PCORI Evaluation Group

Fifth Meeting
Friday, April 25, 2014
Questions about Our Goals

1. What are we doing?
   Are we doing it efficiently and effectively?
   Are we on track?

2. Are we accomplishing our goals?
   Producing useful information?
   Speeding its uptake?
   Influencing research?

3. How do the various components of PCORI’s approach contribute to reaching its goals and achieving its mission?
Focus for Today

Measuring Our First Goal: Useful Information

○ Tracking Funding, Milestones

○ Analyzing Our Portfolio

○ Developing Usefulness Criteria
  ▪ Where we started – What’s useful?
  ▪ What we’ve been doing – Pilot testing
  ▪ Current status – Our thinking has evolved
  ▪ Next Steps – Evaluate potential usefulness of portfolio
“It was later decided that the structure’s crowning glory would be the largest cupola on Earth, ensuring the church would be ‘more useful and beautiful, more powerful and honorable’ than any other ever built, as the grandees of Florence decreed.”

National Geographic, February 2014
Goal 1: Useful Information

Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions.
In our strategic plan, we proposed that our primary measure of success for our first goal would be the amount of useful information we produce (also the proportion of our portfolio that yields useful information).

Then we had to explain what we mean by “useful” and how we would measure it!

Ultimately, we will judge the usefulness of the information we produce by whether or not it gets used (relates also to our second goal to speed implementation).

But we won’t have a significant body of completed studies for a few years and are eager to learn what we can do in the meantime to ensure their usefulness.
Defining Useful

We set out to learn how people would be judging the usefulness of the information we produce so that we could align our processes accordingly.

- Literature Search
- Collaboration with National Health Council
- Input from Stakeholders

We found variation, depending on perspective, but essentially 3 main categories emerged:

- Quality/Methodological Rigor (*Methodology Standards*)
- What Matters to End-Users
- Effective Communication (*Our Second Goal*)
Initially, we were thinking generally about usefulness and so drafted some criteria that are not specific to the purpose for which they would be applied or who would be applying them.

For example, some of the organizations we have been working with would want to use such criteria to determine whether they should give their “seal of approval” to our information, disseminate it, or recommend its use by their constituencies.
Usefulness Criteria

PCORI’s initial purpose for Usefulness Criteria is to assess the potential of the studies in our portfolio to yield useful information.

So we have honed our initial, generic list with this in mind.

And we are also determining the extent to which these criteria overlap with criteria that we already have in place (for example, Merit Review criteria), and asking whether we are capturing anything distinct with our Usefulness Criteria.

Depending on how our portfolio is looking with respect to these criteria, we may learn, for example, that we need to refine our criteria for selecting which studies to fund in order to more effectively select those that will yield useful information.
Proposed Usefulness Criteria

February 2014

- People who would use the information have been identified
- Specific uses for the information have been identified
- People who would use the information have shaped the question(s)
- Study assesses treatment options that are relevant for the people who would use the information
- Study assesses the outcome(s) assessed that matter for the people who would use the information
- Study can / does provide a clear answer to the question
- Results can be / are timely and durable
- Results can be / are tailored to individuals or subgroups
- Results can be scaled / spread beyond the study setting
Proposed Usefulness Criteria – Categorized
March 2014

Rationale/Need for the Research:
- People who would use the information have been identified
- Specific uses for the information have been identified
- People who would use the information are asking the question

Characteristics of the Research Question:
- Study compares options that are relevant for the people who would use the information
- Study assesses the outcome(s) that matter for the people who would use the information

Real-world Application of the Results:
- Results could / do provide a clear answer to the question
- Results could be / are timely and durable
- Results could be / are tailored to individuals or subgroups
- Results could be scaled / spread beyond the study setting
Testing Usefulness Criteria

Purpose: Test and apply the proposed usefulness criteria in a set of unfunded applications before assessing our portfolio

- Total of 12 *unfunded* applications
  - randomly selected from the August 2013 Cycle
  - 3 applications from each of the broad applications (except Methods)
  - Scored better than the 40th percentile

- Total of 7 reviewers
  - each reviewer was assigned to review 3 or 4 applications
  - each application reviewed by at least 2 people

- Reviewers determined:
  - whether the applications met any, all, or none of the criteria
  - what could be learned from the summary vs. full application vs. review
  - how difficult it was to locate answers and where they could generally be found
  - how long it took to review each application
Preliminary Analysis

We are still analyzing the data, but thus far have observed:

