External Meeting Summary

Advisory Panel on Clinical Effectiveness & Decision Science

May 20, 2022

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Overview

The Advisory Panel on Clinical Effectiveness & Decision Science (CEDS) Spring 2022 meeting featured updates on PCORI's CEDS research initiatives, including Intellectual and Developmental Disabilities (IDD) as a key research priority, an update on PCORI's strategic planning activities, and a panelist discussion on patient-centered value in healthcare. The meeting agenda included the following:

- Welcome and Introductions
- Overview and Update on PCORI's CEDS Research Initiatives
- Panel Discussion: PCORI's Intellectual and Developmental Disabilities Priority for Research
- PCORI's Strategic Planning Update
- Healthcare Cost and Value Presentation & Discussion
- Closing Remarks

CEDS Research Area Overview

- Holly Ramsawh, PhD, Senior Program Officer, CEDS, PCORI

Holly Ramsawh reviewed the core aim of the CEDS Advisory Panel: to support research that compares the effectiveness of various clinical options. She provided an overview of the CEDS portfolio, as well as updates regarding recent and upcoming funding announcements and research awards.

Panel Discussion: PCORI's Intellectual and Developmental Disabilities Priority for Research

- Holly Ramsawh, PhD, Senior Program Officer, CEDS, PCORI
- Amanda Barbeau, MPH, Senior Program Officer, CEDS, PCORI
- (Discussion Facilitator) Julie Eller, MPH Candidate, CEDS Advisory Panel Chair

Amanda Barbeau explained that PCORI champions Intellectual and Developmental Disabilities (IDD) research as a nationally mandated priority area through a diverse portfolio of relevant funding announcements, IDD stakeholder engagement activities, and an internal IDD workgroup aiming to identify and remedy gaps in existing research. While briefly reviewing PCORI's IDD research portfolio, she emphasized how valuable advisory panelists' insights are in helping to determine PCORI's future IDD research priorities.

One potential research topic under review would study sleep disturbances in patients with IDD. Holly Ramsawh provided background information on sleep disturbances and the current lack of clinical effectiveness research on treatment options for patients with both IDD and sleep disturbances. As PCORI is still evaluating the feasibility, relevance, and impact of this topic area, Ramsawh initiated a discussion facilitated by panel chair, Julie Eller, seeking panelists' input on meaningful comparative effectiveness research (CER) avenues and frameworks to explore for individuals with IDD and sleep disturbances:

What are the decisional dilemmas people with IDD, their caregivers, and their clinicians face that could be addressed through comparative effectiveness research (CER)? What are the important CER questions on this topic?

The panelists did not directly address the first question on decisional dilemmas during this meeting, however, it may be a future point of discussion.

- There is great difficulty in conducting effective CER on sleep disturbances in people with IDD due to a dearth of data and evidence-based interventions.
- Given both the lack of evidence-based interventions and an inadequate understanding of the diverse landscape of sleep disorders in heterogeneous IDD populations, identifying research questions and specific IDD populations for meaningful CER on this topic area must involve substantial patient, family/caregiver, and stakeholder engagement (clinicians from various specialties, researchers, advocates, etc.)

Are there specific groups or conditions that should be of focus, or should the focus be broad?

- Heterogeneous caregiver perspectives are vital for illuminating how different subpopulations of people with IDD experience sleep disturbances.
    - Even though caregivers' outcomes (i.e., physical health, family stress levels, etc.) are inextricably linked with the health outcomes of patients with IDD, they are often overlooked.

What are the most meaningful and relevant outcomes to consider?

- Because of the dearth of data on and tools for measuring outcomes of sleep disturbances in people with IDD, caregivers' outcomes are especially crucial to this potential research project because they are often neurotypical, making their sleep and wellness outcomes easier to measure with validated tools than in patients with IDD.
    - Measuring caregiver outcomes can consequently help translate the impacts of sleep disturbances on patients with IDD and their families into insightful data for CER.

Given few experts in both IDD and sleep disturbance, how do we maximize the likelihood of strong applications and ensure a robust review process?

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

What future topics should be prioritized within this research area?

- Panelists were asked to identify and rank priority areas for CER in patients with IDD. Their answers highlighted transitions for patients with IDD (i.e., pediatric to adult care), dental health, and assisted and augmentative technologies (i.e., communication devices).
    - Transitions from school to work or in living situations are typically difficult for people with IDD, especially when coupled with sleep deprivation due to sleep disturbances. More research could be done to improve care coordination for people with IDD and sleep disturbances going through transition periods.
    - Dental care is a highly unmet need for patients with IDD, with potentially dire consequences for their health when left unaddressed.

