Update from Patient-Centered Outcomes Research Institute (PCORI)

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Executive Director

Advancing Comparative Effectiveness Research under Health Reform

October 19, 2011
What’s In a Name?

Patient Engagement → Patient-Driven Research → Dissemination

Understanding the choices patients face

Aligning research questions and methods with patient needs

Providing patients and providers with information for better decisions
MISSION STATEMENT
(adopted July 17, 2011)

The Patient-Centered Outcomes Research Institute (PCORI) helps people make informed health care decisions – and improves health care delivery and outcomes – by producing and promoting high integrity, evidence-based information - that comes from research guided by patients, caregivers and the broader health care community.
Working Definition of PCOR  
(posted July 2011)

Patient-Centered Outcomes Research (PCOR) helps people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. This research answers patient-focused questions:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can the health care system improve my chances of achieving the outcomes I prefer?”
 Patient-Engagement Working Group formed
 Job Description for Director of Patient Engagement drafted and posted
 Directors of Stakeholder Engagement and Director of Communications added
Methodology Committee Structure

Methodology Committee
Co Chairs: Sherine Gabriel, MD, Mayo Clinic & Sharon-Lise Normand, PHD, Harvard Medical School

- **Patient-Centeredness**
  - **Methods** to incorporate the patient perspective into all phases of PCOR

- **Research Prioritization**
  - **Methods** to inform prioritization of new research studies

- **Research Methods**
  - **Methods** for using data, design, and statistical analyses to conduct PCOR
Patient Centeredness Workgroup Progress

Released RFP for Literature Review

“Review and Synthesis of Evidence for Eliciting the Patient’s Perspective in Patient-Centered Outcomes Research”

Released RFP for Interviews

“Expert Stakeholder Interviews to Identify Evidence for Eliciting the Patient’s Perspective in Patient-Centered Outcomes Research”
PCORI Pilot Projects
Due: 12/1/11

Areas of Interest

Developing, testing, refining, and/or evaluating new or existing methods (qualitative and quantitative) ..... for incorporating the perspectives of patients or other stakeholders into the development of national priorities.

Developing, testing, and/or refining existing methods for bringing together patients, caregivers, clinicians including non-traditional partners, and other stakeholders in all stages of a multi-stakeholder research process, from the generation and prioritization of research questions to the conduct and analysis of a study to dissemination of study results.

Developing, refining, testing, and/or evaluating patient-centered approaches, including decision-support tools, for translating evidence-based care into health care practice in ways that account for individual patient preferences for various outcomes.
<table>
<thead>
<tr>
<th><strong>PCORI Pilot Projects</strong></th>
<th><strong>Due: 12/1/11</strong></th>
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<tbody>
<tr>
<td><strong>Areas of Interest</strong></td>
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</table>

Identifying, testing, and/or evaluating **patient-centered outcomes instruments**. This may include predictive tools (eg: instruments that measure or predict outcomes of interest to patients) or identifying standards for measurement properties of patient-reported outcomes.

Identifying, testing, and evaluating methods that can be used to **assess the patient perspective** when researching behaviors, lifestyles, and choices within the patient’s control that may influence their outcomes.

Identifying, testing, refining and/or evaluating methods for studying the **patient-care team interaction in situations where multiple options for wellness, prevention, diagnosis or treatment exist.**
Incorporating a stronger patient focus will help to ensure that:

- The questions are practical, and usually comparative
- The variety of outcomes important to patients are not overlooked
- The patients studied come from relevant settings and are representative
- Possibilities of heterogeneity in effectiveness are examined
## To what extent will PCOR be CER?

### Questions in PCOR Definition

<table>
<thead>
<tr>
<th>Questions in PCOR Definition</th>
<th>Is this CER?</th>
</tr>
</thead>
<tbody>
<tr>
<td>“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”</td>
<td>+/-</td>
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<td>“What are my options and what are the benefits and harms of those options?”</td>
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</table>
Defining PCORI’s National Priorities for Research is the critical beginning
# Development of a National Priorities Framework

<table>
<thead>
<tr>
<th>Initial Stakeholder feedback</th>
<th>Environmental scan of existing priorities and criteria</th>
<th>Candidate priorities and criteria identified</th>
<th>Framework to inter-relate Priorities and Criteria</th>
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</thead>
<tbody>
<tr>
<td>Reviewed initial stakeholder input advising us to not “reinvent the wheel.”</td>
<td>Reviewed prior CER frameworks (e.g., IOM, FCCCER, National Priorities Partnership, NQF)</td>
<td>Identified broad priorities and criteria that were used often in prior frameworks and fit PCOR.</td>
<td>Framework to be used for refining priorities, and determining Research Agenda and PFAs.</td>
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</table>

