Welcome PCORI Evaluation Group!

First Meeting
December 13, 2013

Patient-Centered Outcomes Research Institute
Objective for Today: Development of Our Evaluation Framework

Evaluation Questions
- Identify and Frame
- Prioritize
- Develop a set of evaluation questions for us to pursue in subsequent meetings to determine appropriate methods, approaches, and mechanisms

Specify PCORI’s Goals
- Develop Measures
  - Usefulness Criteria
  - Uptake and Implementation
  - Influence
Agenda for Today

- Welcome and Introductions
- Overview of PCORI Logic Model
- Identify Evaluation Questions
- Discuss Measuring PCORI’s Goals
  - Usefulness
  - Use
  - Influence
- Prioritize Evaluation Questions
- Discuss Approaches to Evaluating High Priorities
Agenda Item: Welcome and Introductions
About This Task Force

- Advisory Capacity
- Relation to PCORI Board, MC, Other Panels, etc.
- Transparency
- Conflict-of-Interest
- Non-Disclosure
The PCORI Evaluation Group

From Our Board of Governors

- **Gail Hunt**
  National Alliance for Caregiving
  President and CEO

- **Bob Jesse**
  Department of Veterans Affairs
  Principal Deputy Under Secretary for Health

- **Bob Zwolak**
  Dartmouth-Hitchcock Medical Center, Vascular Surgeon
  Dartmouth Medical School, Professor of Surgery
  Veterans Administration Medical Center
  Chief of Surgery and Director of the Non-invasive Vascular Laboratory

From Our Methodology Committee

- **Naomi Aronson**
  Blue Cross and Blue Shield
  Executive Director, Association Technology Evaluation Center

- **Mike Lauer**
  National Heart, Lung, and Blood Institute
  Director, Division of Cardiovascular Sciences

- **Robin Newhouse**
  University of Maryland School of Nursing
  Chair and Associate Professor, Organizational Systems and Adult Health
  PCORI Methodology Committee Chair

External

- **Kimberly Bailey**
  Families USA
  Research Director and Director of Health System Reform
  PCORI Patient Engagement Advisory Panel Member

- **Claire Brindis**
  University of California, San Francisco
  Caldwell B. Esselstyn Chair in Health Policy Director,
  Philip R. Lee Institute for Health Policy Studies
  Professor of Pediatrics and Health Policy

- **Jack Fowler**
  University of Massachusetts Boston
  Senior Research Fellow
  Center for Survey Research

- **Beverly Parsons**
  InSites
  Executive Director
  American Evaluation Association
  President-elect
PCORI Staff

On the PEG

- Laura Forsythe (Science)
- Lori Frank (Science)
- Michele Orza (Executive Director’s Office)
- Suzanne Schrandt (Engagement)

Supporting the PEG

- Olumide Adeleye (Science)
- Emma Djabali (Science)
- Pavan Jagannathan (Executive Director’s Office)
- Katie Jones (Operations)
- Kristen Konopka (Engagement)
- Katie Rader (Science)
- Victoria Szydlowski (Science)
Our Evaluation Activities

Our objective is to produce information that is useful to us and others to improve our work and advance the science and practice of Patient-Centered Outcomes Research.

We plan to conduct this work in a manner consistent with our values and methods – rigorous, focused on and engaging of stakeholders, efficient.

We are committed to sharing and using this information.
## Evaluation Framework:
### Our Questions and How We Will Answer Them

<table>
<thead>
<tr>
<th>Evaluation Questions</th>
<th>Metrics/Indicators</th>
<th>Methods</th>
<th>Sources</th>
</tr>
</thead>
<tbody>
<tr>
<td>What do PCOR and PCORI stakeholders want/need to know?</td>
<td>For each question, what are we measuring and how will we measure it?</td>
<td>What approach will we take to answering this question?</td>
<td>From where will we get the data to answer this question?</td>
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</tbody>
</table>
Developing Our Evaluation Framework
Guidance from PCORI's Methodology Report

PCORI’s Translation Framework for choosing research designs/methods:

