Advancing Patient Centered Outcomes Research (PCOR): Implications for Research Methods and Standard Terminology

Second International Conference on Research Methods for Standardized Technologies

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Disclosures

Dr. Robin Newhouse has disclosed a relevant financial interest with Patient Centered Outcomes Research Institute (PCORI) and Getwell Network to the learners of this activity.

There are no conflicts of interest or relevant financial interests that have been disclosed by the remaining presenters or planners of this activity that apply to this learning session.

Objective

Discuss patient-centered outcomes research (PCOR) and implications for standardized terminologies

Outline

- PCORI Standards
- PCORnet

Emerging Trends:
- Engagement
- Data Harmonization

PCORI Methodology Standards

Key Points

- Standard terminology is essential to learning health care systems
- Patient and stakeholder engagement are central to developing standard terminology for patient-centered outcomes research
- Patient reported outcomes should be incorporated into Electronic Health Records
- Standard terminology should be disseminated and used in health systems
We Fund Research That...

- Studies the benefits and harms of interventions and strategies delivered in real-world settings
- Compares at least two alternative approaches
- Adheres to PCORI’s Methodology Standards
- Is based on health outcomes that are meaningful to the patient population
- Engages patients and other stakeholders at every stage
- Is likely to improve current clinical practices

We Focus Attention On...

- Conditions
  - Conditions that affect large numbers of people across a range of populations
  - Conditions that place a heavy burden on individuals, families, specific populations, and society
  - Rare diseases, which are difficult to study
- Populations
  - Racial and ethnic minorities
  - Older adults
  - Low-income and rural populations
  - Children

The Research We Fund is Guided by Our National Priorities for Research

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Addressing Disparities
- Accelerating PCOR and Methodological Research

We Target Specific, High-Priority Topics

- Assessment of Prevention, Diagnosis, and Treatment Options
  - PCOR Treatment Options in Uterine Fibroids*
- Improving Healthcare Systems
  - Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons**
  - Effectiveness of Transitional Care
- Addressing Disparities
  - Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma
  - Obesity Treatment Options Set in Primary Care for Underserved Populations
  - Clinical Interventions to Address Hypertension Disparities

* Administered by AHRQ
** Administered by the National Institute on Aging

We Follow a Unique Proposal Review Process

Applications are reviewed against five criteria:

- Impact of the condition on the health of individuals/populations
- Potential for the study to improve healthcare and outcomes
- Technical merit
- Patient-centeredness
- Patient and stakeholder engagement

- Applications are reviewed by a panel of two scientists, one patient, and one other stakeholder.
- PCORI’s Board of Governors makes funding decisions based on merit review and staff recommendations.

We Work to Improve Research Methodology

In any study, methods matter. That’s why we’ve developed methodology standards that all research should follow, at a minimum.

Methodology Standards: 11 Broad Categories

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects
- Data Networks
- Data Registries
- Adaptive and Bayesian Trial Designs
- Causal Inference
- Studies of Diagnostic Tests
- Systematic Reviews
Role of the Standards in PCORI Applications

• The Methodology Standards and report are tools for applicants in preparing their applications.
• Applications must demonstrate adherence to PCORI’s Methodology Standards.
• Not all standards apply to all studies.
• The standards do NOT dictate specific study designs.
• The Methodology Standards map to PCORI’s merit review criteria.
• PCORI technical reviewers assess the methodological rigor of each study’s methods.

Methodology Standards and Proposals

“In the Research Strategy section of the Research Plan Template, you should describe in detail your research strategy and plan, and you should demonstrate how your proposed study is responsive to the PFA. Include the relevant PCORI Methodology Standard citations (e.g., “PC-3”) as identified in the Methodology Report; you should refer to this report for an explanation on each standard. You do not need to list every standard, only standards that are relevant to your proposal.”

See http://www.pcori.org/content/faqs-applicants#WHAT%20TO%20INCLUDE

The Standards Help to Craft a Research Question

RQ-1 Identify gaps in evidence
Gap analysis and systematic reviews should be used to support the need for a proposed study. If a systematic review is not available, a systematic review should be performed using accepted standards in the field (see standard SR-1), or a strong rationale should be presented for proceeding without a systematic review. In the case where a systematic review is not possible, the methods used to review the literature should be explained and justified.

