritized or identified 22 topics for potential PFAs:

- 12 topics were prioritized during the April 2013 meeting
- 10 topics were identified during the September 2013 teleconference

In the April 2013 meeting, the panel selected five top topics:

- Health communication models
- Major vascular events
- Hypertension in minorities
- Interventions for improving perinatal outcomes
- Reducing lower-extremity amputations in minorities
Substantial progress has been made on the top four prioritized topics:

- There have been two workgroups
- AD program has commissioned two landscape reviews
- Developing PFAs for 2014 release

The panel has provided input to the AD program on key strategic questions:

- How should *disparities* be defined?
- What types of studies are likely to make a meaningful impact and reduce disparities?
As the AD portfolio continues to grow in 2014, the AD Advisory Panel will provide input on:

- Research gaps where funding is needed
- Continued development of the AD Conceptual Framework for strategic planning and program evaluation
- Opportunities for collaboration in the disparities-research field
- Research priorities within the Broad PFA and other strategic decisions to guide the evolution of the AD portfolio
- Opportunities for disseminating research findings
Patient Engagement

Charlotte W. Collins, JD—Panel Chair
Vice President of Policy and Programs, Asthma and Allergy Foundation of America

Darius Tandon, PhD—Panel Co-Chair
Associate Professor and Associate Director, Center for Community Health, Institute for Public Health and Medicine, Northwestern Feinberg School of Medicine

Patient-Centered Outcomes Research Institute
Accomplishments of Patient Engagement Advisory Panel

- Provided guidance on the development of PCORI’s Patient and Family Engagement Rubric
  - Engagement should occur throughout the trajectory of a project (pre-award, during award, & post-award)
  - Emphasis on meaningful not symbolic involvement (global principles)

- Provided guidance on ways to promote submissions of Engagement Awards from stakeholders outside academic institutions
Patient Engagement Advisory Panel Members
Thank you!

Emma Djabali, BS
Project Assistant, Advisory Panels, PCORI