

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

# Comparing standards across subjects



	<b>Oxford Outcomes</b>	<b>Northwestern University/ UNC Chapel Hill</b>
Conceptual Framework/ Msmt Model	X	X
Reliability <ul style="list-style-type: none"><li>• Internal consistency</li><li>• Test-retest</li></ul>	X	X
Validity <ul style="list-style-type: none"><li>• Content</li><li>• Construct</li></ul>	X	X
Responsiveness	X	X
Sensitivity	X	X
Interpretability	X	X

# Comparing standards across subjects

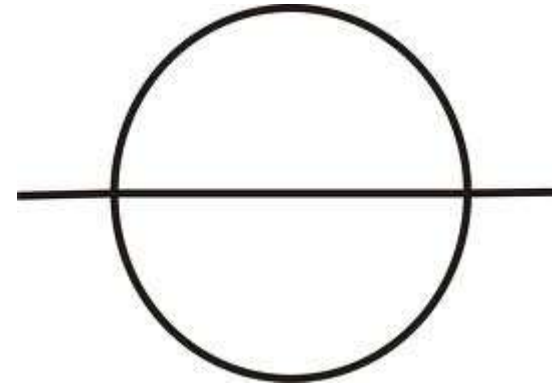


	<b>Oxford Outcomes</b>	<b>Northwestern University/ UNC Chapel Hill</b>
Recall Period	X	X
Burden	X	X
Training Requirements	X	X
Translation	X	X
Modification	X	
Sampling	X	
Multi-Mode Admin		

# The PCOR process – linear or iterative?

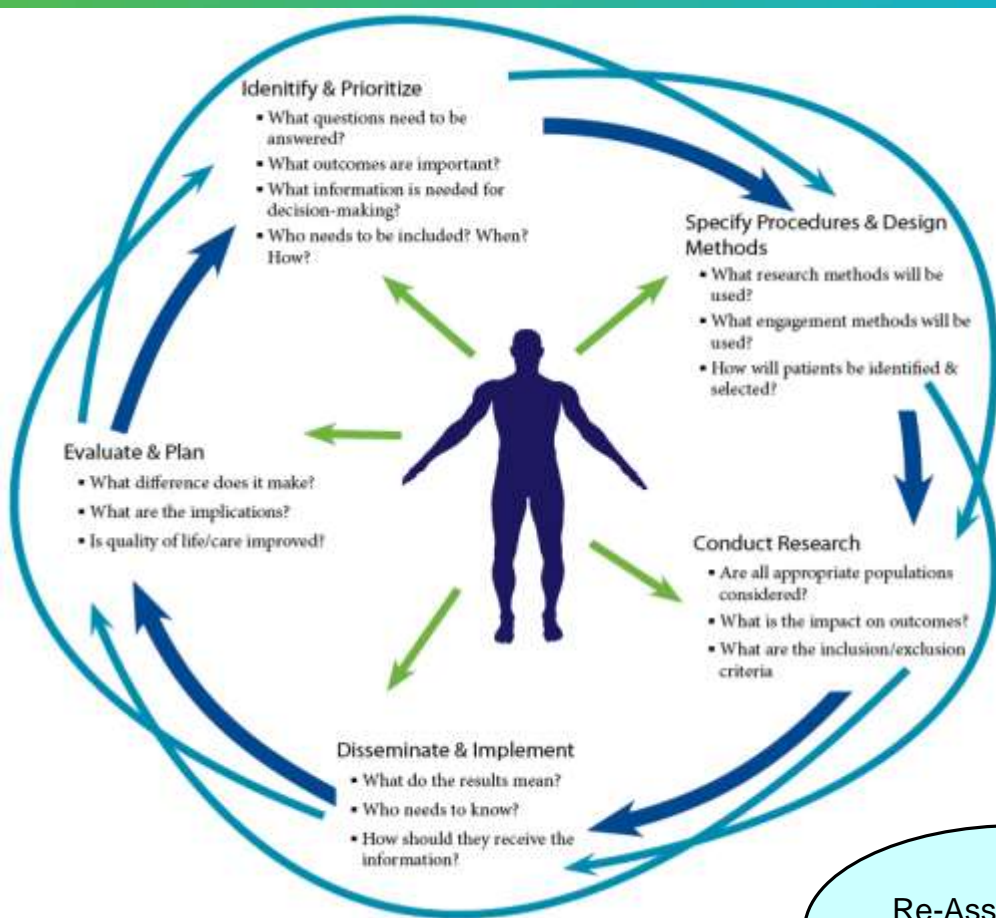


1. selecting research questions
2. identifying comparators
3. selecting outcomes
4. designing the study
5. recruiting participants
6. disseminating results
7. implementing findings