    - One panelist cautioned against an over-emphasis on assisted and augmentative technologies in the IDD research field, while others expressed that there is a need for proper caregiver training on these technologies.

Update on PCORI Strategic Planning

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

Laura Rodriguez briefly reviewed PCORI's most recent strategic planning developments, including the adoption of the National Priorities for Health, as well as PCORI's proposed Research Agenda, which panelists had the opportunity to provide feedback on during their last meeting in December 2021. Rodriguez informed the panel that after further engagement with other stakeholders, PCORI is finalizing this Research Agenda for presentation to the Board of Governors as a strategic plan for all research agenda items for the next decade. She noted that PCORI is continuing to consider how to best make the strategic plan accessible and relevant for all stakeholders.

Rodriguez then initiated a panel discussion on how PCORI can best implement diverse stakeholder feedback and engagement into the development of research agenda items (i.e., specific research questions) in an iterative, responsive, and transparent way for all stakeholder groups:

What are meaningful ways to engage with stakeholder communities represented on this panel to solicit ideas and inform topic development?

- Soliciting patients, caregivers, and advocates' input on the relevance of research topic areas in funding announcements via feedback tools such as surveys.
- Leveraging dissemination channels such as social media platforms, to be more accessible to and reflective of diverse stakeholder audiences.

How can we best ensure that engagement is inclusive of a diversity of stakeholder perspectives?

- Maintaining a continuous improvement mindset in incorporating and balancing diverse stakeholder perspectives in reviewing potential research projects.

What does a responsive, transparent Research Project Agenda development process look like from your perspective? How can we best ensure that this process is inclusive and representative of a diversity of views?

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

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?

- Panelists discussed that PCORI could develop more partnerships with other organizations to holistically improve public health and wellness, acknowledging that the overall reduction of health and socioeconomic disparities through improved food equity or employment assistance, for example, can constitute clinically effective interventions and crucial topics for CER.
- Prioritizing CER for vulnerable patient communities, such as patients with substance use disorders, is crucial.

Based on this panel's expertise in comparative effectiveness research, what are this panel's suggestions to stay up to date on emerging issues in this space?

- PCORI should become more immersed in a range of patient milieus to identify both issues important to stakeholders who may be marginalized, and how they respond to health concerns.

Healthcare Cost and Value Presentation & Discussion

- Greg Martin, Acting Chief Engagement and Dissemination Officer, PCORI
- Kristen Giombi, PhD, Research Economist, RTI International

Greg Martin and Kristen Giombi presented their ongoing research into how different groups in the healthcare sphere (e.g., payers, clinicians, patients) conceptualize patient-centered value in health and healthcare. Their goal in this work is to support researchers on PCORI-funded projects in collecting outcomes data on cost and value, by elucidating and bridging disparate notions about (and measures of) health and healthcare value. This work will then hopefully advance future opportunities for researchers, payers, patients, and providers to collaboratively develop an evidence base for the restructuring of payment models and healthcare delivery systems to prioritize patient-centered value. The following discussion ensued:

Do the direction, activities, and goals resonate with you?

- Panelists concurred that this is a crucial conversation for bridging the gap between patients' and payers' definitions of value in health and healthcare. Elevating patient perspectives on value can incentivize more robust collaboration amongst patients, payers, and other stakeholders in designing care frameworks and payment structures.
- Panelists mentioned that PCORI has the unique ability to utilize these findings in developing patient communities' capacity to advocate for their needs to payers and other stakeholders.
- Similarly, panelists concurred that promoting understandings of patient-centered value provides healthcare stakeholders with the tools to support, deliver, and benefit from quality care.

Will the activities meet the informational needs of your stakeholder community?

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

What additional considerations should be kept in mind as we proceed?

- One panelist mentioned that PCORI can capitalize on this opportunity to prioritize patient-centeredness in discussions around healthcare cost and value by offering funding or programs for health economists to engage in research on patient-centered value.

Closing Remarks

- Julie Eller, MPH Candidate, CEDS Advisory Panel Chair
- Holly Ramsawh, PhD, Senior Program Officer, CEDS, PCORI

Julie Eller and Holly Ramsawh thanked the CEDS panel members and presenters for their time and engagement in the meeting's discussions.

Eller expressed her thanks to Kari Gali for her past work as the previous panel co-chair. Ramsawh then closed out the meeting.