

June 6, 2023

Honorable Robert M. Califf, M.D.  
Administrator  
Food and Drug Administration  
10903 New Hampshire Ave  
Silver Spring, MD 20993-0002

Dear Administrator Califf:

Thank you for this opportunity to submit comments on the FDA's proposed revision of guidance on the voluntary use of patient preference information (PPI) in regulatory submissions. Since its founding, the Partnership to Improve Patient Care (PIPC) has been at the forefront of applying principles of patient-centeredness to the nation's health care system – from the generation of comparative clinical effectiveness research at the Patient-Centered Outcomes Research Institute (PCORI), to the translation of evidence into patient care in a manner that achieves value to the patient. We applaud the FDA's attention to the concepts of patient-centeredness and patient engagement in the conduct of research. PIPC looks forward to continuing its efforts to bring the voices of patients and people with disabilities to the discussion of how to advance patient-centered principles throughout an evolving health care system.

PIPC has long advocated for increased use of patient preference information to guide health care decisions. In the statute creating the Patient-Centered Outcomes Research Institute (PCORI), Congress defined research priorities to include patient needs, outcomes and preferences" and charged PCORI to consider "variations in patient subpopulations," as well as to support patient and consumer representatives.<sup>1</sup> PCORI has since funded several patient preference information studies, including studies looking specifically at novel methods.<sup>2</sup> We urge FDA to review and incorporate as appropriate best practices from PCORI's work as FDA revises its PPI guidance.

Also, the Medical Device Innovation Consortium has specifically invested in developing a patient-centered framework for patient preference studies tailored for medical devices and useful for the regulatory submissions. Their framework, *Using Patient Preference Information in the Design of Clinical Trials*, outlines a systematic approach for patient-focused clinical trial design to better meet patient needs and priorities. They concluded as follows:

The experts identified several key considerations for industry sponsors and regulators interested in applying PPI to clinical trial design, including: (1) pursuing existing opportunities to work with regulators to incorporate PPI in regulatory decision-making,

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<sup>1</sup> <https://www.pcori.org/sites/default/files/PCORI-Authorizing-Legislation.pdf>

<sup>2</sup> [www.pcori.org](http://www.pcori.org)

(2) identifying novel endpoints for patient preference studies, (3) aligning on the “crosswalk” between attributes selected for a patient preference study and end-points used in a clinical trial, (4) ensuring the applicability of PPI to the specific population who will use the medical device under study, and (5) applying the most appropriate methods to leverage PPI to inform the statistical evaluation of trial data.<sup>3</sup>

As factors for designing a patient preference study, we urge reference to pre-existing work on identifying outcomes that matter to patients and people with disabilities and the burdens they experience as a starting point. For example, PCORI is expected to finalize in the near future a report entitled, *Stakeholder Views on Components of “Patient-Centered Value” in Health and Health Care*, that may provide insights useful to sponsors of a PPI study.<sup>4</sup> Additionally, Everylife Foundation for Rare Diseases conducted a comprehensive assessment of the total economic burden of rare diseases (RD) in the United States (U.S.) in 2019 that may provide insights on preferred outcomes, such as impacts on work productivity.<sup>5</sup> Another resource is the Innovation and Value Initiative (IVI), which has invested significant resources into methods to identify and incorporate patient preferences into healthcare research.<sup>6</sup>

There is no substitute for engaging with patients and people with disabilities that have personal experiences with the disease or illness to be treated in the development of a patient preference information study. It is essential to not only understand the common preferences among patients, but also to understand how preferences may be weighted differently among patient subpopulations. As the U.S. seeks to reduce health disparities and promote health equity, understanding how social determinants of health and social identities impact care experiences should also advance a better understanding of the heterogeneity of a treatment’s benefits for subpopulations, and potentially influence the chosen end-points of a clinical trial.<sup>7</sup>

I hope that this information is useful to the FDA as it considers revisions to its voluntary PPI guidance.

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<sup>3</sup> [https://link.springer.com/epdf/10.1007/s43441-022-00450-9?sharing\\_token=fB\\_oerf4E8hSRY\\_1mwyamve4RwlQNchNByi7wbcMAY4FgWWMiYCnQsE3upSBL6qYy1R--0uMj8ZwmzTeBo0aeUTIHmbpXMIIYoU5-JDNf263KJakdftgWtJKP8UVAR1FOwhY9tQziwzos9Sj4OGIGHXOu7Jeu3Vc6b93Z8NyGKs=](https://link.springer.com/epdf/10.1007/s43441-022-00450-9?sharing_token=fB_oerf4E8hSRY_1mwyamve4RwlQNchNByi7wbcMAY4FgWWMiYCnQsE3upSBL6qYy1R--0uMj8ZwmzTeBo0aeUTIHmbpXMIIYoU5-JDNf263KJakdftgWtJKP8UVAR1FOwhY9tQziwzos9Sj4OGIGHXOu7Jeu3Vc6b93Z8NyGKs=)

<sup>4</sup> <https://www.pcori.org/about/provide-input/past-opportunities-provide-input/stakeholder-views-components-patient-centered-value-health-and-health-care-2023>

<sup>5</sup> [https://everylifefoundation.org/wp-content/uploads/2022/04/Orphanet\\_Journal\\_of\\_Rare\\_Diseases.pdf](https://everylifefoundation.org/wp-content/uploads/2022/04/Orphanet_Journal_of_Rare_Diseases.pdf)

<sup>6</sup> <https://thevalueinitiative.org/let-the-work-begin/>

<sup>7</sup>

[http://www.pipcpatients.org/uploads/1/2/9/0/12902828/aligning\\_health\\_technology\\_assessment\\_with\\_efforts\\_to\\_advance\\_health\\_equity.pdf](http://www.pipcpatients.org/uploads/1/2/9/0/12902828/aligning_health_technology_assessment_with_efforts_to_advance_health_equity.pdf)

Sincerely,



Tony Coelho, Chairman  
Partnership to Improve Patient Care