- Applying the criteria was not as straightforward as we had anticipated
- It was easier to be looking at the full application
- Applying the criteria took at least an hour per application on average
- Having the Review Summary was helpful
- “Not Clear” was a frequent choice
- We often had to “read-into” what was there
- Moderate agreement among reviewers
- The “End-Users” are sometimes not patients
  - this can make the interpretation/application of the criteria difficult
- “Usefulness” is closely related to but also distinct from “Patient-centeredness” and “Significance”
Preliminary Analysis

Our initial observations have led to additional steps:

- Attempting to specify what’s distinct about “Useful”

- Refining the criteria and the explanation of how to apply them

- Cross-walking the Usefulness criteria with other PCORI criteria:
  - PCORI Application Guidelines
  - PCORI Methodology Standards
  - Topic Selection
  - Merit Review Criteria
## Refinement of Criteria Grouping

<table>
<thead>
<tr>
<th>Original Criteria Groupings</th>
<th>Revised Criteria Groupings</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rationale</td>
<td>User-Driven</td>
</tr>
<tr>
<td>Research Question</td>
<td>User-Focused</td>
</tr>
<tr>
<td>Real-world application</td>
<td>Real-World Users</td>
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</tbody>
</table>
## Group 1: User-Driven

The research should identify how the information will be used AND demonstrate an understanding of and interaction with the relevant end-users of the information. Relevant end-users are those that would directly use the information, those end users who would be affected by the information should also be a focus of the research.

<table>
<thead>
<tr>
<th>Proposed Usefulness Criterion</th>
<th>Existing Criterion</th>
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<tbody>
<tr>
<td>The research should indicate the holes/gaps/questions which would be answered with this information (e.g., incorporated into shared-decision making tools, implemented in healthcare systems, or to influence payment or policy decisions).</td>
<td>Methods Standards</td>
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<tr>
<td></td>
<td>• RQ-1, RQ-3, PC-1, PC-4</td>
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<tr>
<td></td>
<td>Merit Review Criteria</td>
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<td></td>
<td>• Potential for the study to improve healthcare and outcomes</td>
</tr>
<tr>
<td>The end-users (patients, clinicians, payers, organizations, health systems etc.) of the information have been identified (e.g., in the literature, through engagement with partners).</td>
<td>Methods Standards (RQ-3, PC-1)</td>
</tr>
<tr>
<td>The end-users (patients, clinicians, payers, organizations, health systems etc.) have identified this information would fill a critical gap (e.g., end-users generated the research questions).</td>
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<tr>
<td>The end-users have committed to using the information (e.g., systems administrators/clinicians/etc. have committed to implement the intervention)</td>
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<td>Existing Criterion</td>
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</tbody>
</table>
| • The research **assesses options** that are relevant for the end users of the information.  
  • The end-users were involved in choosing or developing the options. | Methods Standards  
  • RQ-5 |
| • The research **assesses the outcome(s)** that will comprehensively address the needs of the end-users. | Methods Standards  
  • RQ-6, PC-3  
Topic Selection Criteria  
  • Patient centeredness  
Merit Review Criteria  
  • Patient – Centeredness |
### Group 3: Real-World Users

<table>
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<th>Proposed Usefulness Criterion</th>
<th>Existing Criterion</th>
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</table>
| Results can provide a **clinically** (in addition to statistically) **significant answer**. The study would provide a clear answer, rather than calling for further research. | Methods Standards  
  - IR-1 - IR-6  
  Merit Review Criteria  
  - Potential for Study to Improve Healthcare and Outcomes |
| Results can inform decisions of end-user(s) with **specific characteristics, conditions, and preferences.** | Methods Standards  
  - RQ-3, RQ-4  
  Merit Review Criteria  
  - Technical Merit |
| Results can be **scaled/spread** beyond the traditional study setting for a wider net impact. | Merit Review Criteria  
  - Technical Merit |
Highlights of Criteria Crosswalk

- Some overlap between proposed Usefulness Criteria with other sources of PCORI criteria

- User-Driven and User-Focused categories seem to have the same distinct element

- Real-World Use category seemed the least distinctive
Discussion Questions

- How can we capture/define/specify what seems to be a distinction between what “useful” and “patient-centered”?

- Where there is overlap with existing criteria that are capturing the element well, could we simply rely on the existing criteria and condense the usefulness criteria, or would some redundancy be beneficial (and worth the effort)?

- What else should we do before we launch into assessing our portfolio?

