March 15, 2020

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

Re: Docket No. FDA–2019-D-4752: FDA Draft Guidance, Pediatric Study Plans for Oncology Drugs: Questions and Answers.

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(BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding the Draft Guidance, Pediatric Study Plans for Oncology Drugs: Questions and Answers.

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 FDA's efforts to develop a document that outlines questions and answers pertaining to Pediatric Study Plans (PSPs) for oncology products especially in light of the upcoming implementation date for the new pediatric oncology requirements. However, it is unclear whether FDA plans to develop a final version of the draft Q&A guidance, given that many of the questions and answers provide information to drug developers regarding how to prepare and submit PSPs for FDA depending upon whether the marketing application is submitted prior to or after August 18, 2020. BIO sees value in FDA finalizing the guidance and making it available even after August 18, 2020, therefore we request FDA update the Draft Guidance based upon feedback from stakeholders to this docket.

BIO also notes that as written the Draft Guidance is geared more towards drug developers who may be less familiar with the new pediatric oncology requirements. While BIO recognizes value in ensuring that drug developers who are less familiar with the new requirements have resources needed to prepare for implementation,

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BIO has identified additional areas that FDA may consider including in a updated version of the Guidance that will also make the guidance a helpful resource for drug developers who are more familiar with the new requirements, including:

1. In the Draft Guidance FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of FD&C Act, FDA indicates that "*More definite evaluation of a product, if warranted based upon the initial pediatric evaluation described in the iPSP, may be the subject of a Proposed Pediatric Study request (PPSR). Following review of the PPSR and discussions with the sponsor, FDA may issue a Written Request, if appropriate.*" BIO requests that FDA provide additional detail in the Draft Guidance on Pediatric Study Plans for Oncology Drugs: Questions and Answers regarding the sequence of events that may be required for a sponsor to seek a Written Request. Specifically, the text in the previously published FDARA 504 Implementation guidance may suggest that sponsors cannot seek a Written Request until they have satisfied any FDA requirement for a pediatric investigation as described in FDARA Section 504. However, this approach would not allow sufficient time to negotiate a Written Request and complete the requested studies. When the Q&A guidance is finalized, BIO requests that FDA include a question and associated response indicating that sponsors may ask FDA to issue a Written Request at any point in the development of an oncology drug, that the Written Request process for oncology drugs is flexible and should proceed in parallel or consecutively after the iPSP, and that molecularly targeted pediatric cancer investigations be considered as part of the PPSR, in addition to other required investigations.

2. While in the Draft Guidance on Pediatric Study Plans for Oncology Drugs: Questions and Answers FDA indicates that Sponsors should use the existing PSP template for information pertaining to the new pediatric oncology requirements, there is a lack of detail regarding what information should be included in each section of the PSP. Specifically, BIO believes that the information pertaining to substantial relevance of the particular molecule to pediatric cancer as well as a summary of the regulatory history and previous discussions with the FDA can be included in section 2, "Overview of the Drug or Biological Product" of the PSP template, requests for waivers and deferrals can be included under section 4, "Planned Request for Drug-Specific Waiver," and information pertaining to the planned preliminary efficacy study can be included under section 10 "Planned Pediatric Clinical Studies" in the PSP template. BIO requests that FDA include this information in the final version of the Guidance. Additionally, there are some sections of the existing PSP that may not be relevant or applicable to the new requirements (e.g., Section 3 of the PSP template, "Overview of Planned Extrapolation of Effectiveness to Specific Pediatric Populations" and Section 10.2 of the PSP template "Planned Pediatric Clinical Studies-Clinical Effectiveness and Safety Studies Planned"). BIO requests that FDA indicate in the final version of the guidance that such sections may not need to be populated in the context of the new pediatric oncology requirements.

BIO appreciates the opportunity to submit comments regarding FDA's Draft Guidance, Pediatric Study Plans for Oncology Drugs: Questions and Answers. 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