

Patient-Centered Outcomes Research Institute

# Workshop on Incorporating the Patient Perspective into Patient-Centered Outcomes Research

6 and 7 March 2012



# Agenda –Day 1



Start Time	Discussion
12:35 p.m.	Mary Tinetti, Ethan Basch, <b>Welcome</b>
12:40 p.m.	Lori Frank, Andrew Holtz, <b>Workshop Introduction</b>
12:45 p.m.	Hassan Murad, <b>Mayo Clinic</b> , Knowledge & Evaluation Resource Unit
1:05 p.m.	Q&A
1:20 p.m.	Pam Curtis, <b>Oregon Health &amp; Sciences University</b> , Center for Evidence Based Policy
1:40 p.m.	Q&A
1:55 p.m.	Daniel Mullins, <b>University of Maryland</b> , Pharmaceutical Health Services Research Department
2:15 p.m.	Q&A
2:30 p.m.	Break
2:45 p.m.	<b>Group Discussion</b> —Research Teams, External Invitees, & Workgroup Members
4:45 p.m.	Summary & Adjourn
5:00 p.m.	<b>Reception—</b>
—7:00 p.m.	Remarks from Steven Lipstein, Vice Chair, PCORI Board of Governors

# Agenda –Day 2



Start Time	Discussion
8:00 a.m.	Sarah Acaster, Andrew Lloyd, <b>Oxford Outcomes Ltd, Patient Reported Outcomes</b>
8:20 a.m.	Q&A
8:30 a.m.	Zeeshan Butt, Bryce Reeve, <b>Northwestern University</b> , Department of Medical Social Sciences/ <b>University of North Carolina-Chapel Hill</b> , Department of Health Policy & Management
8:50 a.m.	Q&A
9:00 a.m.	<b>Group Discussion</b> —Research Teams, External Invitees, & Workgroup Members
10:00 a.m.	Break
10:15 a.m.	<b>Discussion of Report Content</b> —Research Teams, External Invitees, & Workgroup Members
12:00 p.m.	Ethan Basch, Mary Tinetti, <b>Closing Remarks</b>
12:15 p.m.	Lunch ( <i>Ravenhurst Room</i> )
1:00 p.m.	Adjourn

Patient engagement is an inherent characteristic of PCOR

# Overall Goal



- To inform development of discrete standards for patient engagement in design, implementation, dissemination of PCOR

# Characteristics of Standards



- 1. Actionable:** we wish to operationalize patient engagement to inform PCORI RFAs, investigators, study sections
- 2. Feasible:** we wish to call on examples of how to implement
- 3. Measurable:** we aspire to document impact to inform future research
- 4. Informative:** we wish to generate data that meets decision-making needs of patients and other stakeholders

## **Focus on minimum standards**

## Workshop Goals

- Present and discuss findings
- Identify convergent and any discrepant findings regarding integrating the patient voice into PCOR.
- Discuss proposed minimum methods standards for inclusion of patient voice in PCOR;
  - Get suggestions for standards to include in MC Report recommendations;
  - Informally catalog strength of evidence for each standard;
- Discuss other findings for inclusion in report
- Identify important gaps in knowledge/ next steps

## Board of Governors

### Methodology Committee

Patient Centeredness  
Workgroup

Research Methods  
Workgroup

Research Prioritization  
Workgroup

Report Assimilation  
Workgroup

### Committees of the Board

Program Development  
Committee

Outreach & Engagement  
Committee

Finance & Administration  
Committee



To help people make informed health care decisions and improve health care delivery and outcomes by:

- producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers and the broader health care community



- To define methodological best practices
- To identify gaps in methods knowledge
- To prioritize methodological areas of focus so that PCORI can accomplish its PCOR agenda.

The Methodology Committee is legislatively mandated to prepare a Methodology Report by May 2012 that

- outlines existing methodologies for conducting patient-centered outcomes research (PCOR)
- proposes appropriate methodological standards
- identifies important methodological gaps that need to be addressed

**PUBLIC LAW 111–148**  
**111th Congress.**

MAR. 23, 2010.

An Act.

Entitled The Patient Protection and Affordable Care Act

## **Subtitle D-Patient Centered Outcomes Research**

*The Institute shall establish a standing methodology committee to carry out the functions described in subparagraph (C).*

### **(C) FUNCTIONS.—**

*Subject to subparagraph (D), the methodology committee shall work to develop and improve the science and methods of comparative clinical effectiveness research by, not later than 18 months after the establishment of the Institute, directly or through subcontract, developing and periodically updating the following:*

- (i) Methodological standards for research*
- (ii) A translation table*

A defining principle of PCOR is ensuring that the patient's voice and perspective drive every step of the research process, including prioritizing the research questions, designing and conducting the research, and implementing the results in practice.