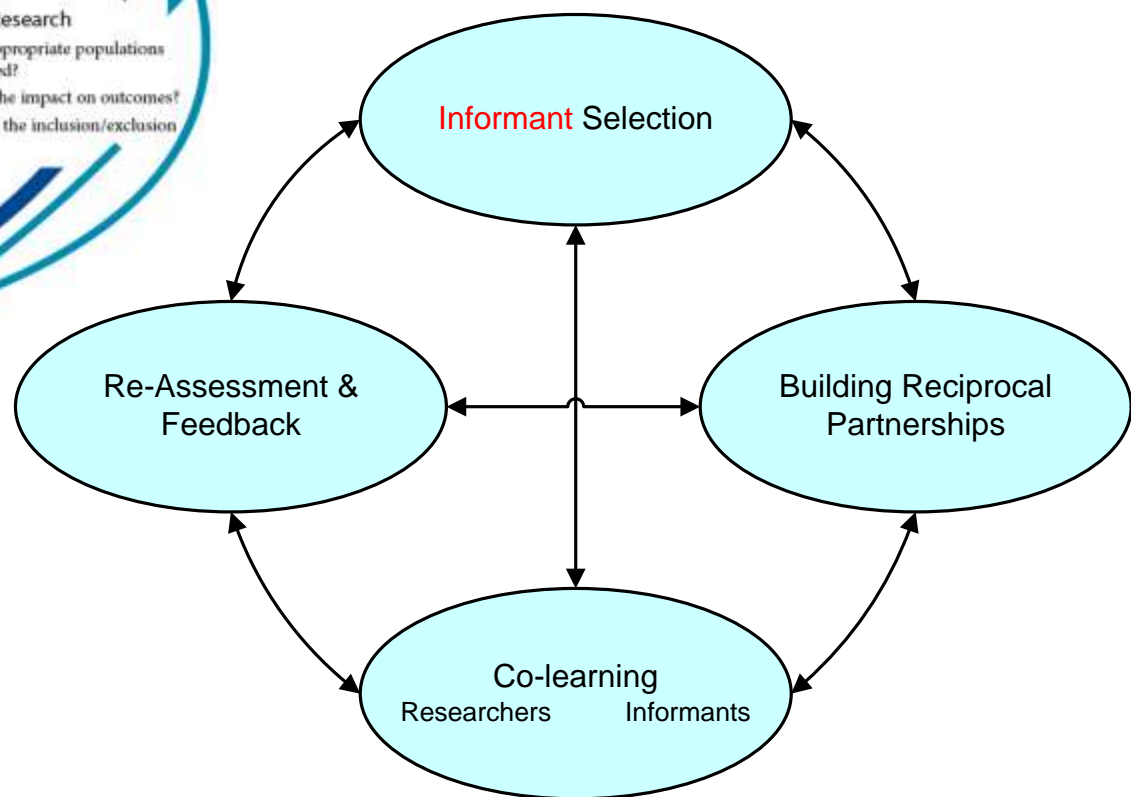
**Where in the process do the standards apply?**

# Representations of the Research Process



**Juan Pablo Domecq Garces, M.D.**  
**Nathan Shippee, Ph.D.**  
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**Pam Curtis, MS**  
**Valerie King, MD, MPH**  
**Cathy Gordon, MPH**



**Oxford Outcomes  
Northwestern University/UNC Chapel Hill**

- Is PROM use for PCOR different in any way from PROM use in research generally?
  - When would this be so?
  - How do the proposed standards for PRO use in PCOR differ from other current standards for PRO use? Should PCOR-specific standards be adopted more generally?
- Scope for this work was limited to PROM use in research settings. How would the standards proposed here relate to PROM use in clinical contexts, and could they support the PCORI research priority on improving healthcare systems?
- What are methodological and logistical challenges of applying the proposed standards in "real-world" or non-experimental settings?
- What are examples of PROMs that adhere to the proposed standards?

- What can we learn about implementing standards from the work so far? What do the findings discussed during this workshop suggest about communication strategies for the proposed standards? Who are the target audiences?
- Do the proposed standards adequately address the role of caregivers or proxies in PCOR?

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



- Trust, Transparency, Co-learning, Reciprocal relationships, Partnerships, Honesty

Ideal practice: “early and often”

- Pre-engagement with target population
- Longitudinal relationship building/maintenance
- Training: Patient and participant
- Require elements for proper indexing –permit evaluation
- Evaluation of comprehension over time

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

# Did we accomplish our goals?



- Review standards suggested for nomination
- Review standards for which consensus did not emerge
- Review confidence in appropriateness of recommended standards for initial Report
  - Are some best kept on hold until the next version of the Report?