- Do we know enough now to begin to think about how we might want to modify LOIs (maybe not too difficult) or Applications (maybe very difficult)?
Next steps

Continue to test our criteria
- Apply to funded applications and to targeted PFA proposals

Identify ways to amend existing PCORI materials and processes (applications, merit review criteria, etc.) to better capture potential usefulness of information produced
Appendix – Additional Materials

- Revised Usefulness Criteria (April 2014)
- Application Guidelines
- Topic Selection Criteria
- Merit Review Criteria
- PCORI Methodology Standards
**Revised Usefulness Criteria**

**April 2014**

**USER-DRIVEN**
- The research should identify **how the information will be used** AND demonstrate an understanding of and interaction with the **relevant end-users of the information**. Relevant end-users are those that would directly use the information, those end users who would be affected by the information should also be a focus of the research.
  - The research should **indicate the holes/gaps/questions** which would be answered with this information (e.g., incorporated into shared-decision making tools, implemented in healthcare systems, or to influence payment or policy decisions).
  - The **end-users** (patients, clinicians, payers, organizations, health systems etc.) of the information have been identified (e.g., in the literature, through engagement with partners).
  - The end-users (patients, clinicians, payers, organizations, health systems etc.) have **identified this information would fill a critical gap** and (e.g., end-users generated the research questions).
  - The end-users have **committed to using the information** (e.g., systems administrators/clinicians/etc have committed to implement the intervention)

**USER-FOCUSED**
- The research **assesses options** that are relevant for the end users of the information.
  - The end-users were involved in choosing or developing the options.
- The research **assesses the outcome(s)** that will comprehensively address the needs of the end-users.

**REAL-WORLD USE**
- Results can provide a **clinically** (in addition to statistically) **significant answer**. The study would provide a clear answer, rather than calling for further research.
- Results can inform decisions of end-user(s) with **specific characteristics, conditions, and preferences**.
- Results can be **scaled/spread** beyond the traditional study setting for a wider net impact.
Topic Selection Criteria

- **Patient centeredness.** Is the proposed research focused on questions and outcomes of specific interest to patients, their caregivers and clinicians?

- **Burden.** Is the condition or disease associated with a significant burden in the US population (in terms of prevalence, mortality, morbidity, individual suffering, or loss of productivity)? Alternatively, is it a rare disease?

- **Potential for improving healthcare practice.** What is the likelihood that this research will change clinical practice or clinical decision-making?

- **Timeliness.** Are potential projects associated with this topic likely to be accomplishable within a short time-frame (3-5 years)?

http://www.pcori.org/funding-opportunities/funding-announcements/from-research-questions-to-research-funding-about-our-topic-generation-and-research-prioritization-process/
Merit Review Criteria

Criterion 1. Impact of the condition on the health of individuals and populations
Criterion 2. Potential for the study to improve health care and outcomes
Criterion 3. Technical merit
Criterion 4. Patient-centeredness
Criterion 5. Patient and stakeholder engagement

http://www.pcori.org/research-we-support/pcori-review-criteria/
Standards for Formulating Research Questions

Standards Associated with Patient-Centeredness

Standards for Data Integrity and Rigorous Analyses

PCORI Methodology Standards
Research Question

- **RQ-1**: Identify gaps in evidence
- **RQ-2**: Develop a formal study protocol
- **RQ-3**: Identify specific populations and health decision(s) affected by the research
- **RQ-4**: Identify and assess participant subgroups
- **RQ-5**: Select appropriate interventions and comparators
- **RQ-6**: Measure outcomes and people representing the population of interest notice and care about
PCORI 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.
IR-1: Assess data source adequacy
IR-2: Describe data linkage plans, if applicable
IR-3: *A priori*, specify plans for data analysis that correspond to major aims
IR-4: Document validated scales and tests
IR-5: Use sensitivity analyses to determine the impact of key assumptions
IR-6: Provide sufficient information in reports to allow for assessments of the study’s internal and external validity
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
Usefulness: What can we say about it in 2014?

How our portfolio stacks-up against our criteria for potential usefulness
- How the studies we are funding compare to those we didn’t
- How our portfolio compares to others

What stakeholders think about the potential usefulness of our portfolio
- For example, what stakeholders think about the cluster of asthma, pediatric, or mental health studies we are funding

Usefulness Criteria Met (Not real data)

- None 10%
- 1 to 3 40%
- 4 to 6 30%
- 7 to 9 20%