MEETING SUMMARY

Advisory Panel on Rare Disease Summer Meeting

June 22, 2020

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OVERVIEW

On June 22, 2020, the PCORI Advisory Panel on Rare Disease (RDAP) held its spring meeting virtually.

RDAP's 12 members include patients, caregivers, representatives of patient advocacy organizations and industry, clinicians, payers, and researchers. The meeting was open to the public via webinar, and the agenda for the meeting was posted to the PCORI website in advance.

The meeting started with brief introductions of the panel members, the PCORI RDAP staff team, and an overview of the meeting agenda. Panel members received a presentation from Nakela L. Cook, MD, MPH, an update on PCORI's National Priorities and Research Agenda, an overview of reauthorization and the cost-data provision, and an introduction to maternal mortality, intellectual and developmental disabilities, and balancing short- and long-term priorities. RDAP members discussed ideas for the RDAP to pursue in the future and recognized departing panel members, including Cindy Luxhoj, MUP, co-chair of the RDAP.

INTRODUCTION TO NAKELA L. COOK, MD, MPH

Cook introduced herself, discussed some highlights of her first few months at PCORI, and described her journey beginning from the University of Alabama up to her current role at PCORI. She noted RDAP's accomplishments including the development of the rare disease research guide, input on PCORI's guidance on research in rare diseases, recommendations on developing core outcomes for pediatric rare disease research, and development of priority areas for the rare disease PCORnet® funding announcement.

Cook highlighted future opportunities for PCORI such as big data, artificial intelligence, and machine learning. She noted that these advances are improving diagnosis and treatment and can be leveraged for PCORI-focused research. She noted that PCORI is primed to advance delivery and improve outcomes through adoption of these innovations and promote a patient-centered and community-centered approach.

Cook provided her insights on healthcare disparities and illustrated the example of a 2019 study published in JAMA. She noted the upward trend in age-adjusted mortality rates for non-Hispanic American Indians and Alaskan Natives. Cook further explained that the outcomes from the current pandemic have also elucidated the disparities in the current healthcare landscape, and highlighted PCORI’s multi-pronged approach to the COVID-19 crisis with focus on four areas: healthcare delivery, vulnerable populations, healthcare workers, and emerging health trends. She reported that PCORI is adopting many approaches to support critical work in these areas including awards such as enhancements of existing awards; solicitation of new awards and the HERO trial/registry; information sharing through webinars and collaboration with other funders; and adaptations for applicants and awardees through changes to existing projects and extending application timelines. Cook highlighted the HERO registry that has recruited over 1,400 healthcare workers and offers opportunities for research. She also informed viewers of the recent targeted funding announcement and COVID-19 enhancement projects that allow for adjustments to project aims of existing research projects to understand implications of the pandemic.

Cook described PCORI’s goals and objectives for the next phase, and listed the priority areas in the first year of reauthorization, including PCORI’s response to the COVID-19 pandemic, launching the process for national priority setting and development of a research agenda and strategic plan, as well as other priorities stemming from legislation. She also described PCORI’s integrated approach to strategic planning. Cook welcomed the RDAP’s input on national priority setting and the research agenda.

DISCUSSION

Scott Berns, MD, MPH, raised the question of how PCORI can help address the pandemic of systemic racism and implicit bias. Cook explained that PCORI has health disparities as a tenet of the national priorities, the underlying causes of which are related to the causes of broader systemic issues. She added that there is an opportunity for PCORI to further its efforts and assess how health disparities can be interwoven into its broader funding landscape.

RDAP members recommended the following topics for possible study:

- Tools to assess fatigue in patients with rare diseases
- PCORI’s engagement with rare disease patients to improve their understanding of CER.
- Increase visibility of PCORI and awareness of the work it does
- Rural telehealth and patient burden

NATIONAL PRIORITIES AND RESEARCH AGENDA

Cindy Luxhoj introduced the topic of national priorities and the research agenda, and presenter Greg Martin. Martin started his presentation by noting that national priorities are required under PCORI's authorizing law and presented relevant language from the statute. He described the mandated process for revision of national priorities that can range from 45 to 60 days, and the different stakeholders that would be engaged throughout the process. Martin also covered the current national priorities that are broad and described the process for establishing a research agenda.

DISCUSSION

RDAP members recommended the following topics for possible study:

- Increasing the relevance and awareness of CER to the average person, specifically patients and caregivers dealing with rare disease
- Partnerships between PCORI and policy makers to alleviate barriers to access to care for rare disease patients from insurance providers
- Direct engagement with rare disease patients rather than only researchers
- Rare disease application review guidance for merit reviewers
- Engagement awards targeted to patients with rare and ultra-rare diseases
- Use of engagement with stakeholders in CER studies to facilitate better dissemination and implementation of research findings

REAUTHORIZATION AND COST DATA PROVISION

Berns introduced the next topic of reauthorization and cost data provision.
Andrew Hu, MPP, started the presentation by introducing the panel to congressional leaders who championed bipartisan efforts to support PCORI's reauthorization and outlined the legislative activity involved in the process that resulted in the 10-year reauthorization. Hu provided an overview of the final reauthorization language, the changes that were made, and the two new research priorities of maternal mortality and intellectual and developmental disabilities.

