Progress from the Patient-Centered Outcomes Research Institute (PCORI)

Anne Beal, Chief Operating Officer of the Patient-Centered Outcomes Research Institute

Sharon-Lise Normand, Vice Chair, Methodology Committee of the Patient-Centered Outcomes Research Institute

July 12, 2012
Some Initial Housekeeping

• To minimize feedback, please confirm that the microphone on your telephone is muted.

• To mute your phone, press the mute button or ‘*6’. (To unmute, press ‘*7’ as well.)

• There will be opportunities for questions and discussion at the end of today’s presentations. Please use the chat box at the right side of your screen to submit your questions into the queue at any point and we will call upon you to state your question.

• We will open up the lines for questions from those participating only by phone at the end of each Q&A session.

• Call the WebEx help line at 1-866-229-3239 with technical problems.
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE (PCORI)

Anne C. Beal, Chief Operating Officer

Brookings Institution
Webinar
July 12, 2012
About PCORI

• An independent, non-profit organization authorized by Congress.

• Mission is to fund research that will provide patients, their caregivers and clinicians with the evidence-based information needed to make better-informed health care decisions.

• Committed to continuously seeking input from patients and a broad range of stakeholders to guide its work.
PCORI’s Board of Governors Represents the Entire Health Care Community

- Patients/consumers
- Caregivers
- Physicians, nurses and clinicians
- Hospitals and health systems
- Health plans
- Health services researchers
- State and federal health officials
- Pharmaceutical, device, and diagnostic manufacturers
- Private payers
- Employers

*PCORI Board Members at March 5, 2012, public board meeting in Baltimore, Md.*
PCORI’s Core Duties

• Establish national research priorities
• Establish and carry out a research agenda
• Develop and update methodological standards
• Disseminate research findings
Why Engage?

Guiding Principles for Stakeholder Engagement

- Ask and Prioritize Meaningful Research Questions
- Review Proposals and Conduct Research
- Feedback on PCORI Impact
- Accelerate Dissemination
Future priorities and agendas will be informed by the development process, ongoing stakeholder engagement, and PCORI’s research results.
Establishing PCORI’s First National Priorities for Research and Initial Research Agenda

- 9 Criteria outlined by law
- 5 Draft priorities proposed
- Corresponding agenda drafted
- Public input received and evaluated
- Priorities and agenda revised and approved
- First primary funding announcements issued

Aug-Dec 2011    Jan-May 2012    May 2012
## Criteria for Research Outlined by Law

<table>
<thead>
<tr>
<th>Impact on Health of Individuals and Populations</th>
<th>Addresses Current Gaps in Knowledge/Variation in Care</th>
<th>Patient-Centeredness</th>
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<tbody>
<tr>
<td>Improvability through Research</td>
<td>Impact on Health Care System Performance</td>
<td>Rigorous Research Methods</td>
</tr>
<tr>
<td>Inclusiveness of Different Populations</td>
<td>Potential to Influence Decision-Making</td>
<td>Efficient Use of Research Resources</td>
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National Priorities for Research and Research Agenda

Assessment of Prevention, Diagnosis, and Treatment Options
- Comparisons of alternative clinical options to support personalized decision-making and self-care
- Identifying patient differences in response to therapy
- Studies of patient preferences for various outcomes

Improving Healthcare Systems
- Improving support of patient self-management
- Focusing on coordination of care for complex conditions and improving access to care
- Comparing alternative strategies for workforce deployment

Communication & Dissemination Research
- Understanding and enhancing shared decision-making
- Alternative strategies for dissemination of evidence
- Exploring opportunities to improve patient health literacy

Addressing Disparities
- Understanding differences in effectiveness across groups
- Understanding differences in preferences across groups
- Reducing disparities through use of findings from PCOR

Accelerating PCOR and Methodological Research
- Improving study designs and analytic methods of PCOR
- Building and improving clinical data networks
- Methods for training researchers, patients to participate in PCOR
- Establishing methodology for the study of rare diseases
PCORI Funding Announcements (PFAs)

- Issued May 22
- Based on first four adopted research priorities
- Announcement based on fifth priority to be issued this summer
- Remain “broad” with respect to interest in any condition, as well as cross-cutting questions
- Point out interest in patients with rare diseases
- Include vignettes drawn from focus groups
- Emphasize outcomes that matter to patients
What Makes PCORI Funding Different?