- Keep the question and the methodology separate
- Focus on clarifying tradeoffs
- Place individual studies in the context of a program
- Have the choice of study design take into account state-of-the-art methodology
Agenda Item:
Overview of PCORI Logic Model
<table>
<thead>
<tr>
<th></th>
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<tbody>
<tr>
<td>Congressional Oversight and Evaluation</td>
<td>Yearly GAO Financial Audits</td>
<td>First GAO 5-year Review</td>
<td>GAO 8-year Review</td>
<td>Second GAO 5-year Review</td>
</tr>
<tr>
<td>PCORI Emphasis or Theme</td>
<td>Building</td>
<td>Implementing</td>
<td>Implementing</td>
<td>Results</td>
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<td></td>
<td></td>
<td>Results</td>
<td>Results</td>
<td>Impact</td>
</tr>
<tr>
<td>Primary Evaluation Metrics</td>
<td>Inputs</td>
<td>Process</td>
<td>Outputs</td>
<td>Outcomes</td>
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<td></td>
<td>Process</td>
<td>Outputs</td>
<td>Outcomes</td>
<td>Impact</td>
</tr>
<tr>
<td>Key Words from GAO Review Mandate in Our Legislation</td>
<td>Processes established</td>
<td>Overall effectiveness of activities</td>
<td></td>
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<tr>
<td></td>
<td>Research priorities and projects</td>
<td>Use by health care decision-makers</td>
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<td></td>
<td>Objective and credible information</td>
<td>Reducing practice variation and disparities</td>
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<td></td>
<td>Transparent process</td>
<td>Effect on innovation and health economy</td>
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<td></td>
<td>Dissemination and training activities</td>
<td>Use by public and private payers</td>
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<td>Data networks</td>
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Congressional inquiries may occur at any time.
Our Strategic Framework – Mission/Vision

IMPACT

Why We Do It
(Mission/Vision)

Better Informed Health Decisions

Improved Health Outcomes

Better Health Care
Our Strategic Framework – Goals

**GOALS**

What We Accomplish (Outcomes)

- Increase Information
- Speed Implementation
- Influence Research

**IMPACT**

Why We Do It

- Better Informed Health Decisions
- Better Health Care
- Improved Health Outcomes

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Our Strategic Framework – Strategic Imperatives

<table>
<thead>
<tr>
<th>STRATEGIC IMPERATIVES</th>
<th>GOALS</th>
<th>IMPACT</th>
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</thead>
<tbody>
<tr>
<td>Engagement</td>
<td>What We Accomplish</td>
<td>Why We Do It</td>
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<tr>
<td>Methods</td>
<td>Increase Information</td>
<td>Better Informed Health Decisions</td>
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<tr>
<td>Research</td>
<td>Speed Implementation</td>
<td>Better Health Care</td>
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<tr>
<td>Dissemination</td>
<td>Influence Research</td>
<td>Improved Health Outcomes</td>
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<td>Infrastructure</td>
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Our Five Strategic Imperatives

To increase information, speed implementation, and influence research, we:

- **Engage** patients, caregivers, and all other stakeholders in our entire research process from topic generation to dissemination and implementation of results.

- Develop and promote rigorous Patient-Centered Outcomes Research **methods**, standards, and best practices.

- Fund a comprehensive agenda of high quality Patient-Centered Outcomes Research **Research** and evaluate its impact.

- **Disseminate** Patient-Centered Outcomes Research to all stakeholders and support its uptake and implementation.

- Promote and facilitate the development of a sustainable **infrastructure** for conducting patient-centered outcomes research.
Our Strategic Framework/High-level Logic Model

<table>
<thead>
<tr>
<th>STRATEGIC IMPERATIVES</th>
<th>OUTPUTS</th>
<th>GOALS</th>
<th>IMPACT</th>
</tr>
</thead>
<tbody>
<tr>
<td>How We Create</td>
<td>What We Create</td>
<td>What We Accomplish</td>
<td>Why We Do It</td>
</tr>
<tr>
<td>Engagement</td>
<td>Skilled Patient-Centered Outcomes Research Community</td>
<td>Increase Information</td>
<td>Better Informed Health Decisions</td>
</tr>
<tr>
<td>Methods</td>
<td>Patient-Centered Outcomes Research Methods</td>
<td>Speed Implementation</td>
<td>Better Health Care</td>
</tr>
<tr>
<td>Research</td>
<td>Portfolio of Patient-Centered Outcomes Research Studies</td>
<td>Influence Research</td>
<td>Improved Health Outcomes</td>
</tr>
<tr>
<td>Dissemination</td>
<td>Communication and Dissemination Activities</td>
<td></td>
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<tr>
<td>Infrastructure</td>
<td>Patient-Centered Research Networks</td>
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</table>
2014 RESEARCH ACTIVITIES