Hu noted that the original authorizing legislation prohibited cost-effectiveness analysis or establishing a quality-adjusted life year threshold. The reauthorization language allows, when appropriate, capture of data on the potential burdens and economic impact of the utilization of medical treatments, items, and services for all stakeholders. This includes medical out-of-pocket costs, healthcare utilization, as well as some nonmedical costs to patients and family. The reauthorization language does not remove the prohibition of cost-effectiveness analysis or quality-adjusted life year threshold. Hu also explained the congressional and stakeholder intent behind these decisions.

Hu provided a summary of the key takeaways from early stakeholder input on implementation. He also provided an overview of the tentative implementation proposal.

DISCUSSION

RDAP members engaged in an insightful discussion with the presenters. Marcia Rupnow, PhD, noted that this would be a good opportunity to capture alternate measures like hospital utilization and ER visits that are ultimately related to healthcare costs but are meaningful to patients. Saira Sultan, JD, warned against adding a dollar value to treatments and interventions and attributing less value to treatments that are more expensive.

In response to questions from the panel members, Hu, Jean Slutsky, PA, MSPH, and Joanna Siegel, SM, ScD, provided further information on different topics of concern.

- Hu described Phase I of the cost data provision implementation and noted that guidance will be developed for applicants regarding what they can consider in their application for PCORI funding. This will be tied into a public comment period in addition to hosting a couple of panel discussions with different stakeholders like patients, consumers, payers, and healthcare systems.
- Slutsky provided a historical perspective of the cost-data provision and explained that PCORI is being deliberate about the implementation of the language in the reauthorization to ensure all perspectives around the use of cost are taken into account and that cost is not the only decision point.
- Slutsky gave an overview of what the cost of a service entails and explained that as a first step, utilization is a good denominator to which per-unit cost data can be attached. She noted that PCORI will need to figure out on behalf of the applicants as to what cost data PCORI can reasonably provide. There are arguments for and against the use of population-level cost data and also focusing on the advantage that a specific study is offering in terms of getting more accurate data on a relatively unfamiliar or unknown area.
- Siegel explained that PCORI looks forward to developing guidance on capturing patients' and caregivers' economic burden through input from stakeholders.

MATERNAL MORTALITY, INTELLECTUAL AND DEVELOPMENTAL DISABILITIES, AND BALANCING SHORT- AND LONG-TERM PRIORITIES

Luxhoj introduced the next topic of maternal mortality, intellectual and developmental disabilities, and balancing short- and long-term priorities, and the presenters.

Caitlin McCormick, JD, explained the importance of the issue of maternal mortality due to data on increasing rates of maternal mortality, particularly among Black and Native American women as well as rising concerns about maternal mortality in rural areas and the impact of closure of rural hospitals. She explained that it was clear by November 2019 that this topic would feature in the reauthorization language and PCORI acknowledged internally that the portfolio contained few studies on this important topic.

McCormick noted that for the topic of intellectual and developmental disabilities (IDD), PCORI pulled together a working group in January 2020. It is a very broad topic with great variability in the severity of the conditions as well as variation in the prevalence of different conditions. There is a focus on care transitions and PCORI is now having several conversations internally and with stakeholders to develop language for the September PFA.

McCormick explained that the topic of balancing long- and short-term priorities came from Congress' interest in achieving return on investment quickly. PCORI is holding policy salons to gather information and inform priorities.

DISCUSSION

- Nora McGhee, PhD, asked if the team could clarify the definition of IDD. McCormick explained there is much variation and many arguments about the definition so PCORI is not looking to set the definition, but rather judge research proposals on their quality.
- Scott Berns asked Greg Martin what PCORI's process of reconciling new priorities with current priorities is. Martin explained that while there are challenges, these are black and white priorities set by the Congress and PCORI is happy to pursue them. They will be reflected in PCORI's priorities and approach moving forward.
- Cindy Luxhoj mentioned that the RDAP discussed identifying cross-cutting topics last year and their findings may be useful to the PCORI team. Caitlin McCormick welcomed notes from this discussion.

FUTURE PLANNING

Scott Berns urged the RDAP members to share one idea each that they think the RDAP should pursue. Ideas generated by the panel included:

- Bundling of rare diseases that have common symptoms
- A central repository in the United States for people with rare diseases on what they should be doing or not doing
- Increased awareness of PCORI
- Leveraging oncology and rare cancer research that may be helpful in the rare diseases space
- The rare disease community can benefit from a focus on IDD
- Increasing PCORI's accessibility to the rare disease community with direct outreach to patients
- Implementation of the cost-data provision in a way that works for patients with rare and ultra-rare diseases
- More work that bundles telehealth and access to care challenges, and linking it with reimbursement policies
- Repository to identify measures to maintain continuity of care among rare disease patients during this pandemic

CONCLUSIONS

Scott Berns acknowledged the four departing members of RDAP and thanked them for their contributions.

Scott Berns and Cindy Luxhoj summarized the day's presentations and themes. The key messages received from the panelists were:

- Increasing the relevance of comparative effectiveness research to patients and caregivers dealing with rare and ultra-rare diseases

- Use of engagement with stakeholders in CER studies to facilitate better dissemination and implementation of research findings
- Increasing awareness of PCORI at the national level
- Need for a targeted rare disease engagement award
- Additional guidelines and guidance for not only merit reviewers but also applicants on rare disease CER