

Patient-Centered Outcomes Research Institute
PCORI Methodology Committee
Report

Setting Standards for Patient-Centeredness and
Research Prioritization

August 14, 2012



Presenters



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Associate Attending Physician &
Outcomes Scientist
Memorial Sloan-Kettering Cancer Center



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Member, Methodology Committee
Chief of the Section of Hospital
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The University of Chicago



Gail Hunt
Member, PCORI Board of Governors
President and CEO of the National
Alliance for Caregiving



Lori Frank, PhD
Director
Engagement Research
PCORI

Webinar Agenda

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|--|--------------------|
| 1. Introduction to PCORI | 1:00pm – 1:05pm ET |
| 2. A Unique Focus on Patient Engagement | 1:05pm – 1:10pm ET |
| 3. Methodology Committee Mission & Report | 1:10pm – 1:20pm ET |
| 4. Patient-Centeredness and Research
Prioritization | 1:20pm – 1:30pm ET |
| 5. Questions and Answers | 1:30pm – 2:00pm ET |

Please submit questions for the Q&A portion of today's webinar to
methodswebinar@pcori.org

Formal public comments can be submitted at
pcori.org/survey/methodology-report/

Poll Questions 1 - 4

1. **Are you familiar with the contents of the Methodology Committee Report?**
(Y/N)
2. **For this webinar, which stakeholder community best describes your interest in engaging PCORI?**
3. **Rate your understanding of the process the Methodology Committee used to generate standards:**
 - a) I do not understand the process the Methodology Committee used to generate standards
 - b) I understand the process somewhat
 - c) I have good understanding of the process the Methodology Committee used to generate standards
4. **Which response most closely matches your opinion of the Standards in the draft Report?**
 - a) The Standards largely cover the main areas important to patient-centered outcomes research
 - b) Several important areas are not covered and additional Standards should be considered
 - c) Don't know/Not sure

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About PCORI

- An independent, non-profit organization authorized by Congress.
- Committed to continuously seeking input from patients and a broad range of stakeholders to guide its work.
- Mission – To help people make informed health care decisions and improve 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.**



Defining 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. This research answers patient-centered questions such as:

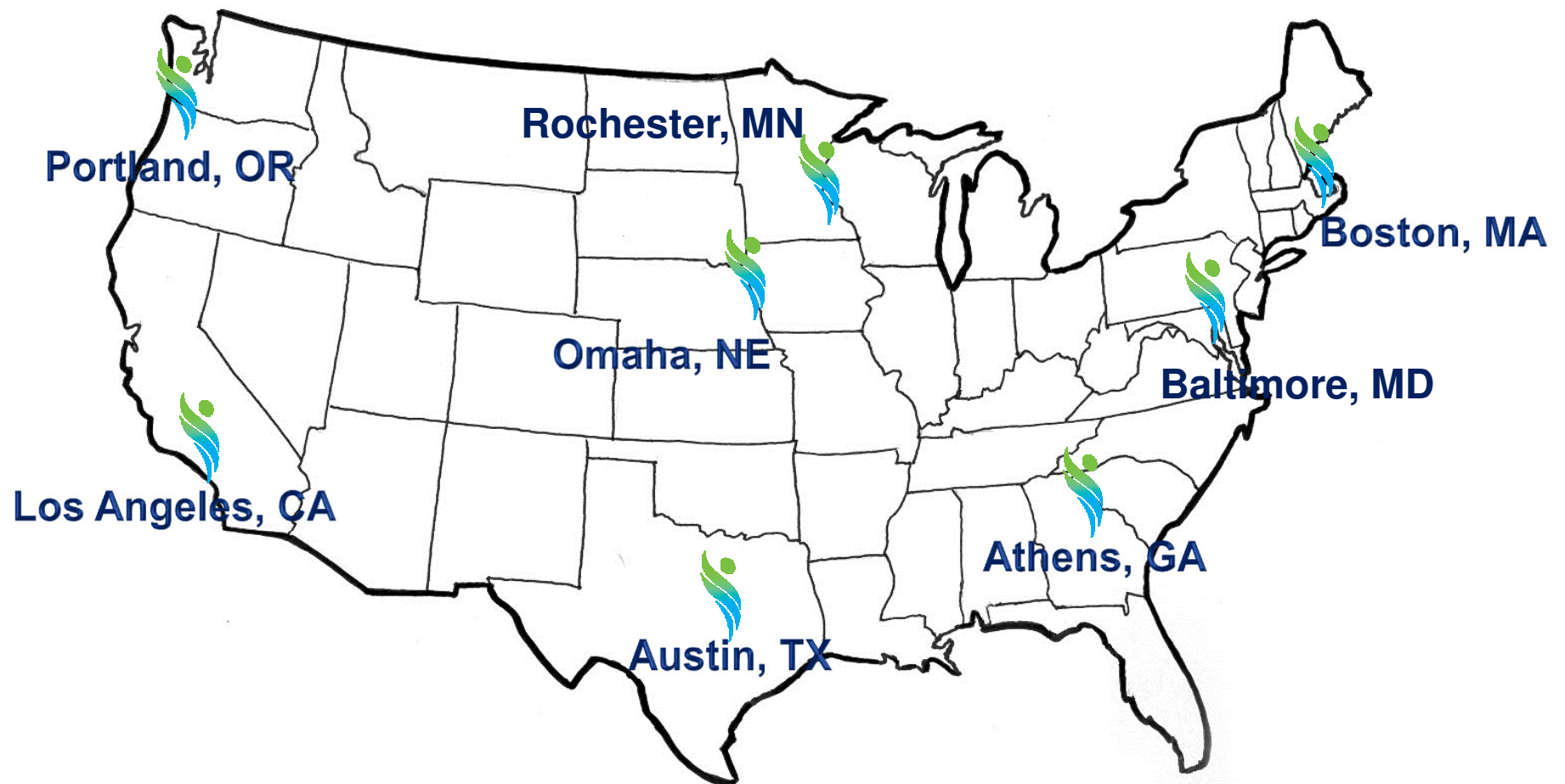
Expectations	Options	Outcomes	Decisions
<p>“Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”</p>	<p>“What are my options and what are the potential benefits and harms of those options?”</p>	<p>“What can I do to improve the outcomes that are most important to me?”</p>	<p>“How can clinicians and the care delivery systems help me make the best decisions about my health and healthcare?”</p>

