



# Striving for Usefulness

Advisory Panels General Session  
January 13, 2014

Patient-Centered Outcomes Research Institute

# PCORI's Three Goals: *Increase Information, Speed Implementation, Influence Research*

Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions

Speed the implementation and use of patient-centered outcomes research evidence

Influence clinical and healthcare research funded by others to be more patient-centered

# Today's Focus Is on a Key Aspect of Goal One: Information *Useful* for Health Decisions

Substantially increase  
the *quantity, quality,*  
and *timeliness* of  
*useful, trustworthy*  
information available *to*  
*support health*  
*decisions*

# How Usefulness Criteria Fit In

Characteristics of the Information We Aim to Produce and How We Plan to Ensure Them			
Quality	Timely	Useful	Trustworthy
Methodology Standards	Engagement	<i>Usefulness Criteria</i>	Methodology Standards
Merit Review Criteria	Prioritization Processes	Patient-Centeredness	Transparency
Portfolio Management	Merit Review Criteria	Engagement	Conflict-of-Interest Safeguards
Engagement	Portfolio Management	Prioritization Processes	Engagement
		Merit Review Criteria	

# Developing criteria to help us identify studies that will yield information *useful* for health decisions

- Informed by our Methodology Standards
- Based on previous work in the field
  - Criteria vary by scope and perspective
- In collaboration with others
  - Effort led by National Health Council
- In conjunction with our PCORI Evaluation Group
- With input from stakeholders
  - Such as today's meeting and follow-up

# Assessing Usefulness: Apply Criteria, Ask People, Monitor Use

How do the studies we fund measure on usefulness criteria?

Would/Do people find information from PCORI studies useful?

Is the information from PCORI studies being used? By whom? How?

Refine Usefulness Criteria and Incorporate into Funding Decisions

# How We Propose to Assess the Potential Usefulness of the Information from the Studies We Fund

[www.pcori.org/usefulness](http://www.pcori.org/usefulness)

- Question arises from people who would use the information
- People who would use the information have helped to shape/vetted the question
- People who would use the information have been identified
- Specific uses for the information have been identified
- Results could provide a clear answer to the question
- Results could help to choose among relevant options
- Results could be acted upon by relevant decision makers
- Results are feasible for people outside of the study setting to apply
- Results could be tailored to individuals or subgroups
- Results could be scaled-up/spread beyond the study setting

# Questions for You to Consider

[www.pcori.org/usefulness](http://www.pcori.org/usefulness)

- 🌐 What would make clinical comparative effectiveness information **useful** for **your** decision making?
  - Assume that standards for quality and trustworthiness are met and also that the information will be effectively translated for/communicated to you
- 🌐 Do these criteria seem like a reasonable starting point for characterizing studies with the potential to yield information that **you** would find useful?
- 🌐 How well do these criteria fit for the research priority that is the focus of your PCORI Advisory Panel? Are there issues specific to each area of research that we haven't captured?



# Appendix – Additional Background Materials

- Proposed Usefulness Criteria
- Patient-Centered Outcomes Research
- Patient-Centeredness
- Engagement

Proposed Usefulness Criterion (12/13/13 Draft)	Application for Specific Purpose/Type of Decision			
	Funding Decision	Dissemination Decision	User Decision	Other?
Question Comes from Potential Users	✓	✓		
Potential Users Identified	✓	✓		
Uses Identified for the Results <ul style="list-style-type: none"> <li>• Systematic Review</li> <li>• Patient Materials</li> <li>• Clinical Guidelines/Protocols</li> <li>• Decision Tools</li> <li>• Programs or Policies</li> <li>• Curriculum</li> </ul>	✓	✓		
Results Can Be/Are Responsive to the/My Question	✓	✓	✓	
Results Can Be/Are Relevant	✓	✓	✓	
Results Can Be/Are Actionable/Feasible	✓	✓	✓	
Results Can Be Tailored to Subgroups/Us	✓	✓	✓	
Results Can Be Tailored to Individuals/Me	✓	✓	✓	
Results Can Be Scaled Beyond the Study Setting	✓	✓	✓	
Results Can Be Spread Beyond the Study Setting	✓	✓	✓	
What Else?				

# Methodology Standards – Useful

## **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 Patient-Centered Outcomes Research

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

# Characterizing Patient-Centeredness

- 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.

# Methodology Standards – Patient-Centeredness

- 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 Engagement in PCOR

## On Three Dimensions:

- 🌐 Who is engaged?
  - List of stakeholder communities from Patient/Consumer to Policymaker
- 🌐 In what are they engaged?
  - List of research phases from Topic Generation through Dissemination
- 🌐 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: What – Research Phases

- 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 – Levels 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.