# Agenda –Day 2



Start Time	Discussion
8:00 a.m.	<b>Oxford Outcomes</b> —Sarah Acaster, Andrew Lloyd
8:20 a.m.	Q&A
8:30 a.m.	<b>Northwestern University/UNC Chapel Hill</b> —Zeeshan Butt, Bryce Reeve
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.	<b>Closing Remarks</b> —Ethan Basch, Mary Tinetti
12:15 p.m.	Lunch ( <i>Ravenhurst Room</i> )
1:00 p.m.	Adjourn

# Thank you!



# Draft Standards

Patient-Centeredness Work Group

- 1. Impact on Decision Making**
- 2. Engagement Plan**
- 3. Evaluating Engagement Impact**
- 4. Generalizability**
- 5. Patient-Centeredness**
- 6. Dissemination**
- 7. Evaluation**

# 1. Impact on Decision-Making



**A study plan must specify to whom and how study results will provide needed knowledge that informs health care decision-making**



## 2. Engagement Plan



**A plan must be documented describing how informants representative of the ultimate target population will be identified, selected, and engaged throughout the research continuum (i.e., during study design, conduct, and dissemination of results) to inform:**

- Identification of study questions meaningful to patients
- Understanding of key characteristics of the population and subpopulations
- Recruitment and retention strategy
- Selection of outcomes meaningful to patients
- Determination of data collection strategies
- Assessment of risks and burden to study participants
- Selection of comparators
- Dissemination plan and conduct

Methods of engagement may include focus groups, individual Interviews, surveys, and/or ongoing partnership, and should be supplemented by literature review. Engagement methods should employ the principles of trust, transparency , co-learning, respect, and partnership.

### 3. Evaluating Engagement Impact



**Following a study, investigators must report how informants were identified and engaged; how engagement differed from the initial plan; and the specific impact of engagement on the design and conduct of the study and dissemination of results.**

## 4. Generalizability



**The research team must identify the spectrum of individuals that are affected by the topic of your study; the research team must articulate a plan and recruit across that entire spectrum.**

This informs decision-making for this spectrum of people.

**If within this spectrum there are “HTR” patients or other patient groups traditionally difficult to include (e.g., multiple comorbidities), then indicate your specific engagement plans to address these participants including likely plan for likely challenges and plan for addressing those challenges.**

- 5. Patient-Centeredness:** All PCOR will include outcomes and comparators based on what is noticeable and meaningful to patients.
- 6. Dissemination:** Study results must be disseminated to informants, participants, PCORI, and the public.
- 7. Evaluation:** In order to facilitate long term evaluation and strengthening methods for engagement, there will be a defined, limited set of key data elements that the investigator team defines; they will report on the contribution of the patient informants to the modification of these elements and will report on their actions and changes they made or did not make in accordance with the patient recommendations.

# Hot off the Press!

## Additional Draft Standards

### Patient-Centeredness Work Group

**PROM(s) should be included in all PCOR with justification provided if not included.**

**Include global and context-specific PROMs in PCOR.**

- **Measurement properties of PROMs must be described including content validity, construct validity, reliability, sensitivity, and clinical meaningfulness of score changes in the target population including important subgroups. If key properties are not known, a plan for establishing these properties should be provided.**



- **Describe how the results of PRO measurement will be used to inform health care decision making.**

# Appendix

# Methodology Committee Awarded Contracts



*15 contracts have been awarded to date (of 48 submissions)*

Awarded RFP	Topic/Type	Contractor
Methods for Setting Priorities in Research (White Paper)	Topic Generation	1. Hayes Inc.
	Value of Information Analysis	2. NORC at The University of Chicago
	Value of Information Analysis	3. Duke University, Evidence-Based Practice Center
	Peer Review	4. University of Wisconsin, Medical College
Evidence for Eliciting the Patient's Perspective in PCOR	Stakeholder Interview	5. Oregon Health & Science University, The Center for Evidence-Based Policy
	Literature Review	6. Mayo Clinic, Knowledge and Evaluation Research Unit
	Supplement to Stakeholder Interviews	7. University of Maryland School of Pharmacy, Pharmaceutical Health Services Research Department
Review of Guidance Documents for Selected Methods in PCOR	Standards in the Prevention and Handling of Missing Data in Observational and Experimental Patient Centered Outcomes Research.	8. Johns Hopkins Bloomberg School of Public Health
	Standards in the Design and Selection of Patient-Reported Outcomes Measures (PROMs) for Use in Patient Centered Outcomes Research.	9. Northwestern University/UNC Chapel Hill
		10. Oxford Outcomes
	Standards in the Design, Conduct, and Evaluation of Adaptive Randomized Clinical Trials.	11. Berry Consultants
	Standards in the Design, Conduct, and Evaluation of Research Evaluating Diagnostic Testing Strategies for Patient Centered Outcomes Research.	12. Brown University
	Standards for Causal Inference Methods in Analyses of Data from Observational and Experimental Studies in Patient Centered Outcomes Research.	13. Division of Pharmacoepidemiology and Pharmacoeconomics, Department of Medicine, Brigham and Women's Hospital and Harvard Medical School
	Standards in the Conduct of Registry Studies for Patient Centered Outcomes Research.	14. Outcome Sciences, Inc. (A Quintiles Company)
	Networks or Distributed Data Networks in Patient Centered Outcomes Research.	15. University of California and San Diego

