

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

Brookings Roundtable on Active Medical Product Surveillance

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. <u>Please use the chat box at the right side of your screen to submit your questions into the queue at any point</u> 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.

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



PCORI Board Members at March 5, 2012, public board meeting in Baltimore, Md.

- 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's Core Duties

- Establish national research priorities
- Establish and carry out a research agenda
- Develop and update methodological standards
- Disseminate research findings

Why Engage?



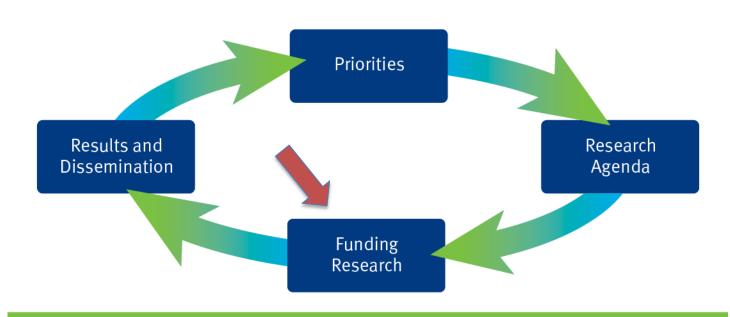
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Guiding Principles for Stakeholder Engagement





PCORI's Path from Priorities to Research Patients can Use



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

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

Impact on Health of Individuals and Populations Addresses
Current Gaps in
Knowledge/
Variation in Care

Patient-Centeredness

Improvability through Research

Impact on Health Care
System Performance

Rigorous Research
Methods

Inclusiveness of Different Populations

Potential to Influence Decision-Making

Efficient Use of Research Resources

National Priorities for Research and Research Agenda



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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

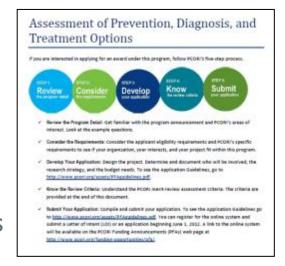
- Improving study designs and analytic methods of PCOR
- · Building and improving clinical data networks
- Methods for training researchers, patients to participate in PCOR
- Establishing methodology for the study of rare diseases





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
- o 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.



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/



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

Generation

Methods MC identified and prioritized major research methods questions to be addressed Selection Researchers contracted to address selected topics and develop research materials (e.g., reports, summary templates for proposed standard) Committee Information MC solicited for external feedback on the translation table (RFI) **Gathering** Workshops held to discuss contractor findings, with invited experts in attendance **Expertise** MC conducted in-depth internal review of materials developed by contractors, and support staff MC independently submitted preliminary votes on proposed **Internal Review** standards MC deliberated to reach consensus on recommendations to be endorsed in the report Refined recommendations and report content per committee Report evaluations and discussions



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

Electronic
Data Systems
Interviewees

57 stakeholders were interviewed to understand CER-use in electronic health records and informatics

Independent Consultants

8 individuals were contracted to serve as report editors and interim researchers

Reproducible Research Results 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



Methodology Report – Internal Review

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

Patient-Centeredness Respect for and responsiveness to individual patient preferences, needs, and values

Scientific Rigor

Objectivity, minimizing bias, improving reproducibility, complete reporting

Transparency

Explicit methods, consistent application, public review

Empirical/ Theoretical Basis

Information upon which a proposed standard is based

Other Considerations

Practicality, feasibility, barriers to implementation, and cost



Methodology Report - Generation

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

Formulating Research Questions

Patient Centeredness

Research Prioritization

Causal Inference General and Crosscutting

Heterogeneity of Treatment Effects

Missing Data

Data Networks Adaptive Trials

Data Registries

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

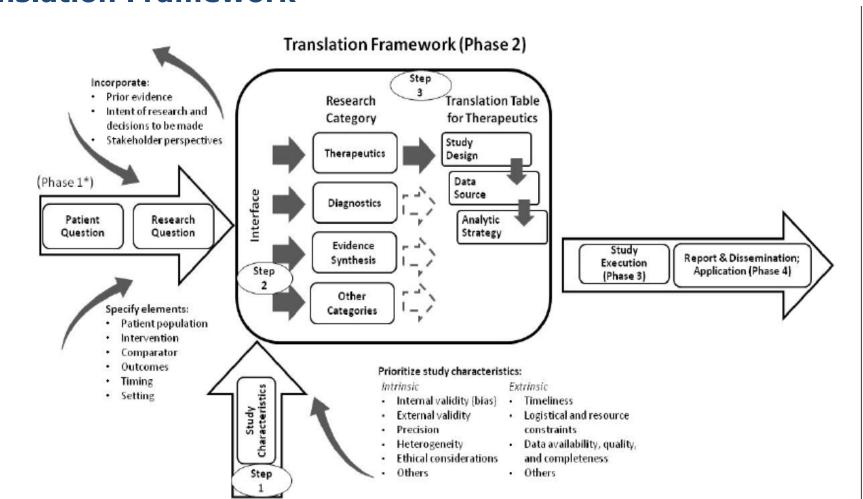
- Defines relative importance of *Evidence Characteristics*
- Identify intrinsic and extrinsic study characteristics
- Facilitates choices/tradeoffs on a set of dimensions

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





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



Patient-Centered Outcomes Research Institute

Preliminary Draft Methodology Report: "Our Questions, Our Decisions: Standards for Patient-centered Outcomes Research"

Presented by the PCORI Methodology Committee to the PCORI Board of Governors May 10, 2012

Published June 4, 2012

A revised draft will be published for public comment in July 2012.

This draft intended as a resource for PCORI Funding Announcements applicants.



Roundtable Discussion and Questions

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