Commitment to Patient-Engagement

- **Bringing patients' and caregivers' voices to research is one of PCORI's core values.**
- The development and widespread adoption of standards for patient-centeredness and engagement in research is critical for helping patients and caregivers make more informed health decisions.
- The draft Report's "patient-centeredness" standards were informed, in part, by patients and caregiver interviews and focus groups throughout the country.
- PCORI looks forward to continually tapping into the energy and wisdom of the patient, caregiver and other stakeholder communities to create **a new model for research.**

Methodology Report

Patient and Stakeholder Input



Patient-Centeredness and the draft Methodology Report

The standards include specific calls for patient involvement in all phases of patient-centered outcomes research (PCOR) including:

- Formulating research questions
 - Defining essential characteristics of study participants, comparators, and outcomes
 - Monitoring study conduct and progress
 - Disseminating results
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- The proposed standards also highlight the importance of patient participation in the process of prioritizing which research proposals are funded.

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PCORI Methodology Committee



MEMBER	TITLE
Sherine Gabriel, MD, MSc (Chair)	Professor of Medicine and of Epidemiology , William J. and Charles H. Mayo Professor at Mayo Clinic
Sharon-Lise Normand, MSc, PhD (Vice Chair)	Professor of Health Care Policy (Biostatistics) in the Department of Health Care Policy at Harvard Medical School and Professor in the Department of Biostatistics at the Harvard School of Public Health
Naomi Aronson, PhD	Executive Director of the Blue Cross and Blue Shield Association Technology Evaluation Center
Ethan Basch, MD, MSc	Associate Attending Physician and Outcomes Scientist at Memorial Sloan-Kettering Cancer Center
Alfred Berg, MD, MPH	Professor in the Department of Family Medicine at the University of Washington in Seattle
David Flum, MD, MPH	Professor in the Department of Surgery and Adjunct Professor in Health Services and Pharmacy at the University of Washington Schools of Medicine, Public Health and Pharmacy
Steven Goodman, MD, PhD	Associate Dean for Clinical and Translational Research, School of Medicine , Stanford University
Mark Helfand, MD, MS, MPH	Professor of Medicine and Professor of Medical Informatics and Clinical Epidemiology at the Oregon Health & Science University
John Ioannidis, MD, DSc	C.F. Rehnberg Chair in Disease Prevention, Professor of Medicine, Professor of Health Research and Policy, and Director of the Stanford Prevention Research Center at Stanford University
Michael Lauer, MD	Director of the Division of Cardiovascular Sciences at the National Heart, Lung, and Blood Institute
David Meltzer, MD, PhD	Chief of the Section of Hospital Medicine, The University of Chicago
Brian Mittman, PhD	Director, VA Center for Implementation Practice and Research Support, Department of Veterans Affairs Greater Los Angeles VA Healthcare System
Robin Newhouse, PhD, RN	Chair and Professor, Organizational Systems and Adult Health at University of Maryland School of Nursing
Sebastian Schneeweiss, MD, ScD	Associate Professor of Medicine and Epidemiology at Harvard Medical School and Vice Chief of the Division of Pharmacoepidemiology and Pharmacoeconomics at the Brigham and Women's Hospital
Jean Slutsky, PA, MSPH	Director of the Center for Outcomes and Evidence , Agency for Healthcare Research and Quality
Mary Tinetti, MD	Gladdys Phillips Crofoot Professor of Medicine, Epidemiology, and Public Health in the Division of Geriatrics at Yale University School of Medicine
Clyde Yancy, MD, MSc	Chief, Cardiology, Northwestern University Feinberg School of Medicine

Methodology Report

- The mandate for PCORI's Methodology Committee is to **define methodological standards** and a **translation table** to guide health care stakeholders towards the best methods for patient-centered outcomes research (PCOR).
- **Rigorous methods** are essential to building trust in research findings.
- The report is the necessary catalyst for **scientifically rigorous, patient-centered** outcomes research that can inform decision-making.
- Once the Report is revised and accepted by the PCORI Board of Governors, future PCORI funding ***applicants will be expected to reference the Standards in their applications and use the Standards in their PCORI funded research.***

Methodology Report – What is a Standard?

Building on the work of the Institute of Medicine*, the Methodology Committee defined a standard as...

- A process, action, or procedure for performing PCOR that is deemed essential to producing scientifically valid, transparent, and reproducible results; a standard may be supported by scientific evidence, reasonable expectation that the standard helps achieve the anticipated level of quality in PCOR, or by broad acceptance of the practice in PCOR
- The recommendation is actionable, feasible, and implementable
- Proposed standards are intended for use by the PCORI Board, in PCORI policies and procedures, and by PCORI researchers

