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

## Hosted by the Patient-Centeredness Work Group
## of the Patient-Centered Outcomes Research Institute
## Methodology Committee

# Executive Summary

## March 6-7, 2012

## Baltimore, Maryland

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# Patient Centeredness Work Group Workshop –Day 1

## Introduction

The Patient Centeredness Work Group (PCWG) of the PCORI Methodology Committee held a workshop over the course of two days in Baltimore, MD to bring together contracted research teams, external invitees, and members of the PCORI Methodology Committee and Board of Governors.

The overarching goal of the workshop was to inform development of discrete methods standards for patient engagement in design, implementation, and dissemination of PCOR. Each of the five research teams with contracts overseen by the PCWG presented their findings as the basis for discussion with the larger group.

**Five research teams were contracted to complete the following work:**

- Mayo Clinic, Knowledge and Evaluation Research Unit —to review and synthesize what is known about eliciting the patient's voice and perspective in research by conducting a systematic review and environmental scan.
- Oregon Health & Science University, The Center for Evidence-Based Policy —to collect qualitative data from experts in consumer and patient engagement, clinicians, and patients on strategies for patient-centered outcomes research.
- University of Maryland, Pharmaceutical Health Services Research Department—to collect qualitative data on methods for engaging hard-to-reach patients in patient-centered outcomes research, from patients and clinicians.
- Oxford Outcomes Ltd, Patient Reported Outcomes —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 PCOR.
- Northwestern University, Department of Medical Social Sciences; University of North Carolina, Department of Health Policy and Management —to provide background and a proposal for minimum methodologic standards in the design and selection of Patient-Reported Outcomes Measures (PROMs) for use in PCOR.

## Background of Patient Centeredness Work Group

The Government Accountability Office (GAO) appointed 17 members to PCORI's Methodology Committee, which has organized itself into four sub committees, one of which is the Patient Centeredness Work Group.

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, and identifies important methodological gaps that need to be addressed.

## Presentation 1: Eliciting the Patient's Voice in PCOR (Literature Review)

*Mayo Clinic, Knowledge & Evaluation Resource Unit*

### Overview

With the ultimate goal of providing recommendations for methodological standards, Mayo Clinic conducted a systematic review of published biomedical literature and an environmental scan of unpublished literature and other information sources. Research questions posed were: Who are the relevant patients for engagement? How to identify and recruit them? How can they engage? How can their engagement result in changes in research design, conduct, analysis and dissemination?

### Engagement Framework

The research team found 34 studies that described a model that included steps for the process of patient engagement. These models converged into three frameworks: (1) common iterative steps for engagement; (2) engagement as a function of research stages—preparatory, execution, and translational; and (3) potential spectrum of patient engagement in research, which ranges from *passive* to *engaged* (subject, respondent, participant, stakeholder, consultant, partner, and researcher).

**Examples of Research Types**

- Interviews with parents of children with cerebral palsy to evaluate four trial designs.
- Mailed questionnaire to a stratified random sample of 4796 patients with diabetes to examine preference regarding trial design.
- Development of a conceptual framework for patient-reported outcomes for metastatic prostate cancer.
- From the environmental scan, patient-initiated study of patients with amyotrophic lateral sclerosis.
- A national advisory group in the UK to support involvement in public health and social care research.

**Methods of Engagement in the Literature**

The team found that most engagement took the form of self-selection out of a convenience sample, particularly among disease specific social networks. In Europe and Canada, there are organized networks designed for patient engagement.

**Limitations** include lack of comparative studies, poor indexing and reporting standards, publication bias, and examples of "tokenistic" engagement.

