



January 31, 2022

Patient Centered Outcomes Research Institute
1828 L Street, NW, Suite 900
Washington, DC 20036

Re: PCORI's Proposed Research Agenda

Dear Members of the PCORI Board of Governors:

On behalf of the more than 30,000 individuals living with cystic fibrosis in the United States, we write to provide public comment on the Patient Centered Outcomes Research Institute (PCORI) Proposed Research Agenda.

About Cystic Fibrosis & the Cystic Fibrosis Foundation

Cystic fibrosis (CF) is a severe, progressive genetic disease that affects over 30,000 children and adults in the United States. CF causes the body to produce thick mucus that clogs the lungs and other organ systems, which results in lung damage, life-threatening infections, and other complications. As a complex, multi-system condition, CF requires targeted, specialized treatment regimens. Despite significant advances in CF care, CF remains a life-shortening disease.

As the world leader in the search for a cure for CF, the Cystic Fibrosis Foundation's mission is to give all people with CF the opportunity to lead long, fulfilling lives. We do so by funding research and drug development, advancing high-quality, specialized care, and partnering with the CF community to ensure that people with cystic fibrosis and their families have the tools, resources, and support they need to thrive. Our approach is collaborative and patient-focused; for that reason, we appreciate PCORI's mission and research efforts.

We are pleased to have the opportunity to review and share our thoughts on this Proposed Research Agenda and are encouraged to see that it addresses several high priority topics for the CF community. These include forming strong ties with patient communities, building and supporting a diverse workforce, reducing health and healthcare disparities, and finding new ways to educate individuals about research developments. Given our overall alignment on these subjects, we would like to offer several recommendations based on the CF Foundation's effort and experiences.

Methods of communication that promote inclusion and equity

Given its intention to fund research that fills patient-prioritized evidence gaps and is representative of diverse patient populations and settings, PCORI should consider prioritizing research projects focused on methods of fostering and improving communication with patient communities – with an emphasis on reaching and engaging with traditionally underrepresented patient populations. Understanding how to effectively communicate is a critical step for gathering information about patient population needs and priorities, which will help guide subsequent PCORI research projects. In that vein, the CF Foundation is also focused on better understanding and improving our communication with people of color with CF, who have traditionally been underrepresented in our community; this work requires building trust and using methodology beyond what is traditionally used to engage these populations.

Involvement of varied and diverse healthcare stakeholders in research study design

We are pleased to see that throughout the agenda PCORI identifies the importance of including a variety of stakeholders in the work to understand and fill research needs. We further would like to emphasize that, if possible, projects funded by PCORI should make it a priority to consult these stakeholders throughout the research process, including design and recruitment. By supporting research that includes these perspectives, PCORI helps ensure data from these projects are actionable and applicable to real-world decision-making processes.

With that in mind, it is also essential that studies funded by PCORI recognize the myriad of populations within the patient community and the ways in which their needs converge or diverge. This diversity should be represented within the research itself, but also in those working on the projects. The CF Foundation encourages PCORI to ensure that project teams are representative of the patient populations being studied.

Improving representation in the research and healthcare workforce

We appreciate and fully agree with the emphasis that PCORI's Proposed Research Agenda places on recruiting diverse and underrepresented populations into the research and healthcare workforce. The CF Foundation itself is currently engaging in efforts to build a diverse research and healthcare workforce and would advise PCORI to expand their research focus to include projects that explore methods of supporting, advancing, and retaining researchers and healthcare workers following their initial recruitment. The importance of retention efforts was recently brought to light when researchers, using data from the National Science Foundation, found that a failure to retain non-White scholars was a significant contributor to their lack of representation in later stages of academia. Supporting research to combat this retention failure will therefore bolster PCORI's efforts to improve workplace representation.

Effective dissemination and communication of research findings

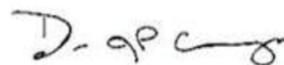
The CF Foundation acknowledges and applauds PCORI's commitment to advancing methods of communicating evidence so the public can access, understand, and act on research findings. As PCORI works to find effective ways to translate and communicate research findings to patients, it should consider how to best leverage patient-provider relationships. From our work examining communications about potential treatment advances in CF—particularly genetic therapies—we have found that clinicians are a critical, trusted part of the communication process with patients. However, CF clinicians sometimes have limited knowledge of future treatment innovations or are not sure how to engage their patients in conversations about these topics. Given that clinicians are key messengers in the process of informing and advising patients about developments in clinical treatments and research, PCORI should consider how to empower providers to actively engage in discussions spanning research teams and patient communities.

The Cystic Fibrosis Foundation appreciates the opportunity to provide input on this important research agenda. We stand by to answer any questions you may have. Please contact Olivia Dieni, MPH, at odieni@cff.org or 240-200-3715 to discuss further.

Sincerely,



Mary B. Dwight
Chief Policy & Advocacy Officer
Senior Vice President of Policy and Advocacy



JP Clancy, MD
Vice President, Clinical Research