

Methodology Committee Teleconference

Steven Goodman, MD, MHS, PhD
Chair, PCORI Methodology Committee

Robin Newhouse, PhD, RN
Vice Chair, PCORI Methodology Committee

October 17, 2022

Notice of Recording



Please note that this webinar is being recorded for posting on PCORI's website. The recording will be available to the public after this event.

Introduction: Acknowledgement of COI Statement & Overview of Agenda

Steven Goodman, MD, MHS, PhD
Chair, PCORI Methodology Committee

COI Statement: Methodology Committee



Welcome to the Methodology Committee meeting. I want to remind everyone that disclosures of conflicts of interest of members of the Methodology Committee are publicly available on PCORI's website and are required to be updated annually. Members of the Methodology Committee are also reminded to update your conflict of interest disclosures if the information has changed. You can do this by contacting your staff representative.

If the Methodology Committee will deliberate or take action on a matter that presents a conflict of interest for you, please inform the Chair so we can discuss how to address the issue. If you have questions about conflict of interest disclosures or recusals relating to you or others, please contact your staff representative, **Vita Pierzchala**.

Meeting Agenda & Topics

Agenda Item	Discussion Lead
Introduction <ul style="list-style-type: none">• Welcome, acknowledgement of COI Statement• Review of agenda• Review of members in attendance	Steve Goodman
PCORI Methodology Committee Overview and Governance	Erin Holve
Methodology Committee: Potential Workstreams	Erin Holve, Jason Gerson, Michelle Johnston-Fleece
Discussion: Opportunities for the MC to Support PCORI - Agenda-Setting & Planning	Steve Goodman, Robin Newhouse
Adjourn	Steve Goodman

Methodology Committee Roster



Steve Goodman, MD, MHS,
PhD (*Chair*)

Robin Newhouse, PhD,
RN (*Vice Chair*)

Naomi Aronson, PhD

Arlene Bierman, MD, MS

David Flum, MD, MPH

Cindy Girman, DrPH, FISPE

Mark Helfand, MD, MS, MPH

Michael Lauer, MD

David Meltzer, MD, PhD

Brian Mittman, PhD

Sally Morton, PhD

Neil Powe, MD, MPH, MBA

Adam Wilcox, PhD

PCORI Methodology Committee Overview and Governance

Erin Holve, PhD, MPH, MPP

Chief Research Infrastructure Officer



PCORI Methodology Committee



- *The Methodology Committee serves as a resource for PCORI and makes recommendations to the Board regarding methods for PCOR. Examples include developing high-quality methodological standards to guide the conduct of PCOR, establishing priorities to address gaps in research methods or their applications, and facilitating the utilization of standards and cutting-edge methods in PCOR.*
- The **Methodology Committee is comprised of up to 15 members appointed by the Board.**
- In addition to the members appointed by the Board, the Directors of the Agency for Healthcare Research and Quality and of the National Institutes of Health (or their designees) also serve on the Methodology Committee.
 - The Board appoints the Chair and Vice Chair of the Methodology Committee.

Revised Methodology Committee Conflict of Interest & Governance Framework

- At the March 8, 2022 Board of Governors meeting, the Board approved the Revised Conflict of Interest and Governance Framework for the Methodology Committee as Recommended by the Governance Committee:
 - *Future MC members will be required to forego the opportunity to apply for PCORI funding during their term on the MC and for one year thereafter, consistent with PCORI's conflict of interest policies*
 - MC will adopt *4-year staggered term* structure (with appointments every two years)
 - Board will be authorized to appoint MC members to a second 4-year term to the extent necessary to fulfill the functions of the MC, requirements of the authorizing law, or needs of PCORI. No MC member can be appointed to serve more than two full consecutive 4-year terms
- On June 1, 2022, PCORI opened a new call for nominations to the PCORI Methodology Committee, which closed July 29, 2022.