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**Mayo Clinic Recommendations**

✓ Engage patients and surrogates in all research phases (benefits outweigh the risks)

✓ Utilize a framework for engaging informants

✓ Select representatives that are similar to the community or population in which the study results are intended to apply

✓ Initiate patient and surrogate engagement as early as possible in the research project and as frequent as feasible

✓ Engage patients based on the research questions being asked and the overall aims of the research

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# Presentation 2: Best Practices for Patient Engagement in Research (Interviews)

*Oregon Health & Sciences University, Center for Evidence Based Policy*

**Overview**

With the goal of soliciting input from knowledgeable and experienced experts, the OHSU team accomplished the following: recruited and convened an 11 member advisory panel to advise the project work; identified 299 engagement experts through professional network environmental scan of the literature (not limited to health); contacted 128 of these experts and completed 87 interviews in approximately two months; and partnered with the University Network of Collaborative Governance to facilitate 12 focus groups with patients across the country in four weeks.

**Engagement Framework**

The OHSU team developed a framework for illustrating the points of patient-centered engagement, paired with a key to explain the strength of evidence for each recommendation.

## Key Themes of Patient Engagement

➢ **Respect**
Commit to meaningful engagement at multiple points, treat patients as equal colleagues (reflecting the idea that everyone possesses expertise), and develop trust.

➢ **Communication**
Actively communicate in a variety of contexts, develop relationships to foster two-way knowledge exchange, keep the patient perspective in mind, and be flexible and transparent.

➢ **Dedicated Resources**
Patient engagement is resource and time intensive. Devote funding and financial support in terms of incentives, provide reimbursements for patients and support for researchers. Develop transparent processes throughout in terms of how those processes will be operationalized. Utilize facilitation skills.

## Presentation 3: Integrating patients' voices in study design elements with a focus on hard-to-reach populations (Interviews)

*University of Maryland, Pharmaceutical Health Services Research Department*

### Overview

With the focus on hard-to-reach patients, the University of Maryland team engaged six categories of patients in focus groups along with focus groups of clinicians and with caregivers

Study participants identified hard-to-reach patients based on impairments, illnesses, age, where they live, other social indicators, and what they do.

### Key Themes and Findings

**What are the practical methods for engaging hard-to-reach patients?**

- ➤ Partner with individuals, groups, associations, networks
- ➤ Meet in places where people are —i.e., public spaces, community-specific spaces, semi-private spaces (clinics and churches), and private spaces (housing and shelters)
- ➤ Utilize Media (each with varying ranges of geographic reach) such as billboards, public

transportation advertising, flyers in billing statements, radio, TV, movie theatres, and social media

- ➤ Attend to components of building and maintaining trust: Pre-engage (to understand the community); Relate (be genuinely willing to partner); Communicate (simple questions/avoid making judgments); Be there (keep "coming back" and interacting with participants)
- ➤ Ensure that patients understand the research process—use plain language, provide information in chunks, ask open-ended questions, be transparent, and develop simple consent forms

### Lessons Learned

- Trust is the key overarching element for PCOR
- 'Outcomes' is an unfamiliar term for patients and some clinicians
- Framing and phrasing of questions is critical for eliciting patients' views
- Community-based PCOR requires flexibility, compromise and time
- Patients do not like being asked repeated questions

## Group Discussion: Comparing standards across research teams

### Introduction

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. With the goal of improving the value and quality of research, the Methodology Committee is developing standards for

that would ideally guide each phase in PCOR. The first step is to identify the minimum standards needed to conduct PCOR.

**Key Questions**

- How do we know we are setting the right minimum standards for conducting PCOR and engaging people in a meaningful way?
- Can we achieve a standard that is actionable, feasible, measurable, and informative?
- Should concepts, such as trust, be thought of as a standard? If so, how do we operationalize? Is this more of an outcome or a principle?
- How do we evaluate a study to ensure patient engagement has been incorporated? What criteria will ensure that the study includes a patient-centered outcome?
- What prevents researchers from engaging patients effectively?
- How do we translate meaningful and valid research results into clinical practice?
- What is the impact of patient engagement on outcomes?

