March 21, 2019

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

Re: Docket No. FDA-2018-D-4455: FDA Draft Guidance, Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data

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(BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding the Draft Guidance on Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data.

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 members are involved in the research and development of innovative healthcare, agricultural, industrial, and environmental biotechnology products.

BIO thanks the Agency for promoting partnership between patients, industry, and the FDA by providing patient groups and other stakeholders with a pathway to submit proposed draft guidance and other resources related to patient experience data to the FDA. We welcome the opportunity to integrate information generated by patient groups and other external stakeholders into our drug development programs, and also seek to contribute to the open exchange of information regarding patient's experiences. We encourage FDA to continue to facilitate the open exchange of patient experience data with all stakeholders such that clinical science is able to advance towards the development of outcomes that are the most meaningful to patients.

BIO believes that strong trilateral communication between the FDA, patients and patient organizations, and Sponsors around patient experience data will result in incorporation of patient experience data throughout the product lifecycle, including drug development and review. BIO appreciates that the Agency included Table 1 (beginning on line 423) in the Draft Guidance as it provides examples for all stakeholders regarding how patient experience data may play a role in the drug development lifecycle; however, as presented, the key stakeholders appear to function in isolation of each other rather than showing how the trilateral communication between patient stakeholders, medical product developers/researchers, and regulators results in effective collection and use of patient experience data. To better support trilateral communication, BIO requests that the FDA clearly outline a process by which the FDA will communicate and provide feedback to stakeholders that submit draft guidance or other patient experience data. Draft guidance development by an external stakeholder (including patients and patient organizations) is time and resource intensive and, for example, if a patient organization submits a draft guidance and the FDA makes the determination to only use some information from the submitted draft guidance, it would be helpful for the FDA share the rationale as to why only portions of the submitted draft guidance will be supported by the Agency. To further support trilateral communication, BIO requests that the Agency communicate how proposed draft guidance

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submitted by external stakeholders is leveraged to influence thinking related to development of draft guidance by the Agency. We acknowledge that this level of communication is time-consuming, but it will help inform efforts by all stakeholders if the Agency provides feedback around why specific data were or were not utilized.

The Draft Guidance also indicates that if a draft guidance is submitted to the Agency, the FDA will open a docket for stakeholder's comments. To support trilateral communication, BIO also requests that when such a docket is opened, the FDA also post notification of that docket in the Federal Register.

Finally, BIO also appreciates that the Draft Guidance continues to emphasize that the FDA encourages stakeholders to contact FDA staff early to discuss patient experience data that may be useful to collect and submit to the FDA; however we note that the FDA has not articulated a process by which Sponsors and patients and patient organizations may engage with the Agency around patient experience data. BIO requests that the Draft Guidance provide details regarding who and when Sponsors' and other stakeholders may engage around patient experience data for the purposes of developing a draft guidance but for other purposes as well (e.g., patient experience data for regulatory decision-making). Along these lines, it would also be helpful for the Agency to clearly indicate how CDER, CBER, and FDA Patient Affairs Staff will be engaged in various interactions with stakeholders around patient experience data. One way that the FDA may clearly provide this information is through a chart or a decision tree based upon the types of stakeholder (e.g., patient, patient organization, or Sponsor), the purpose for which the patient experience data will be used, as well as the time point in the drug development/review lifecycle during which the patient experience data will be collected, analysed, used and submitted.

BIO appreciates this opportunity to submit comments regarding FDA's Draft Guidance, Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

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

SPECIFIC COMMENTS

SECTION	ISSUE	PROPOSED CHANGE
I. INTRODUCTION		
II. BACKGROUND		
Line 110	This section includes reference to questions that will be addressed in the Draft Guidance, and in particular the FDA indicates: "It is important to also understand when stakeholders can communicate with FDA."	BIO requests that the FDA consider the following edit: "It is important to also understand **how and when** stakeholders ~~**can**~~ **should** communicate with FDA." BIO also requests that the FDA provide details regarding the various pathways available for all stakeholders to engage and communicate with the FDA regarding patient experience data.
III. QUESTIONS AND ANSWERS		
Lines 183-186	In this section, the FDA lists several questions that stakeholders should consider when developing planning to develop a draft guidance and the FDA indicates that stakeholders should ask whether there "Are resources, expertise, and stakeholder capacity available to collect any relevant patient experience data, conduct required analysis, and further develop a proposed draft guidance? Would the available resources be more suitable to focus on other efforts (e.g., those discussed in Section II, Other Opportunities for Stakeholders)?" There should be some additional discussion of the data needed to inform the guidance proposal, and	BIO requests the FDA to consider the following edit: "Are resources, expertise, and stakeholder capacity available to collect any relevant patient experience data, conduct required analysis, and further develop proposed draft guidance? **Stakeholders may wish to consider information such as patients' burden of disease, natural history of disease, unmet treatment needs, or lack of available data upon which to make treatment decisions. This information may be submitted with the guidance proposal in the form of a study report, along with appropriate descriptive statistics or qualitative quotes or tables. The methods for collecting and analyzing this data are outlined in the FDA Patient-**

