



External Meeting Summary

Advisory Panel on Clinical Trials

May 13, 2022

[About this Advisory Panel](#) | [Meeting Details and Materials](#)

Overview

The Advisory Panel on Clinical Trials (CTAP) Spring 2022 Meeting provided members with the opportunity to learn about current and ongoing PCORI activities, including PCORI's progress on strategic planning, as well as provide insights from their own experiences that will help inform future PCORI initiatives. The meeting agenda included the following:

- Welcome and Goals for the Day
- Executive Director Welcome
- Strategic Planning
- Healthcare Cost and Value
- Advancing Health Equity
- Panelist Suggestions for Future Topics
- Thank You to Panelists and Closing

Executive Director Welcome

- Nakela L. Cook, MD, MPH, Executive Director, PCORI

Nakela Cook welcomed the panelists and thanked them for their contributions to recent PCORI projects and initiatives, including phased funding of research projects that grew from discussions in this panel. Cook also recognized panelists' significant contributions to PCORI's methodology standards and acknowledged the value of their input on the National Priorities for Health and Research Agenda, core components of PCORI's Strategic Plan.

Strategic Planning

- Laura Rodriguez, PhD, Deputy Executive Director for Strategy and Planning, PCORI

Laura Rodriguez reviewed how PCORI has stayed connected with stakeholders during the strategic planning process. Since the Advisory Panel's last meeting, two key benchmarks have been solidified as part of the Strategic Plan – the [National Priorities for Health](#) (adopted in fall 2021) and the [Research Agenda](#) (adopted in spring 2022). A progress update provided during the last Advisory Panel meeting had outlined goals of the Research Agenda as a framework for the comparative clinical effectiveness research (CER) portfolio that PCORI will fund in the next decade.

Rodriguez discussed components of the Strategic Plan and mechanisms for holding PCORI accountable to these goals. She then reviewed PCORI's potential approaches to sharing the recently updated Strategic Plan accessibly with stakeholders after the Plan's expected approval by the Board of Governors. After introducing the next phase of the Strategic Plan—developing the Research Project Agenda through stakeholder feedback and engagement—Rodriguez initiated a discussion with panelists on meaningful ways of engaging with stakeholder communities to solicit ideas and inform topic development for the Research Project Agenda:

What are meaningful ways to engage with stakeholder communities represented on this panel to solicit ideas and inform topic development (e.g., convenings, surveys, webinars)?

- Connecting with stakeholders in person and going beyond PCORI staff to gather feedback, for example via stakeholder engagement teams through the [PCORnet Coordinated Research Network](#) or other stakeholder engagement teams funded through their projects
- Asking researchers to inform topic development by expressing their priorities and indicating what might be important information to solicit from patients
- Using virtual meetings to reach distant or disconnected (e.g., rural) stakeholder communities

What does a responsive, transparent Research Project Agenda development process look like from your perspective?

- Building trust with stakeholders, especially given how much ground has been lost in response to the COVID-19 pandemic (i.e., due to confusion and mistrust regarding guidance, vaccines, evidence). Suggestions to address this include:
 - Looking at trusted messengers within communities as partners and working with them to both disseminate information and better understand the communities
 - Having stakeholders share the stories versus PCORI
 - Ensuring that PCORI's actions and messages are consistent and congruent with stated values, to gain trust
 - Engaging stakeholders meaningfully throughout the entire research process, including during dissemination, because people remember how they were made to feel

Given the broad, integrated nature of the adopted National Priorities for Health and Research Agenda, how could PCORI demonstrate relevance to stakeholder communities represented by this panel?

The panel did not directly address this question during this meeting; however, it may be a future point for discussion.

Based on CTAP's expertise in clinical trials for patient-centered outcomes research, what are your suggestions to stay up to date on emerging issues in this space?

The panel did not directly address this question during this meeting; however, it may be a future point for discussion.

Healthcare Cost and Value

- Greg Martin, Acting Chief Engagement and Dissemination Officer, PCORI
- Olga Khavjou, MA, Research Economist, RTI International

Greg Martin provided context by reviewing Congress's 2019 reauthorization of PCORI that emphasized PCORI and PCORI-funded investigators' responsibility to collect the "full range of outcomes data," including data on healthcare costs and value. PCORI's progress on fulfilling this mandate has included: establishing an economic resource center; conducting a landscape review on the meaning of patient-centered value in health/health care among stakeholders; and meeting with patient and stakeholder representatives via key informant interviews, small focus groups, and large group discussions. Findings from these activities will be described in a report to the PCORI Board.

Addressing the question "How can PCORI be a helpful voice in the healthcare system?", particularly on the topic of healthcare cost and patient-centered value, Olga Khavjou described PCORI's work so far in

the landscape review. When the landscape review is completed, the team will share the review's findings in a whitepaper with the 48 attributes of value that were identified. As part of this work, PCORI is continuing to solicit stakeholders' different perspectives on value with the goal of identifying components needed to measure patient-centered value.