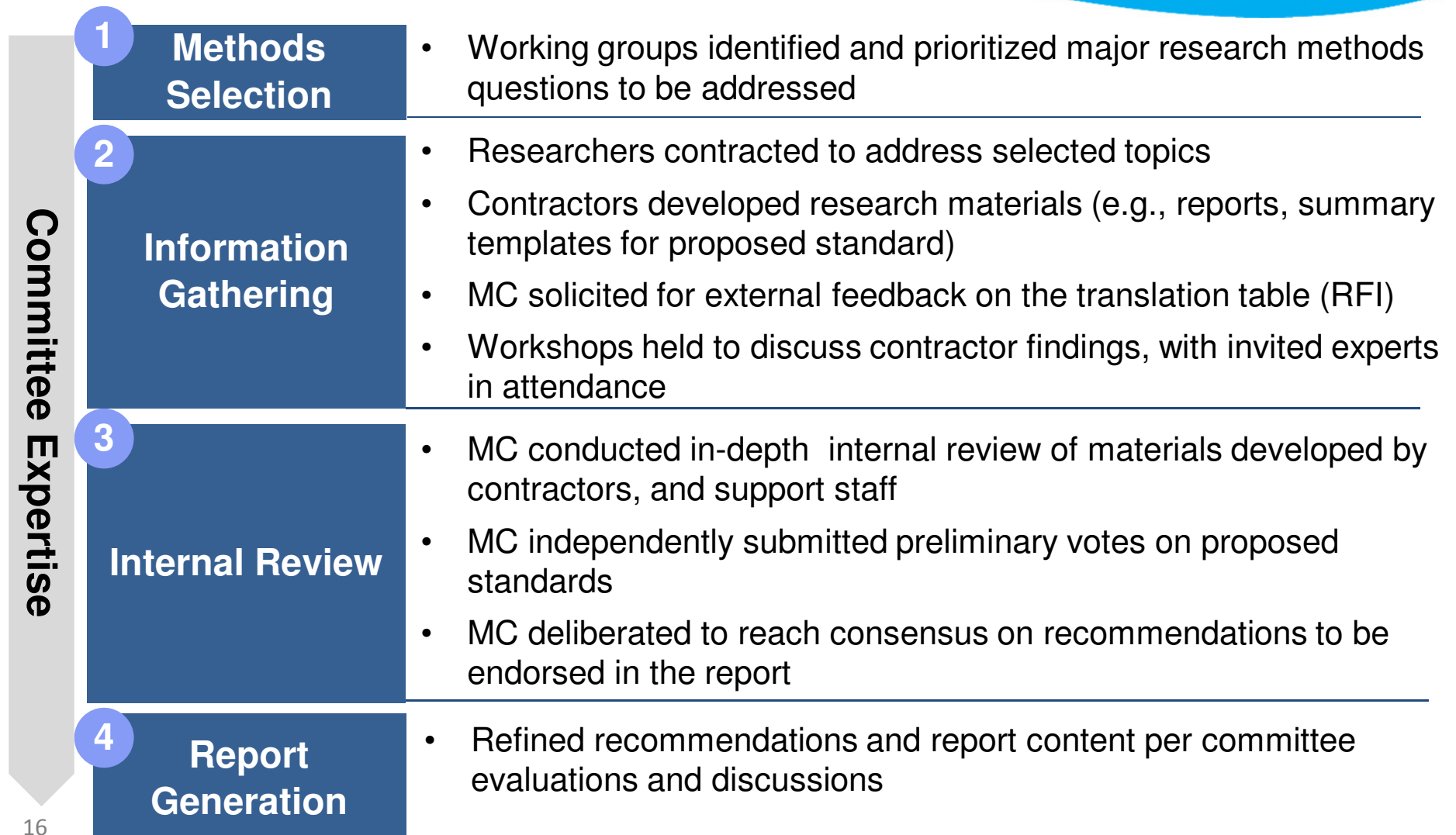
*Reference: National Research Council. Find What Works in Health Care: Standards for Systematic Reviews. Washington, DC: The National Academies Press; 2011.

Methodology Report – What Questions Should our Standards Address?

The MC sought to address selected topics in 4 broad phases of activities in the first Methodology Report:



Methodology Report Development

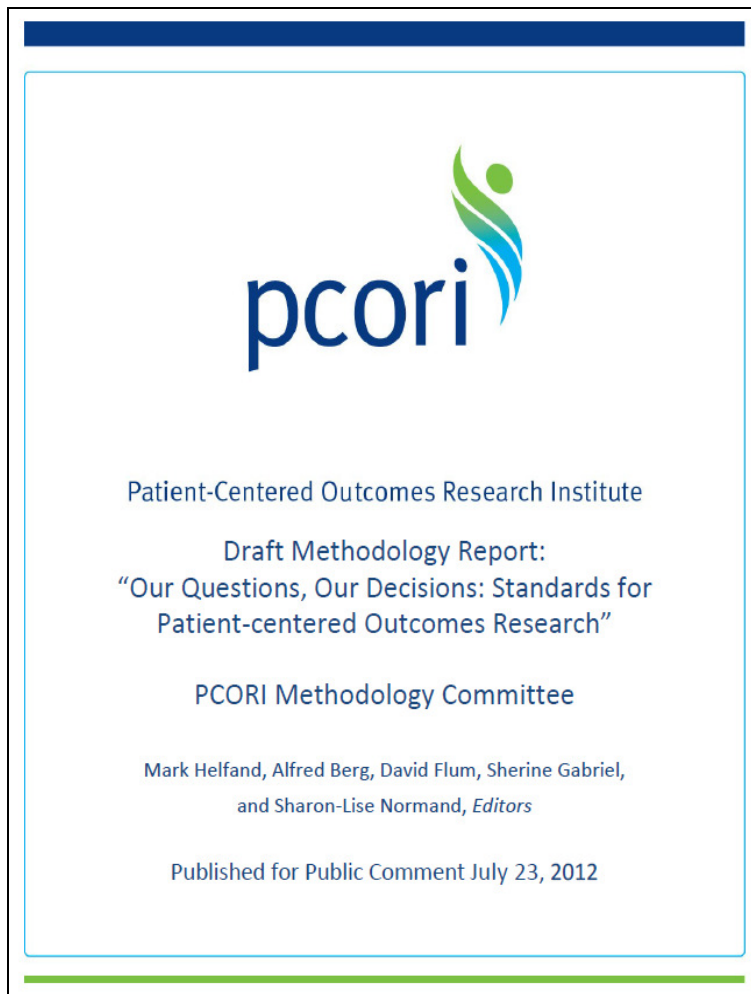


Methodology Report – Internal Review

The MC deliberated and agreed upon standards based on the following:

Patient-Centeredness	Respect for and responsiveness to individual patient preferences, needs, and values
Scientific Rigor	Objectivity, minimizing bias, improving reproducibility, complete reporting
Transparency	Explicit methods, consistent application, public review
Empirical/Theoretical Basis	Information upon which a proposed standard is based
Other Considerations	Practicality, feasibility, barriers to implementation, and cost

Methodology Report



- Submitted to the PCORI Board of Governors on May 10, 2012
- Accepted by the PCORI Board of Governors on May 21, 2012
- **A public comment period on the draft report: Through September 14 2012**
- Revised Report goes to the Board of Governors November 2012

