



PCORI Evaluation Group

Second Meeting
January 24, 2014

Patient-Centered Outcomes Research Institute

Objective for Today: Discuss a Priority Evaluation Question

Some Evaluation Questions Are Rising to the Top:

- About key aspects of “The PCORI Way”
- Important to the field of PCOR generally
- Of interest to a wide range of stakeholders
- Appropriate for PCORI to tackle
- PCORI is in a good/the best position to answer them
- We can get started on them now and have some results before a sizeable number of the studies we fund are complete

Agenda for Today

- 🌱 Frame and Discuss the Question(s):
 - What is the effect of Patient-Centeredness and Engagement on Recruitment and Retention?
- 🌱 Review and Discuss Metrics/Indicators
 - Patient-Centeredness (predictor)
 - Engagement (predictor)
 - Recruitment and Retention (endpoints)
- 🌱 If Time Allows...
 - Discuss Methods
 - Discuss Data Sources

Evaluation Framework: Our Questions and How We Will Answer Them

Evaluation Questions	Metrics/Indicators	Methods	Sources
What do PCOR and PCORI stakeholders want/need to know?	For each question, what are we measuring and how will we measure it?	What approach will we take to answering this question?	From where will we get the data to answer this question?



Why would we do this evaluation – what is the objective?
 How would it fit into the bigger picture – our overall framework?
 What would we do with the results of this evaluation?

Evaluation Framework: Tackling One Question (Set of Questions)

Evaluation Question	Metrics/Indicators	Methods	Sources
What is the effect of Patient-Centeredness & Engagement on Recruitment & Retention?	How will we measure: <ul style="list-style-type: none">• Patient-Centeredness?• Engagement?• Recruitment & Retention?	What approach will we take to answering this question?	From where will we get the data to answer this question?

Getting More Specific, Example: How Do Patient-Centeredness and Engagement Affect Recruitment and Retention?

PREDICTORS

- Patient-Centeredness
 - Endpoints/Outcomes
 - Methods (e.g., Patient-Reported)
 - ???
- Engagement in:
 - Study Team
 - Question Development
 - Study Design
 - Recruitment
 - Data Collection
 - ???

ENDPOINTS

- Pace of Recruitment
- Eligibility Rate
- Pace of Enrollment
- Completion of Recruitment
- Retention Rate
- Attainment of Sample Size
- Follow-up
- Missing Data
- ???

Metrics/Indicators

These slides were in the Appendix for the last meeting, but we didn't have time to discuss them. They show what we currently have available for developing some of the key metrics:

- Characterizing Patient-Centeredness
- Characterizing Engagement
- Recruitment (and Retention)

Characterizing Patient-Centeredness: From our definition of PCOR

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options.

“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”

“What are my options and what are the potential benefits and harms of those options?”

“What can I do to improve the outcomes that are most important to me?”

“How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”

Characterizing Patient-Centeredness: From our definition of PCOR

- Does the proposed research compare the effects of treatment options that matter to patients?
 - Are these realistic choices faced by patients or other decision-makers?
- Does the research focus on outcomes of interest to patients and their caregivers?
 - Outcomes of interest might include: health, health-related quality of life, functioning, symptoms, safety from medical harm, survival and satisfaction with care.

Characterizing Patient-Centeredness: From our Methodology Standards

- PC-1: *Engage people* representing the population of interest and other relevant stakeholders in ways that are appropriate and necessary in a given research context
- PC-2: Identify, select, recruit, and retain ***study participants representative of the spectrum*** of the population of interest and ensure that data are collected thoroughly and systematically from all study participants
- PC-3: Use ***patient-reported outcomes*** when patients or people at risk of a condition are the best source of information
- PC-4: Support ***dissemination and implementation*** of study results

Characterizing Patient-Centeredness: From our Methodology Standards

RQ-3: Identify specific populations and health decision(s) affected by the research

To produce information that is meaningful and useful to people when making specific health decisions, research proposals and protocols should describe:

- 1) the specific health decision the research is intended to inform;
- 2) the specific population for whom the health decision is pertinent;
- 3) how study results will inform the health decision.