See Rationale p.1
Ten candidate priorities consistent with prior efforts

<table>
<thead>
<tr>
<th>Source</th>
<th>Prevention</th>
<th>Acute Care</th>
<th>Chronic Disease Care</th>
<th>Palliative Care</th>
<th>Care Coordination</th>
<th>Patient Engagement</th>
<th>Safety</th>
<th>Appropriate Use</th>
<th>HIT to improve pt. experience</th>
<th>Impact of New Technology</th>
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</thead>
<tbody>
<tr>
<td>IOM 2009: Priorities for CER</td>
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<td>Federal Coordinating Committee for CER</td>
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<td>National Prevention Council</td>
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<td>National Priorities Partnership</td>
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Framework for Refining PCORI National Priorities and Developing Research Agenda

Priority Themes
- Clinical Effectiveness
- Patient-Centered Outcomes
- Improving Healthcare Systems
- Communication & Dissemination
- Fairness / Addressing Disparities
- Accelerating Patient-Centered Research

Proposed PCORI Criteria
- Impact on Health of Individuals and Populations
- Probability of Improvability via Research
- Inclusiveness of Different Sub-populations
- Address Current Gaps in Knowledge
- Address Variation in Care
- Improved Health Care System Performance
- Current Health Disparities
- Potential to Influence Decision Making
- Novel Use of Technology
- Efficient Use of Research Resources

PCORI Research Agenda
# Proposed Stakeholder Engagement Timeline

<table>
<thead>
<tr>
<th>September</th>
<th>October</th>
<th>November</th>
<th>December</th>
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<tbody>
<tr>
<td>Week 1</td>
<td>Week 5</td>
<td>Week 9</td>
<td>Week 13</td>
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<td>Week 2</td>
<td>Week 6</td>
<td>Week 10</td>
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<td>Week 3</td>
<td>Week 7</td>
<td>Week 11</td>
<td>Week 15</td>
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<tr>
<td>Week 4</td>
<td>Week 8</td>
<td>Week 12</td>
<td>Week 16</td>
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- **Create Engagement Plan and Identify Stakeholders**
- **Decide on Content, Methods of Dialogue**
- **Conduct Dialogue with Stakeholders**
- **Review, incorporate and Report on Feedback**

*Board Meetings*
### Who are PCORI’s stakeholders?

#### Key Stakeholder Groups

<table>
<thead>
<tr>
<th>Patients</th>
<th>Professional Organizations</th>
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<tbody>
<tr>
<td>Individuals with Illnesses</td>
<td>Providers &amp; Practitioners</td>
</tr>
<tr>
<td>Families</td>
<td>Health Systems</td>
</tr>
<tr>
<td>Caregivers</td>
<td>Employers</td>
</tr>
<tr>
<td>Patient Organizations/</td>
<td>Payers</td>
</tr>
<tr>
<td>Advocacy Groups</td>
<td>Research Community</td>
</tr>
<tr>
<td>General Public</td>
<td>Federal/State/Local Government</td>
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<td></td>
<td>Life Sciences Industry</td>
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## Stakeholder Engagement - Methods and Timeline

<table>
<thead>
<tr>
<th>October</th>
<th>November</th>
<th>December</th>
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<tbody>
<tr>
<td>1 2 3 4</td>
<td>5 6 7 8 9</td>
<td>10 11 12</td>
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### Surveys
- **Surveys Available**

### Focus Groups
- **Focus Groups Conducted**

### Stakeholder Presentations
- Green diamonds indicating presentations scheduled for October 5, 6, 7, 11, and 12.

### Crowd-sourcing
- Blue squares indicating data collection periods for November 9 and December 12.

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*Board Approves All Changes*
Fundamental Questions on Research Agenda

- Will PCORI’s Research Agenda (and any funding announcements) remain condition-neutral?

- To what extent will PCORI solicit and fund dissemination research vs. effectiveness research?

- To what extent will PCORI invest in CER infrastructure - and what kinds?
  - Large clinical databases? Registries? Patient networks?

- To what extent will PCORI look to partner with NIH / AHRQ / Foundations to fund certain types of activities (e.g., ancillary studies associated with large clinical trials)?

- Will PCORI fund large clinical trials ourselves and if so, what proportion of the budget should be dedicated to this?

- How will PCORI address research on rare diseases? For example registry building to facilitate recruitment for clinical trials?