Methodology Committee – Potential Workstreams

Erin Holve, PhD, MPH, MPP

Jason Gerson, PhD

Michelle Johnston-Fleece, MPH

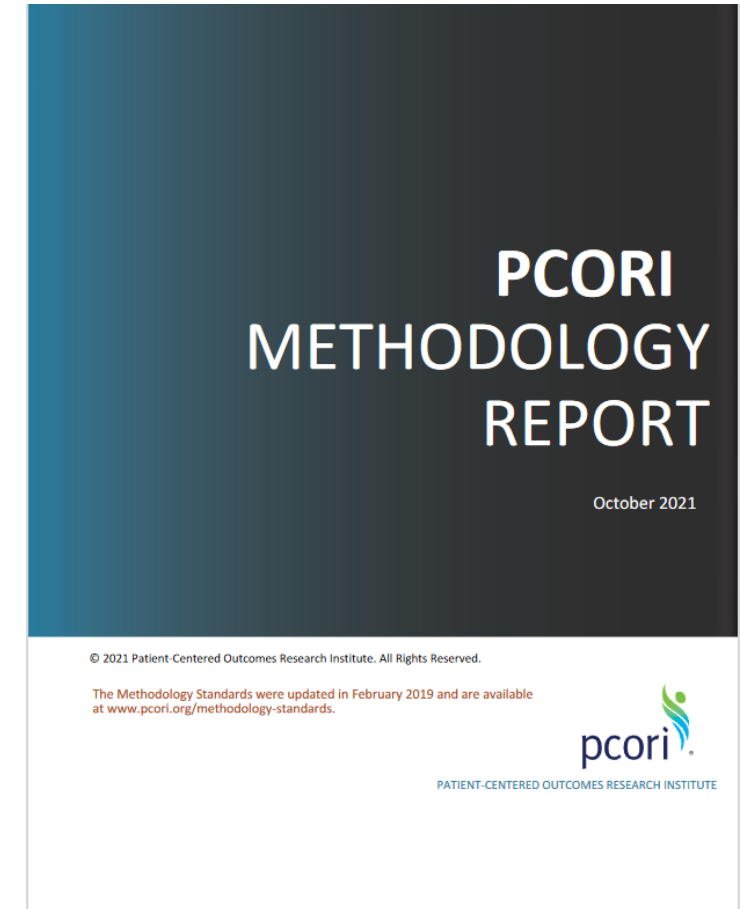
Potential Methodology Committee Workstreams

- Methodology Standards Development
- Methods Research
- Workforce development opportunities to advance Methods
- Innovative Methods to Support PCORI's National Priorities for Health

Methodology Standards Development & Reports

The PCORI Methodology Report includes four primary sections:

- **Section I: Patient-Centered Outcomes Research** reviews the need for methodological standards in patient-centered outcomes research.
- **Section II: Identifying and Addressing Evidence Gaps in PCOR** discusses the importance of a systematic process for prioritizing topics and determining appropriate research designs.
- **Section III: PCORI Methodology Standards** explains how PCORI developed and updated its set of **54 methodology standards**, discusses each group of standards, and summarizes the justifications for the standards.
- **Section IV: Advancing Understanding and Appropriate Use of Methods for PCOR** emphasizes the role of the Methodology Standards in promoting transparency, which is a critical component of good research practices.



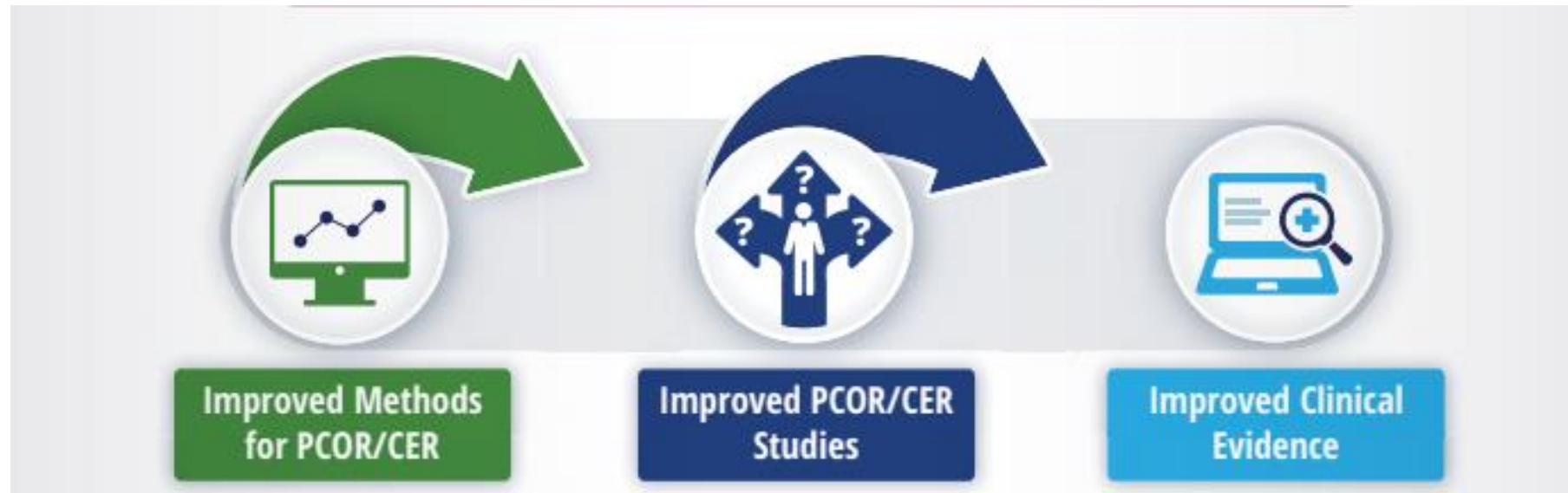
PCORI Methodology Standards – Evolving Over Time