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	how this information should be presented in the proposal, otherwise it may be difficult for stakeholders to understand what is required and whether they have sufficient resources to undertake the work.	**Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making."**
Lines 188-190	This section indicates that the FDA encourages contacting FDA staff early to discuss patient experience data that may be useful to collect and submit to the FDA; however the mechanism for contacting FDA to discuss these questions has not been elucidated, nor is the format, timing for such contacts described, thus is will be difficult for stakeholders to obtain appropriate feedback prior to making a decision to collect data and/or develop and submit a guidance proposal. Specifically, it would be helpful for the FDA to indicate which FDA staff (e.g., review division staff) sponsors should contact to discuss plans for patient experience data.	BIO requests that the FDA consider the following edits: "FDA encourages contacting FDA staff early to discuss patient experience data that may be useful to collect and submit to the FDA. Please refer to the series of FDA Patient-Focused Drug Development guidances before collecting patient experience data. **Stakeholders interested in discussing their plans for collecting and submitting patient experience data to FDA in support of a proposed guidance or other regulatory purpose should submit their request to [FDA to specify relevant FDA office/division]. The request should include a brief description of the condition, patient population, any gaps in available information regarding patient experience or needs, and the proposed methods for collecting and analyzing the data, if a collection tool is used, describe if it has been previously validated as well as a list of questions the stakeholder would like to discuss with FDA staff, the desired meeting format (face to face, telephone/video conference, WRO), and suggested meeting dates and times. FDA will respond to meeting requests within X days of request receipt."**
Lines 193-196	In this section, the FDA lists several questions that stakeholders should consider when developing or planning to develop a draft guidance and the FDA indicates that stakeholders should ask if patient	BIO requests the FDA to consider the following edits: "If patient experience data has been collected and analyzed, is it suitable to provide recommendations or considerations

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	experience data have been collected and analysed, and whether is it suitable to provide recommendations or considerations to include in a proposed draft guidance? The FDA indicates that if not, stakeholders should consider other opportunities to share patient experience data (see Section II, Other Opportunities for Stakeholders), however, there is a lack of information regarding what is suitable to support a proposed guidance which would inform stakeholders' decisions regarding the use of existing data for this purpose.	to include in a proposed draft guidance? If not, stakeholders should consider other opportunities to share patient experience data (see Section II, Other Opportunities for Stakeholders). **Data suitable to support the recommendations or considerations in a proposed guidance should be collected using sound methods as outlined in the FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making https://www.fda.gov/drugs/developmentapprovalprocess/ucm610279.htm, should demonstrate the need for additional guidance based upon patient experience, and should be representative of the patient population in question."** BIO also requests the FDA to provide additional detail regarding factors that should be used to determine whether patient experience data is suitable to provide recommendation or considerations to include in a draft guidance.
Lines 240-251	In this section the FDA provides guidance on what general considerations regarding the format of a proposed draft guidance and indicates that stakeholders should include an introduction clearly stating the purpose of the proposed draft guidance; provide high-level background that includes a brief description of the disease, patient population, severity of the condition, available treatment options, and topics or issues to be addressed in the document; and propose considerations and recommendations on the topics or issues relevant to	BIO requests the FDA to consider the following edit: "Include an introduction clearly stating the purpose of the proposed draft guidance; provide high-level background that includes a brief description of the disease, patient population, severity of the condition, available treatment options, **gaps in available information or knowledge regarding patient experience,** and topics or issues to be addressed in the document; and propose considerations and recommendations on the topics or issues relevant to the purpose of the proposed draft guidance."

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	the purpose of the proposed draft guidance, however in this section the FDA does not indicate that stakeholders should include a statement addressing the gaps that the guidance will fill.	
Lines 262-263	The FDA indicates in this section that stakeholders should include a study report and protocol when submitting patient experience data as supporting information with a proposed draft guidance. The FDA indicates that if applicable, stakeholders should also include additional information such as the primary data capture, however, as the statement currently stands, it is not clear what it means in this context. Specifically, the FDA's reference to the population sampled, methods of primary data collection.	For clarification, BIO requests the following edit: "Include a study report and protocol when submitting methodologically collected patient experience data as supporting information with a proposed draft guidance. ~~**If applicable, also include additional information such as the primary data capture.**~~"
Lines 291-295	As the FDA can appreciate, development of a proposed draft guidance related to patient experience data by external stakeholders (including patients and patient advocacy groups) is extremely resource intensive. To further enhance transparency, we request that the Agency communicate how proposed draft guidance submitted by external stakeholders is leveraged to influence thinking related to development of draft guidance by the Agency.	BIO requests that the FDA consider the following edits: "Submission of a proposed draft guidance to FDA does not mean that FDA will publish its own draft guidance on the topic(s) identified. FDA has its own process for developing guidance based on several factors including the state of the science in a given area, policy priorities, and Agency resources. The stakeholder-submitted proposed draft guidance can be used to inform FDA's thinking and future guidance development work, if applicable. **If FDA decides to publish draft guidance following receipt of a proposed draft guidance from external stakeholders, the Agency will explain how the proposed guidance influenced the published draft guidance, including rationales for any key concepts in the proposed draft guidance that were included or excluded from the Agency's draft guidance. The mechanism for**

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		communicating this explanation will be at the discretion of the Agency and could include the Federal Register notice announcing the draft guidance or a teleconference with the submitter of the proposed draft guidance."
Development and Submission of Proposed Draft Guidance Relating to Patient Experience Data		
Other Opportunities for Stakeholders		
Lines 423-424, Table 1	Table 1 appears to be missing categories of patient experience data.	BIO requests that the FDA consider another line in the table for patient input regarding proposed risk minimization activities (including design and pilot testing of such measures). BIO also requests that the FDA insert another line for patient experience data regarding their experience with actual (i.e., implemented) risk minimization activities, how effective they have been and how patients would suggest modifying them further to improve them.