## Describe a unique PCORI "lens"

- Individuals who get studied
- Questions that matter
- Comparators that matter
- Outcomes that matter
- Settings that matter
- Dissemination that works

A process, action, or procedure for performing PCOR that is deemed essential to producing scientifically valid, transparent, and reproducible results.

## **A standard may be supported by:**

- scientific evidence
- a reasonable expectation that the standard helps achieve the anticipated level of quality in PCOR, or
- broad acceptance of the practice in PCOR

# Focus on standards: Elements to consider



- Standards or principles?
- “Minimum”
- Levels: supra- vs. subordinate
- Prioritization
- Actionable
- Strength of evidence
- How to communicate?
- Knowledge gaps

- ***Which proposed standards should be nominated for inclusion?***
- Is there a hierarchy that emerges from the full set of standards?
- Is this set of standards comprehensive for the PCWG component of the Report?
- What is the relationship between strength of evidence and recommendation for inclusion for this set?
  - Do PCWG standards differ on this dimension relative to other standards that will be nominated?

# Track additional work recommended



- MC should...
- PCORI should...
- PCOR researchers should...

## Review and Synthesis of Evidence for Eliciting the Patient's Perspective in Patient-Centered Outcomes Research (SOL-PCWG-001)

**Scope:** To conduct a structured and comprehensive review of the literature and environmental scan on the topic.

### **Goals**

- Incorporate the patient (or surrogate) perspective into development of specific research questions within the broad topic.
- Identify methodological standards for incorporating the patient (or surrogate) perspective into study design components, including selection of population, interventions, comparators, outcomes, setting/timing and others.
- Report what approaches have been effective and why, and describe how these approaches can directly inform PCORI's work

## Expert Stakeholder Interviews to Identify Evidence for Eliciting the Patient's Perspective in Patient-Centered Outcomes Research (SOL-PCWG-002)

**Scope:** To conduct key informant interviews with individuals from several stakeholder groups who are expert in eliciting the patients' perspective stakeholder.

### **Goals**

- Incorporate the patient (or surrogate) perspective into development of specific research questions.
- Identify methodological standards for incorporating the patient (or surrogate) perspective into study design components, including selection of population, interventions, comparators, outcomes, and setting/timing and others.
- Report what approaches have been effective and why; and describe how these approaches can directly inform PCORI.

## Review of Guidance Documents for Selected Methods in Patient Centered Outcomes Research (Design and Selection of PROMs) (SOL-RMWG-001)

**Scope:** To produce background papers that propose and justify minimum methodologic standards in the Design and Selection of Patient-Reported Outcomes Measures (PROMs) for Use in Patient Centered Outcomes Research

### **Goals**

- Propose minimum standards for the development and use of tools to assess PROs.
- Review standards in qualitative and quantitative methods used to develop and select measures of the patient experience in experimental and observational clinical CER.
- Examine the primary literature and guidance statements for recommended minimum standards, as well as the properties to be sought or assessed in PROMs proposed for use.
- Include content validity; construct validity; reliability; sensitivity/responsiveness to change; how clinically meaningful change is determined, and feasibility in non-English speaking and/or low literacy populations.
- Include the potential methodological and logistical challenges of applying such standards in "real-world" or non-experimental settings.

# Focus on standards: Elements to consider



- Standards or principles?
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- Strength of evidence
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1. PCORI is interested in methods for gathering patient and other stakeholder input that borrow from a wide range of disciplines. Are we looking in the right places?
2. Are there other sources to consider for an environmental scan that have not been covered?
3. Have we obtained adequate coverage of non-healthcare sectors (business, marketing, transportation, consumer generally)?
4. How can we best capture the heterogeneity of expert and patient experiences and views?

1. Who are the hard-to-reach (HTR) patients? Is the list of HTR categories University of Maryland generated sufficiently comprehensive?
  - a. Does comprehensiveness matter or does an evaluation of the HTR just need to be “representative” of the HTR? Is it possible to be representative of HTR?
  - b. How much do we need to understand about why groups are HTR in order to successfully engage these populations?
2. Are the barriers to “reachability” a meaningful index relevant to participation in PCOR? Does this differ by category?
3. Are hard to reach different from accessible patients in ways important to PCORI goals? What are implications for addressing selection bias?
4. If barrier of “reachability” is overcome, will information obtained about engagement be substantially the same as for all patients?