EXPECTED OUTPUTS

GOALS

Strategic Priority: Fund High Impact Research

- Continue refining topic generation, prioritization, and selection processes
- Continue refining merit review process
- Fund multiple cycles of research via broad and targeted PFAs
- Increase funding for focused and targeted topics

Skilled PCOR Community

- Expanded range of funders involved in PCOR

PCOR Methods

- Enhanced methods for:
  - Research prioritization
  - Merit review

Portfolio of PCOR Studies

- Agenda of high priority topics
- Portfolio of studies that are:
  - High impact
  - Useful
  - On-track

Strategic Priority: Carefully Manage Research Portfolio

- Continue refining funding application and contracting processes
- Continue implementing portfolio planning, management, and evaluation

Strategic Priority: Partner With Other Funders

- Co-fund and co-sponsor studies with AHRQ, NIH, VA, and others

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

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

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

Patient-Centered Outcomes Research Institute
The Big Questions

Does “Research Done Differently” make a difference? Is it worth it?
- In the usefulness of information? (First Goal)
- In the use of information? (Second Goal)
- In how others conduct research? (Third Goal)
- In health outcomes? (Impact)

What are the effects of “the PCORI way” on the quality of research and usefulness of the information that results? Is it worth it?
- Patient-centeredness
- Engagement
- Methodology Standards
- Active Portfolio Management (Prioritization, Merit Review, Oversight)
- Communication and Dissemination
- Infrastructure Building – The National Patient-Centered Clinical Research Network

If it works, what’s the best way to do it? For example, Engagement:
- Who needs to be engaged?
- In what should they be engaged?
- How best to engage them?
Agenda Item: Identify Evaluation Questions

Choose a “hat”: patient, caregiver, clinician, administrator, Congressperson, insurer, drug or device developer, payer, policymaker, etc.

With your hat on, think about: In 5 or 10 years from now, what will you want to know about Patient-Centered Outcomes Research and the PCORI way of doing it?

In your mind, to what do you compare PCOR and PCORI?
Prioritize Evaluation Questions

Right for PCORI
- Uniqueness
- Appropriateness
- Capability

Potential to Impact PCORI’s Work and the Field

Collaborate
Lead
Monitor
Coordinate
Facilitate
Fund

Low
High

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Please peruse the questions on the posters and think about where you want to place your stickers!
Agenda Item:
Discuss Measuring PCORI’s Goals
Goal 1: Useful Information

Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions.
Goal 1: Useful Information

First, we are developing Criteria for Usefulness
- Based on previous work in the field
- In collaboration with others
- With input from stakeholders

Criteria are intended to capture features of studies such as whether:
- Study question came from potential users
- Potential uses have been identified
- Results would have potential to be tailored to different individuals, subgroups, settings
Assessing Usefulness: Apply Criteria, Ask Users, Monitor Use

How do the studies we fund measure on usefulness criteria?

Do people find information from PCORI studies useful?

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

Refine Criteria and Incorporate into Funding Decisions
Questions to Consider

- Do these criteria reflect the perspectives of most or all users?

- Do these criteria seem like a reasonable starting point?
  - Are there some that should be dropped?
  - Any that should be added?

- How should PCORI apply these criteria?

