Biotechnology Innovation Organization

October 30, 2020

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: Docket No. FDA–2020-D-1564: FDA Draft Guidance, Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation.

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding the Draft Guidance, *Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome (PRO) Instruments for Use in Medical Device Evaluation*.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to prevent them in the first place.

BIO appreciates the efforts of both the Center for Devices and Radiological Health (CDRH) and the Center for Biologics Evaluation and Research (CBER) for the development of the guidance on patient reported outcome (PRO) instruments for use in medical product evaluation. Such guidance is important for supporting patient-centric medical product development and review. BIO recognizes that while PROs are an important tool for collecting and reporting patient experiences, we believe that a patient-centric approach to drug review includes other clinical outcome assessments (COAs) in addition to PROs (e.g., observer, clinician, performance). We encourage CDRH and CBER to consider these other types of COAs when finalizing the guidance. BIO also recognizes there are similar efforts underway in the Center for Drug Evaluation and Research (CDER) focused on the incorporation of patient experiences, and COAs in particular, into drug development and review. While we recognize that the Draft Guidance references CDER guidance, we encourage the Agency to leverage learnings and principles for COA development and other methods (e.g., focus groups, patient interviews, qualitative research, preference studies) for collecting patient experiences, including patient preference, across FDA Centers. Furthermore, we request that CDRH make it more clear in the guidance the type of evidence needed to demonstrate that a PRO is sufficiently validated for the purpose for which it is being used and is acceptable for inclusion in labeling. When referencing CDER guidance, we request that CDRH make it clear whether the evidence required is the same or different as outlined in CDER guidance.

While BIO notes that the guidance applies to devices regulated by CDRH and CBER we encourage the Agency to describe their expectations for the incorporation of patients' perspectives and experiences into the development and use of digital health technology tools (DHTTs) used for data collection in clinical trials that would not be classified as medical

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devices (e.g., wellness apps, commercial wearables). Consideration of such data will help to ensure that these clinical trials are conducted in a patient-centric manner and that data collected with DHTTs can support regulatory decision-making. In addition, BIO encourages FDA Centers to enhance, or modify as appropriate, the FDA COA roadmap[1] to incorporate digitally derived endpoint considerations.

Finally, BIO recognizes that the guidance considers a range of practices and circumstances that may exist in the context of PRO validation and relevance to clinical data. In particular, the guidance notes that generation of validity evidence as part of a pivotal study may not support specific statements regarding safety and/or effectiveness in that pivotal study in the labeling or public summaries.[2] Instead, FDA recommends that validity evidence generated as part of, or during, the clinical study may be supportive of use of the same PRO instrument in future studies.[3] In general, we agree that it is preferable for a PRO instrument's validity evidence to be generated prior to the pivotal trial where the PRO measurement will be specified as a key study endpoint to support medical product labeling claims. However, in some circumstances, it may be necessary – and sometimes is requested by FDA – that specific measurement characteristics be confirmed in tandem with ongoing clinical research. We therefore request that the final version of the guidance clarify some of these points, as reflective of ongoing practices around PRO development and use to support study data, including, potentially, labeling claims.

BIO appreciates this opportunity to submit comments regarding FDA's Draft Guidance, *Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation*. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/
Danielle Friend, Ph.D.
Senior Director, Science and Regulatory Affairs
Biotechnology Innovation Organization

[1] FDA Roadmap on Roadmap to Patient-Focused Outcome Measurement in Clinical Trials.
[2] Lines 277 to 281.
[3] Id.