- 2012: PCORI Releases the first set of Methodology standards in the first PCORI Methodology Report
- 2016: New standard added on Research Designs Using Clusters
- *2017: PCORI updates Methodology Report*
- 2018: New standard added on Complex Interventions
- 2019: Three (3) new standards on for qualitative methods (QM), mixed methods research (MM), and individual participant–level data meta-analysis (IPD-MA).
- *2021: PCORI updates Methodology Report*
- In 2022 the Methodology Committee is undertaking development of a new standard for Usual Care

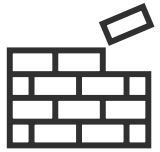
Methods Program and PFA Overview

Objective: Fund studies that advance understanding and appropriate use of methods for the conduct of PCOR/CER



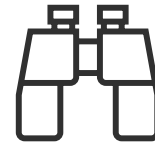
Research Supported by the Methods PFA

Methods Portfolio Size, Scope, and Impact



**134 funded projects
awarded through Cycle 3
2021**

89 completed
45 in-progress*



**\$164 million awarded
across all projects****

4% of total research funding



544 peer-reviewed publications*

Methods publications comprise ~15% of all peer-reviewed publications from PCORI-funded studies

*as of September, 2022 ** As of August 30, 2022

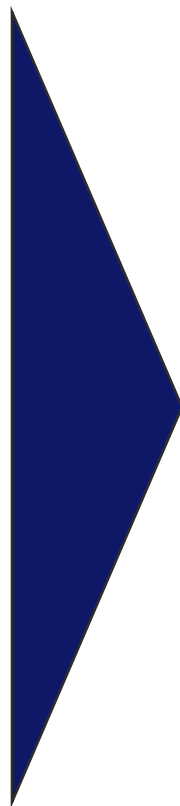
PCOR Infrastructure & Innovation: Areas of Programmatic Focus in Methods



In alignment with the PCORI Strategic Plan, new areas of programmatic focus in PCOR methods have been identified to reflect the outcomes we aim to achieve through funding of methodological study contracts.

Legacy Areas of Focus

- Improve Validity and Efficiency of Analyses (Analytic Methods)
- Patient and Stakeholder Engagement
- Evidence Synthesis
- Patient-Centered Outcomes (PCOs) and Patient-Reported Outcomes (PROs)
- Improve the Use of Natural Language Processing (NLP)



2022 Areas of Focus



Methods to Improve Study Design



Methods to Support Data Research Networks



Methods to Improve Use of Artificial Intelligence (AI) and Machine Learning in Clinical Research (new as of 20C3)