**Considerations for PCOR**

- Recruiting, identifying, and selecting patients
- How patients self-identify, where they are coming from, and how to build trust
- Early and frequent engagement
- Give back to patient, community, etc.
- Cultural sensitivity
- Privacy
- Training (for patients and researchers), co-learning, and facilitation
- No one-size-fits-all method/match the method with the purpose of involvement
- Include engagement efforts in research budgets

- Patient burden –i.e., evaluate impact on patients of engagement (positive or negative)
- Researcher burden
- Differentiate between patients in a disease area offering their thoughts about study design vs. the participants in a study

**Patient Engagement Challenges**

- Impediments to 'two-way learning'
- Dissemination
- Impact on the greater population
- Bias
- Communication and transparency with study participants and affected communities
- Level of specificity of the standards is limited due to a lack of knowledge
- Limited evidence
- Principal Investigator acceptance/understanding
- Targeting the population that needs to be targeted
- Ensure that what they are asking of participants is reasonable

**Key elements of the reporting standards for PCOR**

- Description of plan/approach
- Description of the process, method, and outcomes
- How were informants selected? How was their feedback obtained? How were they engaged? What was the impact of research and research methodology on the lives of the patients engaged?

**First steps to evaluating patient engagement**

- Outline a process model for conducting PCOR
- Develop principles and indicators of engagements
- Define patient engagement and clarify criteria
- Involve a patient engagement expert
- Recognize that successful patient engagement doesn't necessarily mean changing a study design

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**Summary of Day 1**

The long-term goal of PCOR is to improve healthcare decision-making at the point of care. We accept patient engagement in research as an important principle, even though standards for PCOR may lack strength of evidence at this time. Our approach will be to begin with minimum standards and raise standards as time goes on. There's a limit to how specific standards can be due to lack of evidence.

➢ **Principles** –Trust, Transparency, Co-learning, Honesty, Reciprocal Relationships
➢ **Ideal Practice** –Engage <u>early</u> and <u>often</u>, provide training for patients and researchers, index properly, evaluate over time, and give back
➢ **Critical Question** – How will we know patient engagement has been successful?

# Patient Centeredness Work Group Workshop –Day 2

## Presentation 4: Standards for the Development and Selection of Patient Reported Outcome Measures (PROMs) for Use in Patient-Centered Outcomes Research

*Oxford Outcomes Ltd, Patient Reported Outcomes*

**Overview**

The Oxford Outcomes team identified themes to be considered for minimum standards by gathering guidance documents from the literature.

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**Oxford Outcomes Recommended Standards**

✓ Consideration of Patient Burden (within an entire study design)

✓ Estimating and Reporting Reliability (recommend threshold guideline 0.7-0.9 range)

✓ Staff Training

✓ Choosing an Appropriate Recall Period

✓ Selecting a PROM that is concept driven and meaningful to patients

✓ Interpretation of Meaningful Change

✓ Establishing and Assessing Content Validity

✓ Sampling (recruitment of diverse, fully representative samples)

✓ Estimating and reporting construct validity to detect change

✓ Modification of an existing PROM (patient involvement to ensure relevancy)

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**Other issues for consideration include:**

1) response shift; 2) confirmation of measurement properties; 3) interpretation of profile measures; 4) development of short forms; 5) lessons learned from health technology appraisals in other countries; 6) low literacy and non-English speakers; 7) proxy and caregiver reported outcomes; 8) patient involvement beyond the PROM; and 9) communication of PROM data to patients and clinicians.

**Key Questions**

- How does selection and use of Patient Reported Outcomes (PROs) differ in the context of PCOR compared to other contexts?
- Should PROs be considered for use in all PCOR?
- Do we need allowance for surrogate or others to report?
- Should content validity be in a broader population?
- Should we think about saturation more broadly than at the single study level?
- Should there be global measures/instruments used across all PCOR?
- Where do we want to move in terms of best practices (beyond minimum standards)?