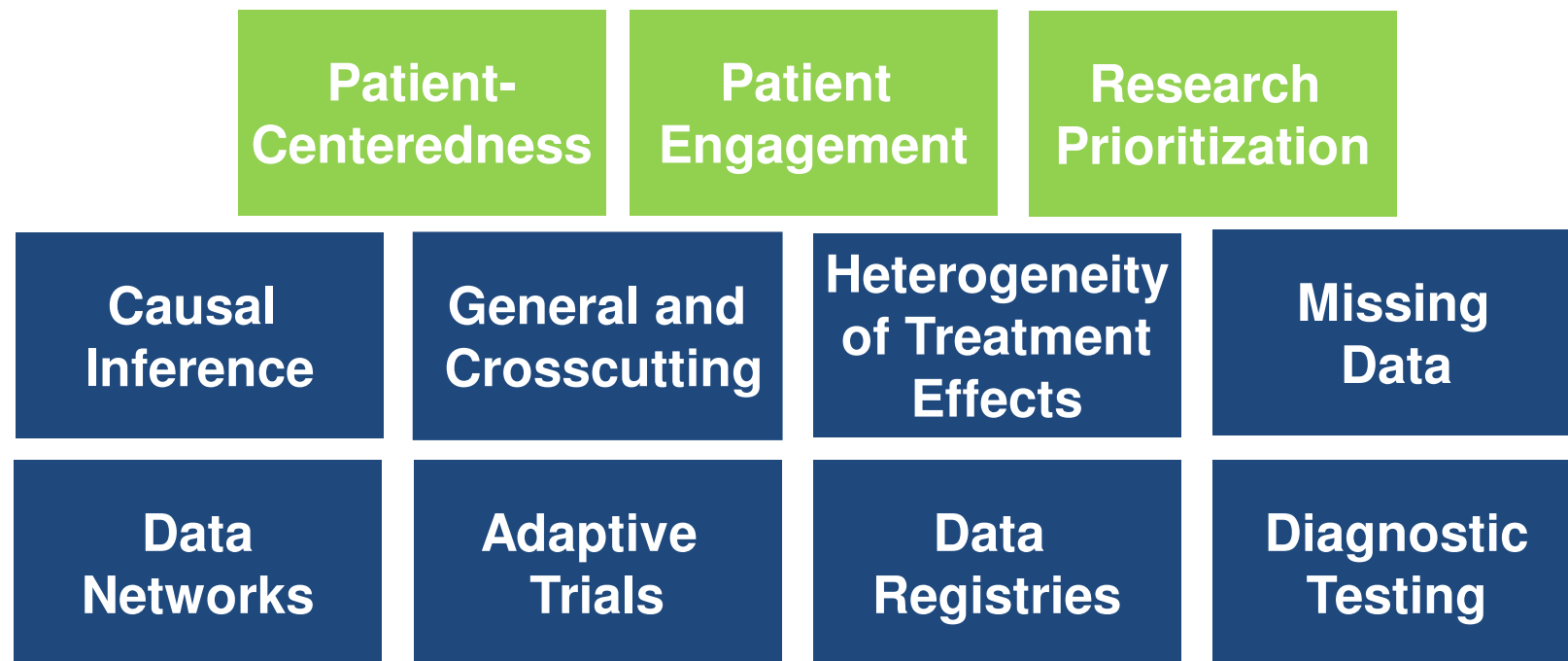
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