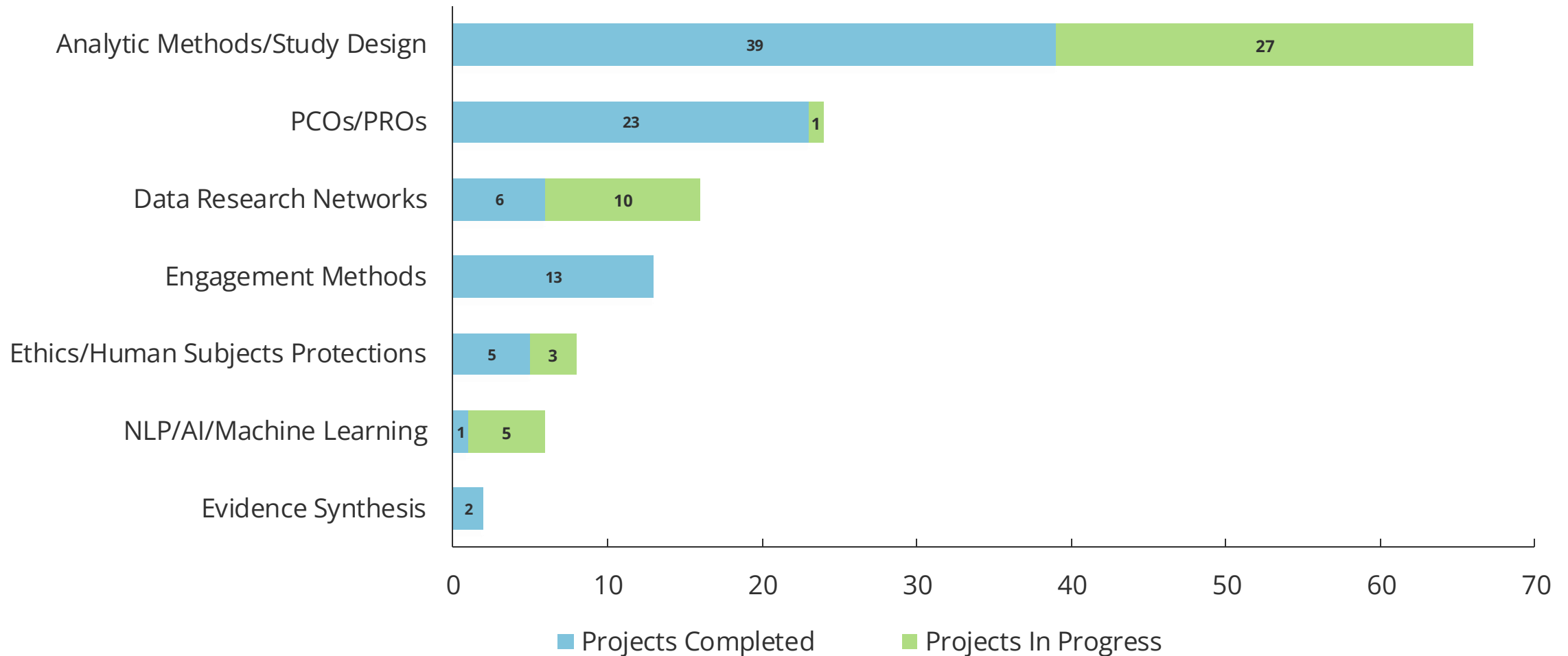


Methods Related to Ethical and Human Subjects Protection

*Explore new directions for the methods portfolio with the Methodology Committee in FY23

Number of Awarded Methods Projects Completed and In Progress, by Programmatic Area of Focus

Programmatic areas vary in the rates of project completion to date



Workforce Development

Develop, execute, and lead strategies to develop and support the PCOR workforce to lead and partner in research

- Develop portfolio of funding and strategic partnerships to support **development of training and career development programs, curricula, materials, among other resources.**
- Includes **clinicians, researchers, communities, patient and caregiver partners, and others from diverse backgrounds**
- PCORI's workforce program will conduct its work in alignment with [PCORI's commitment to diversity, equity, and inclusion](#) in all initiatives.



AHRQ-PCORI LHS Program Objectives Have Evolved

Purpose: To train clinical and research scientists to have the skills to support and lead efforts to apply PCOR methods and conduct PCOR research in a LHS and to facilitate rapid implementation of evidence that will improve quality of care and patient outcomes.