RQ-6 Measure outcomes that people representing the population of interest notice and care about
Identify and include outcomes the population of interest notices and cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform an identified health decision. Define outcomes clearly, especially for complex conditions or outcomes that may not have established clinical criteria. Provide information that supports the selection of outcomes as meeting the criteria of “patient-centered,” and “relevant to decision makers” such as patient and decision maker input from meetings, surveys, or published studies. Select outcomes based on input directly elicited from patient informants, people representative of the population of interest, either in previous studies or in the proposed research.

The Standards Guide Development of Patient-Centered Projects

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

The Standards Address Missing Data

MD-1 Describe methods to prevent and monitor missing data

MD-3 Use validated methods to deal with missing data that properly account for statistical uncertainty due to missingness

Stories Highlight Important Methods Issues

The stories are not intended to endorse specific research approaches; they demonstrate that good methods make a difference.
Patient Engagement vs. Patient-Centeredness

- **Patient engagement** is about having patients as partners in research as opposed to merely subjects
- Active engagement between scientists, patients, and stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought-out plan

- **Patient-Centeredness** is a component of what PCOR is looking for in research applications
- Does the project aim to answer questions or examine outcomes that matter to patients within the context of patient preferences?
- Research questions and outcomes should reflect what is important to patients and caregivers

Pivotal $100M Infrastructure Investment

11 Clinical Data Research Networks (CDRNs)
- System-based networks, such as integrated delivery systems, academic medical centers, federally qualified health centers,

18 Patient-Powered Research Networks (PPRNs)
- Participants working together to discover, propose, and answer relevant research questions. Building the tools to engage people more broadly in research from end to end.

Coordinating Center
- Provides technical and logistical assistance under the direction of a steering committee and PCORI program staff

Spring 2015: Coming Into View

PCORnet's infrastructure built to:
- To leverage rich clinical electronic health data linking EHR data with private and public claims data (incl. CMS)
- Support both large observational studies and embedded randomized clinical trials
- Support novel models of patient-led research
- Involve patients, clinicians, and health systems leaders in governance and use of the network

DataMarts leveraging the CDRNs Electronic Health Data

- Each CDRN Network will have 1-10 DataMarts
- Total anticipated DataMarts: 75
- Annotated Data Dictionaries received: 62
- Software installation completed: 30
- Nine of 11 CDRNs have transformed data for at least one million individuals

CDRNs Disease Cohorts

<table>
<thead>
<tr>
<th>Organization</th>
<th>Common Cohort</th>
<th>Rare Cohort</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADVANCE</td>
<td>Diabetes</td>
<td>Diabetes with HIV and hepatitis C virus</td>
</tr>
<tr>
<td>CAPriCORN</td>
<td>Asthma</td>
<td>Sickle cell disease, Renal cystic disease</td>
</tr>
<tr>
<td>Great Plains Collaborative</td>
<td>Breast Cancer</td>
<td>Renal cell cancer (RCC)</td>
</tr>
<tr>
<td>Louisiana Clinical Data</td>
<td>Asthma</td>
<td>Sickle cell disease, Rare cancers</td>
</tr>
<tr>
<td>Research Network</td>
<td>Diarrhea</td>
<td></td>
</tr>
<tr>
<td>Mid-South CDRN</td>
<td>Diabetes</td>
<td>Sickle cell disease (SCD)</td>
</tr>
<tr>
<td>Nationwide Medical</td>
<td>Coronary Heart Disease (CHD)</td>
<td>Sickle cell disease (SCD)</td>
</tr>
<tr>
<td>Research Network</td>
<td>Colorectal Cancer</td>
<td></td>
</tr>
<tr>
<td>PORTAL</td>
<td>Colorectal Cancer</td>
<td></td>
</tr>
<tr>
<td>PEDSNet</td>
<td>Inflammatory bowel disease</td>
<td></td>
</tr>
<tr>
<td>PORTAL</td>
<td>Congenital Heart failure</td>
<td></td>
</tr>
<tr>
<td>pSCANNER</td>
<td>Congenital Heart failure</td>
<td></td>
</tr>
<tr>
<td>P2ATH</td>
<td>Atrial Fibrillation</td>
<td></td>
</tr>
<tr>
<td>PORTAL</td>
<td>Idiopathic Pulmonary Fibrosis</td>
<td></td>
</tr>
<tr>
<td>SCIHLS</td>
<td>Osteoarthritis</td>
<td></td>
</tr>
<tr>
<td>P2ATH</td>
<td>Pulmonary arterial hypertension</td>
<td></td>
</tr>
</tbody>
</table>
Comparative effectiveness of