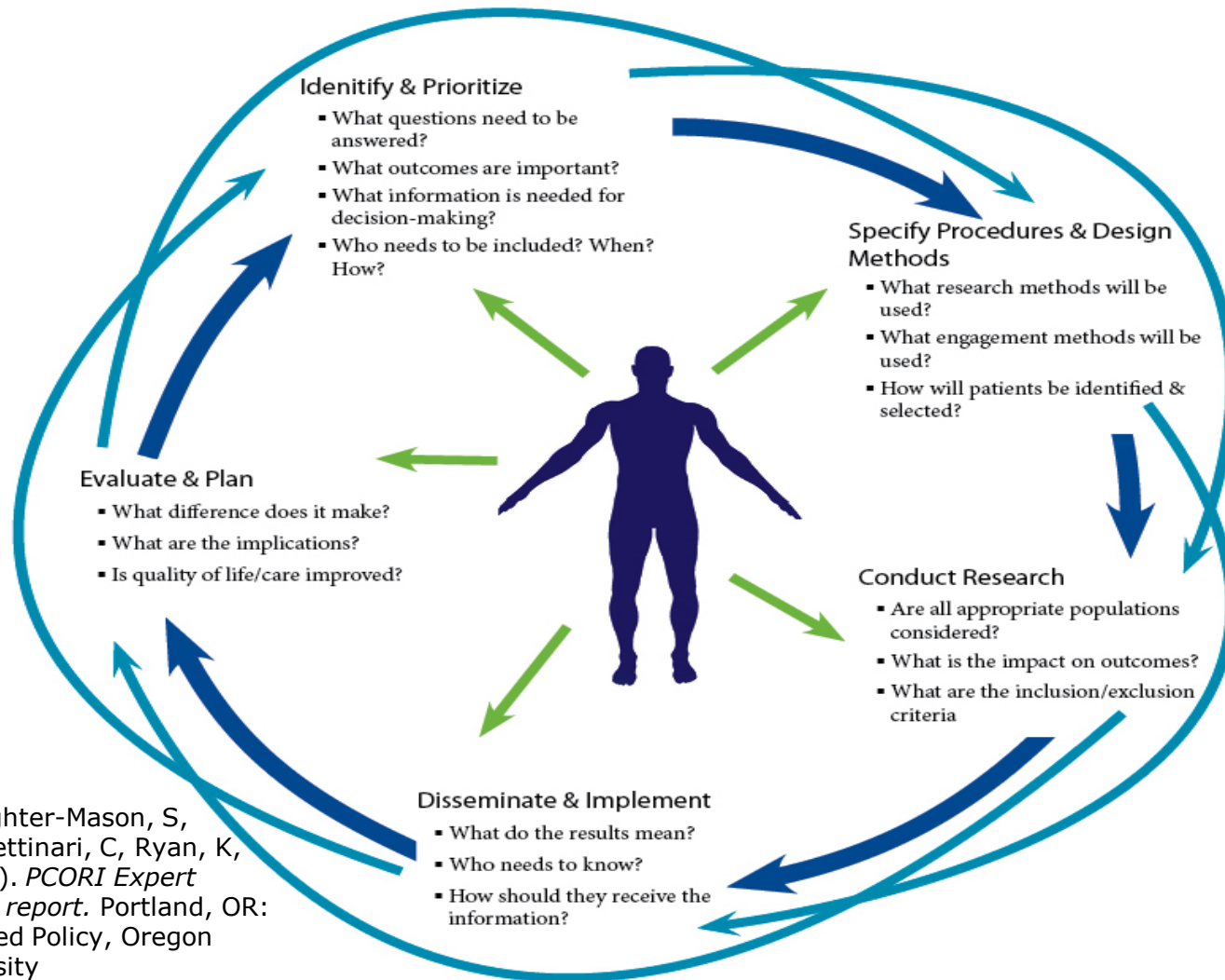
Methodology Report – Research Domains



What Makes a Study Patient-Centered?