Characterizing Engagement in PCOR: From our Engagement Rubric

On Three Dimensions

Who is engaged?

- List of stakeholder communities from Patient/Consumer to Policymaker

In what are they engaged?

- List of steps from Topic Generation through Dissemination

How/In what manner are they engaged?

- Ranges from recipients of Information to partners in Direction

Characterizing Engagement: Who? – Stakeholder Communities

- **Patient/Consumer** – someone with a condition or at risk of a condition (unaffiliated individual)
- **Caregiver/ Family Member of Patient** – unpaid caregivers to someone who is affected by an illness, unaffiliated individual
- **Patient/Consumer/Caregiver/ Advocacy Organizations** – those who serve in a patient advocacy role on behalf of an individual or an organization
- **Clinician** – Nurse, Physician, etc., or an organization that represents clinicians
- **Clinic/ Hospital/ Health System Representative** – Federally Qualified Health Center (FQHC), Rural Health Clinic, etc., or an organization that represents hospitals/health systems
- **Purchaser** – Employer or an organization that represents purchasers
- **Payer** – Health Insurer, Medicaid, etc., or an organization that represents payers
- **Industry Representative** – Device or Pharmaceutical Manufacturer, or an organization that represents industry
- **Policy Maker** – State Legislator, Executive Agency Employee, etc., or an organization that represents policy makers

Characterizing Engagement: In What? – Phases of Research

- ④ Topic solicitation/Agenda setting
- ④ Question development/framing
- ④ Study design
 - Defining comparators
 - Defining outcomes
 - Selection or development of intervention
- ④ Recruitment/ Data collection
- ④ Data analysis
- ④ Results review/Interpretation/Translation
- ④ Dissemination

Characterizing Engagement: How? – Manner/Level of Engagement

- 🌐 **Information** – Researcher(s) describe decisions to patients and other stakeholder partners after decisions are made.
- 🌐 **Consultation** – Patients and other stakeholders provide feedback to researchers that can inform decision-making. Consultation allows the researcher to obtain views without necessarily being committed to act on them.
- 🌐 **Collaboration** – Researchers, patients and other stakeholders have an active partnership. Patients and other stakeholders work directly with the researcher to ensure that their perspectives are consistently understood and incorporated in decision making, and have greater ownership of the project.
- 🌐 **Patient/Stakeholder Direction** – also known as “user control,” patients and other stakeholder(s) are empowered to have control over the research process and the final decision-making.

Questions about Measuring Engagement: Trade-offs between Burden/Feasibility and Explanatory Power

- Frequency of Assessment?
 - Recall bias
 - Burden to respondents
 - Dynamic nature of engagement
- Reporters (scientists, patient partners, other types of stakeholder partners, etc.)?
 - How many of each?
 - Burden in completion and administration
 - Heterogeneity in the kinds of partners across studies and their involvement
 - Ability of one patient to speak for the perceptions of many, etc.
- Assessment of engagement of patients vs. other stakeholders
 - Possible/appropriate to evaluate separately?
 - Assessment burden

Recruitment and Retention

- What aspects of recruitment/retention best serve as a proxy for what we are most interested in – Trust, Relevance, etc. of the research?
- Which aspects are most important to the quality, usefulness, impact of the resulting information?
- What else do we need to think about?

Measuring Recruitment and Retention: From Our Project Progress Reports

Target number	Projected Completion Date	Actual Completion Date	Status (include one): <ul style="list-style-type: none"> • Completed as proposed in contract • Not yet initiated • In progress, as proposed in contract • Delayed • Dropped/terminated
Start of recruitment			
25% of recruitment			
50% of recruitment			
75% of recruitment			
Completion of recruitment			

If time allows...

- 🌐 Study Design/Methods to Consider
- 🌐 Data Needed and Sources
 - Internal
 - External