Advancing Health Equity

- Sarah Ruiz, PhD, Senior Program Officer, Healthcare Delivery and Disparities Research, PCORI
- Vivian Towe, PhD, MSc, MA, Program Officer, Healthcare Delivery and Disparities Research, PCORI

Sarah Ruiz and Vivian Towe's presentation highlighted intersections between health equity research and PCORI's National Priorities for Health and Research Agenda, to help ensure PCORI's current and ongoing work aligns with the National Priority aim of *Achieving Health Equity*. The speakers addressed this priority in the context of CER, looking at a sample of studies across the CER spectrum to compare PCORI's research portfolio against the "Entry Points for Comparative Effectiveness Research" framework.

Towe shared two current studies related to achieving health equity: (1) [Health Aging: Optimizing Physical and Mental Functioning Across the Aging Continuum](#), and (2) [Addressing Racism, Discrimination, and Bias in Healthcare Systems and Care Delivery](#). In doing so, Towe introduced PCORI's [Caregiver-Delivered Interventions for Intellectual and Developmental Disabilities \(IDDS\)](#) and shared examples of PCORI studies using upstream funding approaches. Panelists discussed the following questions:

What are topic areas of growth for health equity research using clinical trials?

- Equity must be clearly defined because it means different things for different people. It is also important to recognize the intersectionality of people's experiences.
- Clinical trials often focus on secondary or tertiary prevention, but it would help to have greater focus on preventive care (however difficult in standard clinical trial contexts).
- It is worth exploring ways to make health equity the primary outcome or an important secondary outcome, rather than looking at health equity by assessing the heterogeneity of treatment effect (HTE).
- Panelists recommend looking at provider bias in public and community health (from both the provider and patient perspectives).
- PCORI could consider positioning communities as lead researchers since funding announcements rarely go out to community-based organizations (CBOs) themselves. People living in these communities are rarely encouraged to create a research study and solicit partners themselves, rather than simply being approached by academics.
- For research on health equity and racism, it would be good to hear evidence on what is working versus only what is not working.

What are the potential challenges of testing social determinants interventions in trials?

- It is important to be intentional and consistent in defining and using terms, and in measuring things in a meaningful way (i.e., how do we measure racism?). Trust and engagement can be difficult, and these barriers persist.
- Other challenges are related to the roles of payors (e.g., enrollment in some programs is based on what the payor funds) or other civic stakeholders. Can PCORI partner with payors if they are

willing to fund these interventions? Can PCORI partner with cities (i.e., if they are willing to fund these interventions and PCORI can fund the evaluations)?

How might clinical trials consider optimizing recruitment to ensure diverse and underrepresented populations participate in health equity research?

- Many patients would like to participate in clinical trials but have had negative experiences in trials that were not developed with participants' perspectives in mind.
- Consider ways to engage with trust messengers and/or gatekeepers in communities, to reach potential participants and keep them involved. Panelists noted that these relationships, as well as community and participant advisory panels, can be established beyond the context of a single trial to support long-term and more sustainable community-researcher partnerships.
- Connecting with stakeholders in their communities has proven very effective for some, and others have emphasized the importance of providing technical assistance or other skill- and capacity-building services to empower stakeholders in these communities. Such activities need to be accounted for in the research budget up front.

How can subgroup analyses—HTE—in CER be leveraged to advance health equity?

- In tailoring an intervention to a subgroup, data can get sliced very thin, resulting in a loss of power as more covariates are included. While increasing sample size can help account for this, it is worth considering other alternatives (e.g., machine learning).
- Future methodology and grant announcements could specifically address these issues.
- Hosting specific minority recruitment sites and closing other recruitment channels can ensure adequate sample size and focus. This should be considered at the onset of work.

What would be fruitful topics to follow up with CTAP in the future?

- Topics already suggested and related updates should be revisited in future.

Panelist Suggestions for Future Topics

- Anne Trontell, MD, MPH, Associate Director, Clinical Effectiveness and Decision Science, PCORI

Anne Trontell highlighted several topics for discussion in future CTAP meetings, including:

- Inclusion of diverse populations (particularly those facing adverse social determinants of health, including systemic racism)
- Interventions for those with IDDs
- Support for studies on social and environmental drivers of health
- Upfront considerations for trial design and conduct
- Practices for building relationships and sustaining engagement

Panelists also suggested the following topics:

- Leveraging the power of clinical trials where exposure can be controlled, and medical records or patient-reported outcomes can be used to assess long-term results
- Policy implementation and its impact on health as well as current studies or projects
- Hybrid effectiveness and implementation studies
- Translation of research results into policy interventions, and their scalability
- Trials that begin with information about the person and then build in a hierarchical analysis
- Structure that factors in personal characteristics and circumstances
- Sharing emerging practices in clinical trials related to intervention development

Thank You to Panelists and Closing

- Michael Jones, PhD, MBA, RN, Richards Endowed Graduate Nursing Chair and Research Development Coordinator, Brenau University; Advisory Panel Chair
- Anne Trontell, MD, MPH, Associate Director, Clinical Effectiveness and Decision Science, PCORI

Anne Trontell and Michael Jones thanked four panelists who have reached the end of their three-year term for their contributions.