# Patient Centeredness Contractors



*The following institutions have been contracted by the Methodology Committee and are managed by the Patient Centeredness Workgroup*

## 1. Oregon Health & Science University, The Center for Evidence-Based Policy

- **Scope of Work:** Identify organization/individuals with expertise in eliciting patients' and individuals' perspectives and conduct qualitative interviews (Stakeholder Interviews)

## 2. Mayo Clinic, Knowledge and Evaluation Research Unit

- **Scope of Work:** Conduct a systematic review and environmental scan on the topic of incorporating the patient's voice into research (Literature Review)

## 3. University of Maryland, Pharmaceutical Health Services Research Department

- **Scope of Work:** Conduct a smaller scale project to supplement the primary contract award (Stakeholder Interviews), specifically to gain information about hard-to-reach populations

## 4. Northwestern University / UNC Chapel Hill

- **Scope of Work:** Produce background papers that propose and justify minimum methodologic standards in the following research methodology domain: *Standards in the Design and Selection of Patient-Reported Outcomes Measures (PROMs) for Use in Patient Centered Outcomes Research*

## 4. Oxford Outcomes

- **Scope of Work:** Produce background papers that propose and justify minimum methodologic standards in the following research methodology domain: *Standards in the Design and Selection of Patient-Reported Outcomes Measures (PROMs) for Use in Patient Centered Outcomes Research*

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

**PCORI-SOL-PCWG-001**

**Awardee: Mayo Clinic, Knowledge and Evaluation Research Unit**

**Solicitation Focus:** Methods for eliciting the patient's voice or perspective.

**Scope:** To conduct a structured and comprehensive review of the literature and environmental scan on the topic. This includes conducting an environmental scan of other relevant sources, as well as review processes employed to engage people in non-health care as well as health care activities.

### **Additional Requirements:**

- Include both U.S. and international sources.
- Describe the methods that have been effective at addressing the issues listed
- Describe the methods employed in synthesizing the applicants' findings
- Summarize identified best practices, recommendations, and knowledge gaps

### **Goal:**

- 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.

# RFP Scope of Work



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

### **Awardees:**

**(1) Oregon Health & Sciences University**

**(2) University of Maryland**

Scope: identify organization and individuals with expertise in eliciting patients' and individuals' perspectives and conduct qualitative interviews of these individuals, addressing the questions posed in the application.

### **Additional Requirements:**

- Interview non-health care (e.g. industry) organizations and individuals as well as health care sources.
- Include both U.S. and international experts as appropriate.
- Identify and interview key informants from the following groups: patients; caregivers; advocates/community organizers ; health care providers (including but not limited to physicians and nurses); payors (private and public); researchers, including those engaged in community participatory research; pharmaceutical and medical device industry representatives; government representatives/regulators (e.g., NIH, AHRQ, FDA, CMS); consumer advocacy organizations (inside and/or outside of health care); and industry/business (outside of health care).

### **Goal:**

- 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, and setting/timing and others.
- Report what approaches have been effective and why; and describe how these approaches can directly inform PCORI.

# RFP Scope of Work



## ***Review of Guidance Documents for Selected Methods in Patient Centered Outcomes Research*** **PCORI-SOL-RMWG-001**

### **Awardees:**

**(1) Northwestern University / UNC Chapel Hill**

**(2) Oxford Outcomes**

**Scope:** Background papers that propose and justify minimum methodologic standards in the following research methodology domains: **Standards in the Design and Selection of Patient-Reported Outcomes Measures (PROMs) for Use in Patient Centered Outcomes Research**

### **Details:**

- Propose minimum standards for the development and use of tools to assess patient reported outcomes.
- Review standards in qualitative and quantitative methods used to develop and select measures of the patient experience in experimental and observational clinical comparative effectiveness research.
- Examine the primary literature and guidance statements for recommended minimum standards, as well as the properties to be sought or assessed in PROMs that are 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. Examples of PROMs that adhere to the standards should be discussed.
- Include the potential methodological and logistical challenges of applying such standards in "real-world" or non-experimental settings.

### **Additional Requirements:**

- Complete a systematic review to include any currently existing methodology guidance statements and relevant literature,
- Provide a succinct summary of minimum standards for the conduct of research using the designated methodology, with illustrative examples of publications that demonstrate adherence to these standards, and
- Include an explanation for how/which proposed guidelines will help further the goals of research sponsored by PCORI with particular attention to patient-centeredness