Biotechnology Industry Organization

June 9, 2014

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

Re: Docket No. FDA-2014-D-0313: Draft Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff: Meetings with the Office of Orphan Products Development

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) is pleased to submit the following comments on the draft guidance issued by the Food and Drug Administration (FDA) on April 9, 2014, entitled "Draft Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff: Meetings with the Office of Orphan Products Development" (the "Draft Guidance").[1]

BIO represents more than 1,000 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, thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment.

We want first to express our continuing appreciation for the specific attention paid to the unique challenges of developing therapies for rare diseases. Recognizing the differences between how drugs and biologics for rare diseases can be developed—given the small patient populations—and drug development for non-rare conditions is crucial to the creation and implementation of innovative strategies to overcome these challenges. The designated Office of Orphan Products Development (OOPD) is an important aspect of this recognition, as it offers manufacturers of rare disease therapies a dedicated interface with the Agency and a central coordinator throughout the regulatory review process.

We commend FDA for establishing concrete expectations for interactions with OOPD and believe the Draft Guidance is important in order to establish a common understanding of the goals and scope of both informal and formal meetings between the Agency and

[1] U.S. Food and Drug Administration (FDA). 2014 (April 9). Draft Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff: Meetings with the Office of Orphan Products Development. Silver Spring, MD: FDA, Available at: http://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/OOPDNewsArchive/UCM392593.pdf. [Hereafter "FDA Draft Guidance."]

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manufacturers. While the Draft Guidance is, on the whole, a thorough and thoughtful document, we offer several suggestions that we believe will further the FDA-stated aim of providing for "consistent procedures to promote well-managed meetings".[2] We identify three areas below in which we think the Draft Guidance would benefit from greater clarity and specificity.

First, it is unclear whether the Draft Guidance applies only to original requests for orphan designation or if it is also meant to apply to planned resubmissions or pending applications. This lack of clarity comes from inconsistencies in the text of the Draft Guidance. For example, while FDA states that the formal meetings herein described "are not a forum … for OOPD to determine whether information in a pending or forthcoming designation request or grant application is complete and adequate",[3] the Draft Guidance regardless goes on to specify the meeting materials stakeholders should provide "if the requested meeting concerns a designation request or grant application that has already been submitted to OOPD".[4] Accordingly, the latter appears to suggest that requests for meetings on these subjects—orphan designation request or grant application already submitted—are appropriate, without limiting the scope of those requests, as seems to be the intent of the former statement. Therefore, BIO asks FDA to clarify the appropriate scope of requests for informal and formal meetings governed by the final guidance. Moreover, we urge the Agency to include within that scope issues related to planned resubmissions. If excluded, however, the FDA should establish an equally comprehensive guidance on communicating with OOPD on planned resubmission since it would be beneficial to ensure clear avenues for discussion between manufacturers and the Agency, especially in the case of a resubmission in which the history is complicated with an orphan subset.

If requests for meetings regarding pending applications are within the scope of the final guidance, BIO asks the Agency also to include a process whereby manufacturers can discuss the necessary contents of the meeting package in advance of its assembly and submission to OOPD. This is especially important in the case of meetings regarding pending applications because many aspects of the meeting package identified in the Draft Guidance may have been submitted already as part of the application submission. Establishing a step in the process leading up to the submission of the meeting package, which allows a preliminary discussion (likely electronically) between a manufacturer and OOPD, would serve to improve efficiency and avoid unnecessary redundancy.

Second, we note that the meeting timelines included in the Draft Guidance are not comprehensive. For example, while the Draft Guidance notes that "OOPD will aim to respond to a meeting request within 5 working days of receipt",[5] the Draft Guidance does not identify a target timeframe within which the meeting, formal or informal, should be scheduled after the request is received. Additionally, while the Draft Guidance establishes a timeline for the submission of draft meeting minutes to OOPD (15 working

[2] FDA Draft Guidance at 2.
[3] Id. at 3.
[4] Id. at 5.
[5] Ibid.

days) and for the initial OOPD comments on that draft ("generally within 15 working days"),[6] there is no timeframe established for the completion of those minutes should edits need to be made after these initial drafting and comment periods. BIO asks FDA to include comprehensive timeline details in the final guidance, as we believe that the timeliness of informal and formal communications between manufacturers and OOPD is crucial to ensure the efficient progress of the regulatory review process.

Finally, FDA should clarify that the same obligations—regarding meeting package content and submission, protocol, and documentation—apply to both Sponsors and OOPD if OOPD, rather than a Sponsor, requests a meeting that is governed by this guidance. As we noted above, BIO shares the Agency's goal of achieving productive, well-managed meetings no matter how the meeting is initiated.

In conclusion, BIO appreciates the opportunity to comment on this Draft Guidance on "Meetings with the Office of Orphan Products Development." We look forward to continuing to work with FDA, and OOPD specifically, to ensure that communications between the Agency and stakeholders are timely, efficient, productive, and meaningful. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/

Andrew J. Emmett
Managing Director, Science and Regulatory Affairs
Biotechnology Industry Organization (BIO)

[6] FDA Draft Guidance at 7.