- Patient-centered outcomes research *starts from the perspective of individual facing health decisions.*
- Every phase of patient-centered outcomes research should be *directed towards informing health decisions* that affect outcomes meaningful to patients.
- Patient-centered outcomes research *helps people make informed health care decisions.*

Patient Engagement



Source: ¹Curtis, P, Slaughter-Mason, S, Thielke, A, Gordon, C, Pettinari, C, Ryan, K, Church, B, King, V(2012). *PCORI Expert Interviews Project: Final report*. Portland, OR: Center for Evidence-based Policy, Oregon Health & Science University

Standards for Patient-Centeredness and Engagement

3.1.2 Identify Specific Populations and Health Decision(s) Affected by the Research

3.1.5 Measure Outcomes that People in the Population of Interest Notice and Care About

4.1.1 Engage Patient Informants, Persons Representative of the Population of Interest, in All Phases of Patient-centered Outcomes Research (PCOR)

4.1.2 Identify, Select, Recruit, and Retain Study Participants Representative of the Spectrum of the Population of Interest Facing the Health Decision of Interest and Ensure that Data Are Collected Thoroughly and Systematically from All Study Participants

4.1.3 Use Patient-Reported Outcomes When Patients or People at Risk of a Condition Are the Best Source of Information

4.1.4 Develop and Implement a Dissemination Assessment to Achieve Broad Awareness of Study Results

Research Prioritization

- Need to select from among all possible research topics
 - Methodology Committee Process
 - Consider Prioritization Factors
 - Develop Framework for Establishing Priorities
 - Created Standards for selected components of Framework
- Standards must align with overall PCORI approach
 - Promote patient-centeredness and engagement