# Presentation 5: Guidance on Minimum Standards for the Design and Selection of Patient-Reported Outcomes Measures for Use in Patient-Centered Outcomes Research

***Northwestern University & the University of North Carolina at Chapel Hill***

### Overview

The Northwestern University team conducted a comprehensive literature review and the UNC Chapel Hill team designed a survey for members of the International Society for Quality of Life (ISOQOL) to gain consensus on draft recommendations. The project team convened the ISOQOL Scientific Advisory Task Force to guide the work.

### ISOQOL Survey Outcome

The survey was received by approximately 500 members; approximately 100 responses were received within the nine-day turnaround time. The team

> **Northwestern University/UNC Chapel Hill Recommended Standards for PRO Measures**
> ✓ Conceptual and Measurement Model
> ✓ Reliability (recommended goal of 0.7 or above)
> ✓ Content Validity (evidence that patients and/or experts consider the content valid)
> ✓ Construct Validity
> ✓ Interpretability of scores
> ✓ Translations
> ✓ Patient and Administrative Burden (grade 6 level OR adapted for the context of the proposed application

instructed the respondents to judge what constitutes "minimum" standards for design and selection of PROs. If more than 50% of respondents indicated a standard is required, the team nominated the standard. If less than 50% indicated a standard was required, the team nominated this for a best practice.

## Discussion

### Overview

The group discussed the differences of PROM use (and the proposed standards) for PCOR relative to other study types, highlighted the methodologic and logistical challenges in administering PROs, and deliberated about the priorities.

### Considerations for PCOR

- The goal is to inform clinical decision making.
- Patients should be the gold standard for reporting.
- How can we mitigate measurement error when a study uses different modes to collect data (phone, computer, paper, etc.)?
- There is a tension between standardization vs. customization. Should we look towards standardization of data elements but also measures being used to capture those data elements?

- How much can we ask of patients particularly those with multiple co-morbidities who might be asked to complete measures at multiple places within a health system? How can we coordinate the effort across clinics so you get good data and are not overwhelming the patients with duplicate requests?
- Incorporate training as a part of the standards
- Consider how electronic data systems might ease patient burden
- Focus on broad quality of life outcomes
- Exit interviews/questionnaires with patients. What is important to patients may not be important to their caregivers
- What is the difference being measured from the patient's point of view? Patients are going to be concerned about privacy and the modes used

**Key Considerations when Prioritizing PROs**

- Conceptual framework or measurement model
- Evidence for Content Validity
- Internal consistency
- Interoperability
- Documentation
- Transparency
- Responsiveness
- Burden

**Principles vs. standards**

The group considered standards suggested for nomination, reviewed standards for which consensus did not emerge, and identified principles for conducting PCOR.

**Feedback on Proposed Standards**
- Patient-centered does not always mean patient reported
- Clinical outcomes are not always patient-centered
- Objective measures vs. symptoms
- Link patient-centeredness to the decision-making process
- Data must be meaningful and interpretable to patients
- *Meaningfulness* needs to be defined
- Dissemination and implementation plans are crucial
- Primary prevention and self-care should be considered
- Caregiver burden should be addressed
- Researchers need training on how to engage (i.e., complete online module in understanding PCOR)
- Do research participants understand what the research is about?
- Evaluation of the engagement
- Resource intensive nature

| Comparing standards across subjects | | |
|---|---|---|
| | **Oxford Outcomes** | **Northwestern University/ UNC Chapel Hill** |
| Conceptual Framework/ Msmt Model | X | X |
| Reliability <br> • Internal consistency <br> • Test-retest | X | X |
| Validity <br> • Content <br> • Construct | X | X |
| Responsiveness | X | X |
| Sensitivity | X | X |
| Interpretability | X | X |
| Recall Period | X | X |
| Burden | X | X |
| Training Requirements | X | X |
| Translation | X | X |
| Modification | X | |
| Sampling | X | |
| Multi-Mode Admin | | |

**Principles**
- ★ 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
- ★ Proper indexing –permit evaluation
- ★ Evaluation of comprehension over time

# Attendees

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