1.0 Approach

- **Develop and implement a training program** including didactic and experiential learning, that embeds scholars within LHS
- **Identify, recruit, and train clinician and research scientists** committed to conducting PCOR in health care settings
- **Establish 11 Centers of Excellence** in LHS Research Training
- **Support a learning collaborative** across funded Centers of Excellence

2.0 Approach

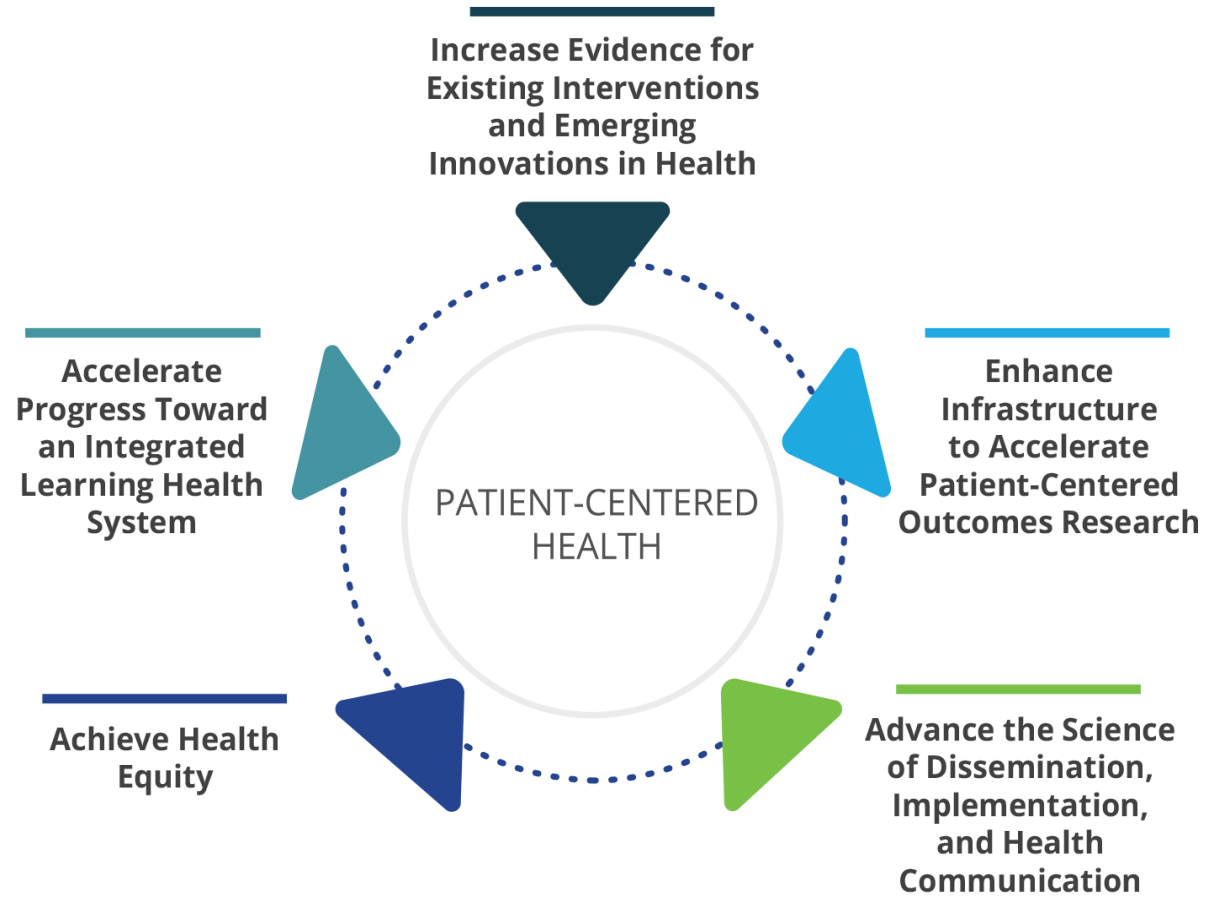
- **Establish 10 programs** to support LHS scientists
- Provide **flexible scholar appointments**
- **Expand impact by deploying a new model** to strengthen embedded research projects
- **Strengthen partnerships** between health systems, academic health centers, and community partners
- Ensure alignment of research projects with **PCORI, AHRQ, and DHHS priorities**
- Promote **use of PCORnet®**
- **Enhance the diversity** in the LHS research workforce