• Special features include:
  – Patient & stakeholder engagement plan
  – Dissemination and implementation Assessment
  – Reproducible and transparent research plan
  – PCORI criteria outlined by statute
  – References methodology standards
  – User-friendly announcements to encourage broader range of applicants
Stakeholder Engagement in PCORI-funded Research

- It is important that key stakeholders are engaged early and throughout the research process.
- PCORI will score applications on how meaningfully patients and stakeholders are engaged.
- Key stakeholders include those for whom the results of the research will be relevant:
  - patients
  - nonprofessional caregivers
  - clinicians (e.g. physicians, nurses, pharmacists, counselors, and other providers of care and support services)
  - patient-advocacy groups
  - community groups
  - researchers,
  - health-related associations,
  - policy makers, and
  - institutions, including organizational providers, purchasers, payers, and industry

What roles should patients and stakeholders play in research teams?

The engagement of patients and stakeholders should include:

• Participation in formulation of research questions;
• Defining essential characteristics of study participants, comparators, and outcomes;
• Monitoring of study conduct and progress; and
• Dissemination of research results.

Source: PCORI PFA Application Guidelines (Sec. 3.1.3.4) http://www.pcori.org/assets/PFAGuidelines.pdf
Be a Reviewer of PCORI Funding Applications

• PCORI invites professional and lay audiences to be reviewers of research applications submitted in response to PCORI funding announcements.

• Reviewers are highly valued members of the PCORI community whose work is essential to helping us support research that will be scientifically rigorous and truly patient-centered.

• Learn more and apply online: [http://www.pcori.org/get-involved/call-for-reviewers/](http://www.pcori.org/get-involved/call-for-reviewers/)
PCORI Pilot Projects Program

• Allocates $30 million in funding over two years for a slate of 50 pilot projects.

• Supports the collection of preliminary data that can be used to advance the field of patient-centered outcomes research.

• Research will address how to engage patients in the research and dissemination process.
PCORI Pilot Project Research Topics

Methods for engaging patients and stakeholders in:

- Informing PCORI’s national priorities
- The research process, along with other stakeholders
- Developing evidence-based decision support tools that account for patient preferences
- Developing patient-centered outcomes instruments
- Researching behaviors, lifestyles, and choices
- Studying patient care team interactions in situations where multiple options exist
- Analytical methods for CER
PCORI’s Next Steps

- Issue draft Methodology Report for public comment
- Establish advisory groups
- Issue targeted funding announcements
- Award first round of primary research contracts
The Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee (MC)

Sharon-Lise Normand on behalf of the MC
July 12, 2012
The PCORI Methodology Committee: Developing and Advancing the Science and Methodology of CER

Comprised of 17 experts in:
- Health services research
- Clinical research
- Comparative clinical effectiveness research
- Biostatistics
- Genomics
- Research methodologies
Objectives of the Methodology Committee

• Provide guidance about the appropriate use of methods in such research

• Establish priorities to address gaps in research methods or their application

• Recommend actions to support standards

• Map research methods to specific research questions (Translation Table)
Developing the Methodology Report

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<tbody>
<tr>
<td></td>
<td>• MC identified and prioritized major research methods questions to be addressed</td>
<td><strong>• Researchers contracted to address selected topics and develop research materials (e.g., reports, summary templates for proposed standard)</strong></td>
<td><strong>• MC conducted in-depth internal review of materials developed by contractors, and support staff</strong></td>
<td><strong>• Refined recommendations and report content per committee evaluations and discussions</strong></td>
</tr>
<tr>
<td></td>
<td></td>
<td><strong>• MC solicited for external feedback on the translation table (RFI)</strong></td>
<td><strong>• MC independently submitted preliminary votes on proposed standards</strong></td>
<td><strong>• MC deliberated to reach consensus on recommendations to be endorsed in the report</strong></td>
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Methodology Report – Information Gathering