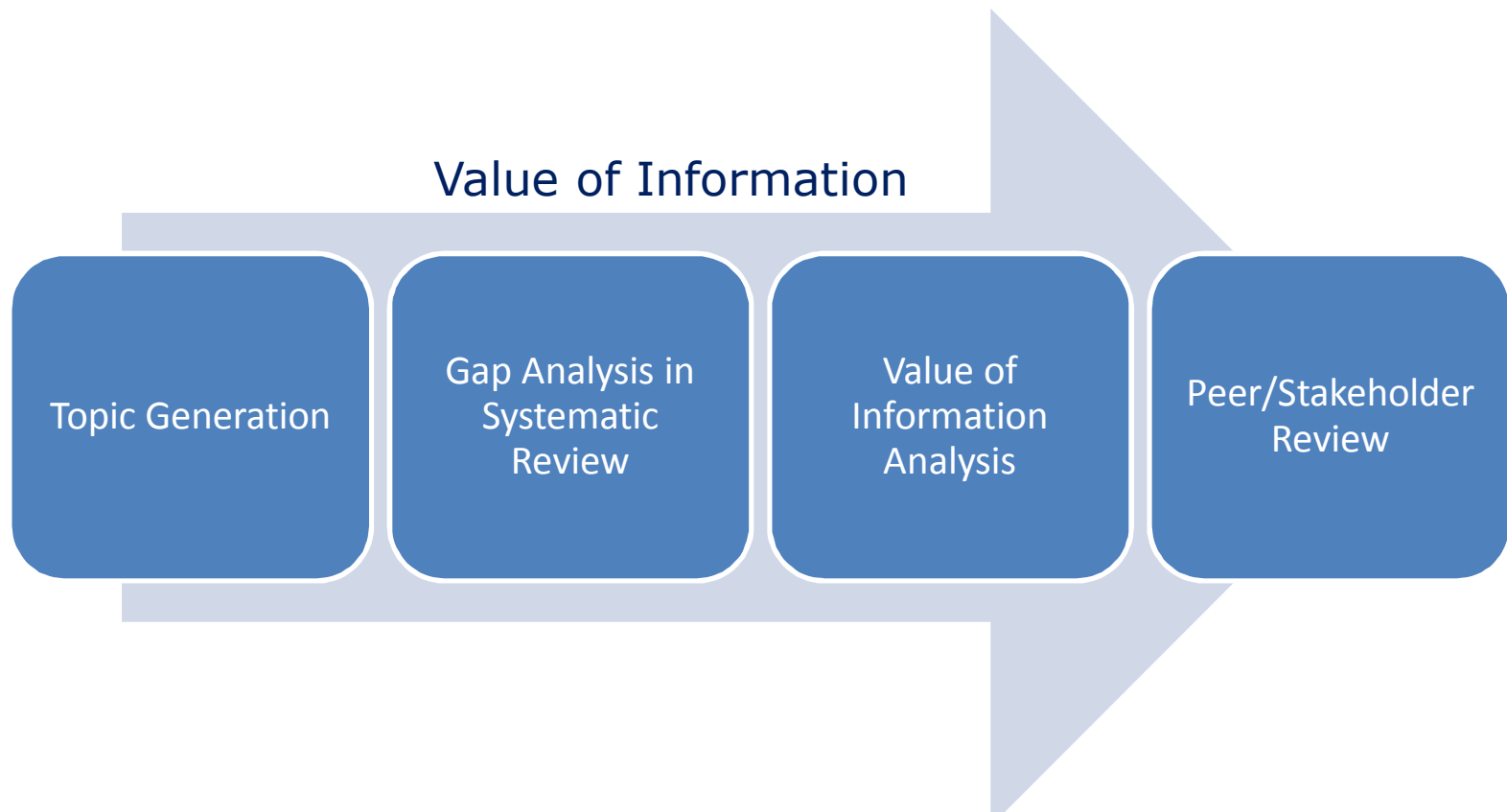
Prioritization Factors

- Disease or condition incidence, prevalence and burden
- Patient needs, outcomes and preferences
- Gaps in evidence
- Relevance to informed health decisions
- Potential for improvement based on new evidence
- Efficient use of PCORI research resources
- Priorities developed by other organizations

Methods to Assist in Prioritization

- Topic Generation
 - Identify questions that could be studied
- Gap Analysis in Systematic Review
 - Reviewing what has already been studied and figuring out what questions research has not answered yet
- Value of Information Analysis
 - A conceptually-driven framework for estimating the impact that new information from research could have
- Peer/Stakeholder Review
 - Involving patients and other decision-makers in deciding what to study

Framework for Prioritization



Standards for Research Prioritization

- **5.1.1** Use Systematic Reviews to Identify Gaps in Evidence
- **5.1.2** Protect Independence in Peer Review of Research Funding Proposals
- **5.1.3** Ensure Adequate Representation of Minorities and Disadvantaged Segments of the Population in Peer Review of Research Funding Proposals

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Poll Questions 5 - 8

- 5. Have you submitted an application for funding to PCORI in the past?
(Y/N)**
- 6. Do you plan to submit an application for funding to PCORI in the future?
(Y/N)**
- 7. Rate your understanding of the process the Methodology Committee used to generate standards:**
 - a) I do not understand the process the Methodology Committee used to generate standards
 - b) I understand the process somewhat
 - c) I have good understanding of the process the Methodology Committee used to generate standards
- 8. Do you plan to submit comments on the Report through the PCORI website? (Y/N)**

We look forward to your comments on the Draft Methodology Report

- Visit us at www.pcori.org (today's webinar will be archived there)
- Subscribe to PCORI updates at pcori.org/subscribe
- Follow @PCORI on Twitter
- Watch our YouTube channel [PCORINews](http://www.youtube.com/PCORINews)



The screenshot shows the PCORI website homepage. At the top, there is a navigation bar with links for Home, Subscribe, Contact Us, and Search. Below this is a secondary navigation bar with links for About Us, What We Do, Funding Opportunities, Meetings & Events, Get Involved, News Room, and Employment. The main content area features a large graphic with the text "Patient-Centered Methods Matter" and "Standards for Patient-Centered Outcomes Research". Below this graphic is a section titled "Defining Methodological Standards for Patient-Centered Outcomes Research" with a "Public Comment Period" button. To the right, there is a "More News From PCORI" section with several news items, including "PCORI Seeks Input on Draft Methodology Report..." and "Read the 2011 Annual Report...". At the bottom, there are four columns of content: "Get Involved" with a photo of two people talking, "Funding Opportunities" with a photo of a presentation, "Meetings & Events" with a photo of a calendar, and "The PCORI Blog" with a photo of a laptop and a cup of coffee. Each column has a brief text description and a "Get Involved" link.