Biotechnology Industry Organization

January 22, 2013

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

**Re: Docket No. FDA–2012-N-0974: Development of Prioritized Therapeutic Data Standards; Request for Comments**

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to submit comments on the "Development of Prioritized Therapeutic Data Standards (the Notice)." BIO supports FDA's efforts to implement the PDUFA V performance agreement provisions regarding the development of standardized clinical data terminology to facilitate the conduct of clinical research and regulatory review of medical products. BIO welcomes the availability of and the opportunity to comment on the FDA's Therapeutic Area Data Standards Roadmap (Roadmap) which will help facilitate the Agency's creation of a plan for distinct therapeutic area data standards.

BIO represents more than 1,100 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.

Under the Section XII.E of the PDUFA V technical agreement, FDA committed to a public process to develop standardized clinical data terminology through open standards development organizations, such as the Clinical Data Interchange Standards Consortium (CDISC), and stakeholder input with the goal of completing clinical data terminology and detailed implementation guides by December 2017. To facilitate the standards development process, the agreement specifically calls for FDA to develop a project plan for distinct therapeutic indications, prioritizing clinical terminology, for stakeholder review and comment by June 30, 2013. BIO believes the Roadmap is a welcome first step in the project plan process and offers the following comments in the spirit of collaboration toward the shared goal of improving the efficiency of the human drug regulatory review process.

**Comments:**

We encourage FDA to continue work with external stakeholders on a concrete plan to ensure development of high quality standards. We note that the number of indications covered has increased slightly since the original Roadmap was developed, but the

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timeline for completion remains unchanged; and accordingly, we are concerned that there is a risk that the quality of the standards developed may be severely impacted. We question whether it is feasible to create a standard in less than eighteen months given the magnitude of the project and tremendous amount of FDA resources required for successful and meaningful project completion. We stress the importance of developing high quality standards (*e.g.*, tested, effective, and publishable) that facilitate data analysis and timely review over lower quality standards that require reevaluation and changes, but are completed within the Roadmap specified time frame.

Toward this end, BIO believes that collaboration can be better achieved with increased transparency and clarity around (1) the criteria for the development and qualification process; (2) the standards development process; (3) stakeholder involvement and engagement; and (4) FDA collaboration with third party standard setting organizations.

First, BIO believes that greater transparency behind the Agency's criteria and weighting for selecting a particular clinical data standard and the timeline for its development will help facilitate planning and implementation by both the Agency and industry. Because, the Agency states that periodically, the timeline may be updated,[1] clear and established criteria will signal FDA commitment to the Roadmap, help ensure that future Roadmap revisions are more readily understood by all stakeholders, and allow industry to justify corresponding investments and undertake long-term planning decisions.

Second, success of the project will depend on a clear understanding of what exactly FDA expects to be defined by the data standards. Currently it is unclear whether the output is expected to be more (*e.g.*, basic results reporting, analysis specifications) than what CDISC normally provides. Increased clarity and transparency around the development standards framework (*i.e.*, whether the Agency intends to use the CDISC framework and model) will help set expectations and provide data collection efficiencies by enabling industry to develop specific electronic case report forms (eCRFs) and data standards that match those expectations.

Third, while the Agency states it will publish notices soliciting input on, and engagement in, standards development, and will periodically issue guidances specifying completed data standards, the Agency does not provide a mechanism for true, real-time, stakeholder involvement and engagement. Such a mechanism would not only inform stakeholders of the development and qualification processes but also provide an understanding of how to propose new standards.

Last, BIO requests the Agency also elaborate on its collaboration process with third party standard setting organizations. The Agency specifically cites its collaboration with CDISC and the Critical Path Institute to support development of therapeutic data standards and the Agency's collaboration with Health Level Seven's (HL7) Clinical Interoperability

[1] http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm287408.htm

Council and other consortia to define related clinical concepts and encourages stakeholders to engage in and, where possible support such collaborative efforts,[2] but does not provide a clear framework for the actual standards development process and the role intended for the third party organizations, as opposed to other stakeholders, including industry. Providing a clear understanding of the collaborative framework will support and sustain stakeholder collaboration in an efficient and meaningful manner.

**CONCLUSION:**

BIO appreciates this opportunity to comment on the "Development of Prioritized Therapeutic Data Standards." We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/

Ruth DeLuca
Manager, Science and Regulatory Affairs
Biotechnology Industry Organization (BIO)

[2] Id.