**Research Teams**

~100 individuals comprised of 17 groups from across the country were contracted to conduct research from Nov. 2011 to May 2012 (totaling ~$1.5M)

**Workshop External Invitees**

15 experts attended two workshops in March 2012 to provide additional perspectives

**Translation Table RFI Respondents**

24 submissions were received in response to a Request for Information (RFI) to provide input on the translation table framework
## Methodology Report – Information Gathering

<table>
<thead>
<tr>
<th>Electronic Data Systems Interviewees</th>
<th>57 stakeholders were interviewed to understand CER-use in electronic health records and informatics</th>
</tr>
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<tbody>
<tr>
<td>Independent Consultants</td>
<td>8 individuals were contracted to serve as report editors and interim researchers</td>
</tr>
<tr>
<td>Reproducible Research Results</td>
<td>An interim PCORI researcher in partnership with a member of the MC conducted a literature review, which directly informed PCORI’s reproducible and data sharing policies</td>
</tr>
</tbody>
</table>
Methodology Report – Internal Review

The MC deliberated and agreed upon standards using a standardized template, based on the following:

<table>
<thead>
<tr>
<th>Category</th>
<th>Considerations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient-Centeredness</td>
<td>Respect for and responsiveness to individual patient preferences, needs, and values</td>
</tr>
<tr>
<td>Scientific Rigor</td>
<td>Objectivity, minimizing bias, improving reproducibility, complete reporting</td>
</tr>
<tr>
<td>Transparency</td>
<td>Explicit methods, consistent application, public review</td>
</tr>
<tr>
<td>Empirical/Theoretical Basis</td>
<td>Information upon which a proposed standard is based</td>
</tr>
<tr>
<td>Other Considerations</td>
<td>Practicality, feasibility, barriers to implementation, and cost</td>
</tr>
</tbody>
</table>
Methodology Report – Generation

Through consensus, the MC recommended methodologic standards across **eleven research domains**

1. Formulating Research Questions
2. Patient Centeredness
3. Research Prioritization
4. Causal Inference
5. General and Crosscutting
6. Heterogeneity of Treatment Effects
7. Missing Data
8. Data Networks
9. Adaptive Trials
10. Data Registries
11. Diagnostic Testing
Methodology Report – Generation

The **Translation Table** maps research methods to specific research questions.

### Research Question
- Prioritized research questions
- Formulated patient-centered research question

### Interface
- Defines relative importance of *Evidence Characteristics*
- Identify *intrinsic and extrinsic study characteristics*
- Facilitates choices/tradeoffs on a set of dimensions

### Translation Framework
- Matches research question to study design, data source, analytic strategy
- Separate *Frameworks* for different *Research Dimensions*, e.g. therapeutics, diagnostics, evidence synthesis, etc.
Proposed Structure and Function of the Translation Framework

Translation Framework (Phase 2)

Incorporate:
- Prior evidence
- Intent of research and decisions to be made
- Stakeholder perspectives

Patient Question

Research Question

Specify elements:
- Patient population
- Intervention
- Comparator
- Outcomes
- Timing
- Setting

Step 1

Prioritize study characteristics:

Intrinsic:
- Internal validity (bias)
- External validity
- Precision
- Heterogeneity
- Ethical considerations
- Others

Extrinsic:
- Timeliness
- Logistical and resource constraints
- Data availability, quality, and completeness
- Others

Step 2

Step 3

Translation Table for Therapeutics

Study Design

Data Source

Analytic Strategy

Interface

Research Category

Therapeutics

Diagnostics

Evidence Synthesis

Other Categories

Study Execution (Phase 3)

Report & Dissemination; Application (Phase 4)
Connect with PCORI

- Read the Preliminary Draft Methodology Report at http://www.pcori.org/what-we-do/methodology/

- Subscribe to PCORI updates at www.pcori.org/subscribe

- Follow @PCORI on Twitter

- Watch our YouTube channel PCORINews
Roundtable Discussion and Questions

View this and past Active Medical Product Surveillance webinars at:
http://www.brookings.edu/health/Projects/surveillance/roundtables.aspx