Next Steps to Advance the Workforce Development Discussion



- Request for Information
 - January – February 2023
- Strategic landscape assessment
 - Identify **relevant workforce initiatives** and **key agencies/organizations** leading and funding them
 - Identify **gaps & challenges** in the field
 - **Identify strategic options:**
 - where PCORI might lead efforts or where it may choose to partner on existing or new initiatives
 - where PCORI might leverage existing PCORI mechanisms to advance workforce goals
 - All in the context of PCORI's National Priorities and Strategic Plan

Methods to Support PCORI's National Priorities for Health & Strategic Plan



Methodological Challenges in Intellectual and Developmental Disabilities Meeting



MEETING SUMMARY

Methodologic Challenges in Intellectual and Developmental Disabilities Research Meeting Summary

March 15, 2021

[Meeting Details and Materials](#)

Overview

On March 15, 2021, PCORI's Methodology Committee held a public workshop to discuss methodologic issues encountered in research evaluating the effects of interventions for children and adults with intellectual and developmental disabilities (IDD). IDD is a new research priority for PCORI, which is responding to a congressional mandate in the legislation that reauthorized PCORI in 2019 for another 10 years. The purpose of this workshop was to offer an overview of IDD methodology issues.

The first session of the workshop addressed a broad range of measurement issues. Maureen Durkin, PhD, DrPH, University of Wisconsin, called for the use of social contract theory, which aims for the greatest benefit for the least advantaged members of a society, to study IDD. Dr. Durkin also described IDD definitions, types of outcomes (beyond biomedical ones) that IDD studies should measure, and categorization schemes for interventions. Tracy M. King, MD, MPH, of the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), commented that more work is needed to identify which outcomes are most meaningful to persons with IDD and how best to measure these meaningful outcomes. She also discussed that IDD studies need to measure the many levels of individual exposures, interpersonal factors, and societal influences that affect outcomes in people with IDD, and emphasized the need to measure both positive and negative outcomes.

The second session focused on observational and interventional study designs. David Mandell, ScD, University of Pennsylvania, called for merging public and private healthcare claims data with special education data from the Department of Education. Combining these data and linking them to registry data could help answer important questions about children with IDD. Luther Kalb, PhD, MHS, Kennedy Krieger Institute (KKI), described the learning health system (LHS) model and its use to analyze the data collected during the delivery of services to children with IDD. Such a system can track changes over time, collect information from several informants, and facilitate recruitment for traditional efficacy studies.

The topic of the third session was heterogeneity. Sarabeth Broder-Fingert, MD, MPH, Boston University and Boston Medical Center, described the many differences among people with autism spectrum disorder (ASD) and their families. The experiences and characteristics of families that deliver interventions to children with IDD can affect how they implement these interventions and thus the outcomes in their children. Tawara Goode, MA, Georgetown University, called for greater recognition of the diversity of people with IDD in research by, for example, not treating all persons with IDD as a homogeneous group and using approaches that produce nuanced portraits of these individuals.

- On March 15, 2021, the PCORI Methodology Committee public workshop on *methodologic issues encountered in research evaluating the effects of interventions for children and adults with intellectual and developmental disabilities (IDD)*
- Three themes:
 - Measurement issues
 - Issues in observational and interventional designs (including infrastructure needs and implementation science)
 - Heterogeneity (conditions, treatments, and effects, including disparities)
- The workshop did not address the prioritization of conditions, interventions, or research questions.

Discussion: Opportunities for the MC to Support PCORI- Agenda-Setting & Planning

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Methods Opportunities for Discussion

- **Methodology Standards**
 - Are there new standards that the MC feels are a priority to develop?
 - Standards that may need to be revisited due to evolution in the field? Revised for clarity?
- **Methods Research**
 - Strategies to update and/or develop new areas of focus for Methods? Examples:
 - New areas of focus (e.g., applied methods, health equity measurement)
 - Re-introduction of legacy areas of focus (e.g., PROs)
 - Retirement (permanent or temporary) of current areas of focus
- **Workforce Development**
 - What additional approaches could PCORI take to support development and advancement of the PCOR workforce in Methods?
 - Are there opportunities to advance new PCORI strategies or partnerships to support Methods?
- **Innovative Methods to Support PCORI's National Priorities for Health and Strategic Plan?**
- **Other?**

Adjourn

Steve Goodman, MD, MHS, PhD

Chair, PCORI Methodology Committee