- How could PCORI validate these criteria?
## How Usefulness Criteria Fit In

### Characteristics of the Information We Produce and How We Ensure Them

<table>
<thead>
<tr>
<th>Quality</th>
<th>Timely</th>
<th>Useful</th>
<th>Trustworthy</th>
</tr>
</thead>
<tbody>
<tr>
<td>Methodology Standards</td>
<td>Engagement</td>
<td><strong>Usefulness Criteria</strong></td>
<td>Methodology Standards</td>
</tr>
<tr>
<td>Merit Review Criteria</td>
<td>Prioritization Criteria</td>
<td>Patient-Centeredness</td>
<td>Transparency</td>
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<tr>
<td>Portfolio Management</td>
<td>Merit Review Criteria</td>
<td>Engagement</td>
<td>Conflict-of-Interest Safeguards</td>
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<td></td>
<td>Portfolio Management</td>
<td>Prioritization Criteria</td>
<td>Engagement</td>
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<td></td>
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<td>Merit Review Criteria</td>
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<table>
<thead>
<tr>
<th>Proposed Usefulness Criterion</th>
<th>Application for Specific Purpose/Type of Decision</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Funding Decision</td>
</tr>
<tr>
<td>Question Comes from Potential Users</td>
<td>✔</td>
</tr>
<tr>
<td>Potential Users Identified</td>
<td>✔</td>
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<tr>
<td>Receptors Identified for the Results</td>
<td>✔</td>
</tr>
<tr>
<td>- Systematic Review</td>
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<tr>
<td>- Patient Materials</td>
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<tr>
<td>- Clinical Guidelines/Protocols</td>
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<tr>
<td>- Decision Tools</td>
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<tr>
<td>- Programs or Policies</td>
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</tr>
<tr>
<td>Results Can Be/Are Responsive to the/My Question</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be/Are Relevant</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be/Are Actionable</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be Tailored to Subgroups/Us</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be Tailored to Individuals/Me</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be Scaled Beyond the Study Setting</td>
<td>✔</td>
</tr>
<tr>
<td>Results Can Be Spread Beyond the Study Setting</td>
<td>✔</td>
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<tr>
<td>What Else?</td>
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</table>
Goal 2: Use of PCORI Information

Speed the implementation and use of patient-centered outcomes research evidence
Possible Indicators of Clinical Uptake of PCORI Findings

<table>
<thead>
<tr>
<th>Cited in subsequent Research*</th>
<th>Manuscript bookmarked by clinicians*</th>
<th>Incorporated into patient education materials</th>
<th>Incorporated into clinical practice guidelines</th>
<th>Change in clinical practice based on clinician self-report*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Manuscript downloads*</td>
<td>Incorporation into systematic reviews*</td>
<td>Incorporation into Graduate Medical Education/Continuing Medical Education</td>
<td>Incorporated into clinical decision making infrastructure (e.g., electronic decision aids, clinical reference tools)</td>
<td>Change in clinical practice based on medical record data or claims data*</td>
</tr>
</tbody>
</table>

*Indicates precedent in literature
Questions to Consider

- What points along this would indicate that the information we’ve produced is useful and being used?
- Should PCORI track the uptake and use of all its research studies or a subset of studies?
  - How might PCORI identify a subset of projects to track for uptake into clinical practice?
- For priority indicators for which there is no precedent, how might PCORI operationalize these indicators of clinical uptake?
Goal 3: Influence Research

Influence clinical and health care research funded by others to be more patient-centered.
PCORI’s Influence on Research

**Funders and Others**

**Intermediate Indicators of Influence**

- Use of Methodology Standards
- Use of PCORI Prioritization and Review Criteria or Processes
- Use and Support of PCORI Networks
- Use of PCORI Evaluation Evidence
- Use of PCORI-Supported Curricula or Training
- Collaborations/Co-funding with Other Funders
- Dissemination of PCORI Research Findings

For each dollar of PCORI funding, how much do other funders spend on PCOR?
Questions to Consider

What other indicators of influence should we consider? Which should we prioritize?

How can we define “PCOR” vs. research other than PCOR?
  - Is this determination based solely on a focus on outcomes patients care about?
  - Does it also include consideration of research approach (that is, engagement of patients/stakeholders)?
  - Does it also include adherence to Methodology Standards for PCOR?

Which types of funders can PCORI expect to influence (governmental, industry, non-profits)?

How willing might other types of funders be to collaborate in determining which of the work is considered PCOR and their financial investments in these projects?
Please get some lunch and finish placing your stickers!
Agenda Item:
Prioritize Evaluation Questions
Prioritize Evaluation Questions

Right for PCORI
- Uniqueness
- Appropriateness
- Capability

Potential to Impact PCORI’s Work and the Field
Agenda Item:
Discuss Evaluation Approaches
Next Steps
Appendix – Additional Materials

- Patient-Centered Outcomes Research
- Patient-Centeredness
- Engagement
**Characterizing Patient-Centered Outcomes Research**

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.

<table>
<thead>
<tr>
<th>Question</th>
<th>Answer</th>
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</thead>
<tbody>
<tr>
<td>“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”</td>
<td>“What are my options and what are the potential benefits and harms of those options?”</td>
</tr>
<tr>
<td>“How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?”</td>
<td></td>
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</tbody>
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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 steps 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 – Project Activities

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