Data captured from processes associated with healthcare delivery

Interventional studies

Goal of demonstration observational and interventional studies

Guiding principle: Make research easier

Requirements for Network Participation in Observational Studies

Data Quality Assurance review process
Find Us Online

www.pcori.org

PCOR Infrastructure and Training
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PROEM Center Director
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PATIENTS Program Director

Patient-Centered Research for Outcomes, Effectiveness and Measurement (PROEM)
A Center of Excellence in Comparative Effectiveness and Patient-Centered Outcomes Research (CER-PCOR) Training
Eleanor M. Perfetto, PhD, MS, Professor
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Patient Engagement is a Means for:
Empowering and Inspiring the Community
Building Trust for Authentic Partnerships
Improving Health and Healthcare Decisions
Advancing Patient-Driven Scientific Discovery

Course Content

<table>
<thead>
<tr>
<th>Clinical Outcomes Assessment</th>
<th>Introduction to CER/PCOR</th>
<th>Quality and Performance</th>
</tr>
</thead>
<tbody>
<tr>
<td>Standardized Terminology</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Data Harmonization</td>
<td>X</td>
<td>X</td>
</tr>
</tbody>
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Experiential sites
Conferences
Weblinks
Program Collaborations
Surveys
Newsletters
**10-STEP FRAMEWORK FOR CONTINUOUS PATIENT ENGAGEMENT IN RESEARCH**

**A. PLANNING RESEARCH**
1. Topic Solicitation
2. Prioritization
3. Framing the Question

**B. DOING IT**
4. Selection of Comparators and Outcomes
5. Creation of Conceptual Framework
6. Analysis Plan
7. Data Collection

**C. DELIVERING SOLUTIONS**
8. Reviewing & Interpreting Results
9. Translation
10. Dissemination


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**Data Harmonization for Patient-Centered Clinical Research**

- Meet networks where they are
- Capitalize on diversity of networks
- Specify the “must have narrow neck”; make everything else modular
- Pay special attention to patient-generated data
- Maximize transparency
- Leverage existing standards and support institutional priorities
- Ground the approach in iteration and validation


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**Committee on Core Metrics for Better Health at Lower Cost**

“The Committee on Core Metrics for Better Health at Lower Cost will conduct a study and prepare a report directed at exploring measurement of individual and population health outcomes and costs, identifying fragilities and gaps in available systems, and considering approaches and priorities for developing the measures necessary for a continuously learning and improving health system.”


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**PCOR and Nursing Science**

**TABLE 1. Comparison of Nursing Research Goals and Patient-Centered Outcomes Research Institute Activities**

<table>
<thead>
<tr>
<th>Nursing research</th>
<th>Patient-Centered Outcomes Research Institute</th>
</tr>
</thead>
<tbody>
<tr>
<td>Build the scientific foundation for clinical practice</td>
<td>Produces dependable information that can inform health decisions</td>
</tr>
<tr>
<td>Prevent disease and disability</td>
<td>Provides evidence that guides decisions about prevention of disease</td>
</tr>
<tr>
<td>Manage and eliminate symptoms caused by illness</td>
<td>Provides evidence to improve outcomes that patients care about, such as function, symptoms, health-related quality of life, and survival</td>
</tr>
</tbody>
</table>

*Source: National Institute of Nursing Research (n.d.).


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**Key Points**

- Standard terminology is essential to for learning health care systems
- Patient and stakeholder engagement are central to developing standard terminology for patient-centered outcomes research
- Patient reported outcomes should be incorporated into Electronic Health Records
- Standard terminology should be disseminated and used in health systems
References


See summary of PCORI study results and related papers at:
http://www.pcori.org/content/pcori-literature

Using the PCORI Methodology Standards available at:
http://www.pcori.org/events/2013/using-pcori-methodology-standards

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