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September 26, 2011

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

Re: Docket No. FDA-2011-D-0239: Identifying the Center for Drug Evaluation and Research's Science and Research Needs

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to submit comments on the draft report, "Identifying the Center for Drug Evaluation and Research's Science and Research (CDER) Needs." BIO supports CDER's efforts to identify and improve the Center's scientific needs to better fulfill the Agency's regulatory mission. BIO has long supported the goals of the Critical Path Initiative and other regulatory science initiatives, such as the recently released "Advancing Regulatory Science and FDA" strategic plan.[1] Ongoing efforts to prioritize regulatory science research objectives coupled with clear and achievable milestones will help to encourage productive partnerships between FDA, the private sector, and academia to successfully modernize drug development and evaluation methodologies, and ultimately provide patients with access to safe and effective new therapies.

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,

[1] Please see BIO's report "Unleashing the Promise of Biotechnology: Advancing American Innovation to Cure Disease and Save Lives", specifically the Advancing Regulatory Science proposals at http://www.bio.org/sites/default/files/PromiseofBiotech.pdf.

thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment.

I. Prioritization of Categories and Initiatives

While BIO appreciates the breadth of initiatives included in the draft report, we suggest establishing a prioritization among these initiatives. BIO feels that, with limited resources, it would be helpful to decide where the biggest impact can be made within these options, and focus efforts on furthering those goals.

Toward that end, BIO has reached out to members of its Critical Path Work Group to ask for BIO members' perspectives on which initiatives are most promising from the perspective of the biotechnology industry. BIO looked at the categories as a whole, as well as the initiatives within each category. Among the broad categories listed in the draft report, BIO found the following to have the greatest potential for positive impacts:

- Category I: Improve access to postmarket data sources and explore feasibility of their use in different types of analyses;
- Category II: improving risk assessment and management strategies;
- Category III: evaluating the effectiveness and impact of different types of regulatory communications to the public and other stakeholders; and
- Category VI: improving clinical trial design, analysis, and conduct.

Among the more specific initiatives listed in the draft report, BIO found the following to be of particular importance to our members:

- accuracy and availability of postmarket data;
- evaluating and improving the impact of regulatory actions on patient outcomes;
- labels and similar modes of communication;
- improving selection and definition of study endpoints for various conditions;
- improving nonclinical science testing paradigms to predict human risk; and
- identifying and qualifying biomarkers for regulatory use.

BIO would also like to recommend a number of initiatives/topic areas not specifically mentioned in the report:

- better methodologies and more common approaches of benefit to risk assessment across multiple stakeholders would help to improve CDER's ability to fulfill its regulatory mission (*e.g.*, how should benefit be weighted for a population and/or individual against the risk to a population and/or individual? How can transparent, systematic, and qualitative/semi-quantifiable analyses of benefit to risk promote the public health?);
- frequent and effective communication with sponsors would likely lead to improvements in regulatory science; and
- more disease expertise within FDA in genetic diseases would be productive, particularly in orphan diseases.

BIO is pleased that FDA has committed to advancing many of these initiatives under the proposed PDUFA V technical agreement[2], and these programs should be appropriately highlighted and cross-referenced in CDER's regulatory science strategic priorities.

II. Establishment of Milestones

While the report lays out areas of need and specifies some actions and projects that can be undertaken to further CDER's use of regulatory science, there are no milestones, timeframes, or other mechanisms mentioned in the report to track progress. BIO suggests including metrics for tracking progress made on these important initiatives.

III. Private Sector Engagement in Regulatory Science Partnerships

BIO recognizes that improving regulatory science is a shared responsibility, and hopes that the Agency will view industry as a constructive partner and resource as FDA works to identify and address CDER's science and research needs. In the draft report, there is little discussion of how the private sector can best collaborate with FDA to leverage scientific expertise and infrastructure to advance regulatory science needs. Biotechnology companies often find it challenging to determine how and when to engage FDA or other public entities on regulatory science opportunities. BIO suggests, when feasible, adopting a regular, systematic approach to seeking input from the private sector.

IV. Regulatory Acceptance of New Methodologies and Other Outputs

While working toward improving the science and research tools available to CDER, it is critical that whatever outputs come from this report are fully validated and embraced by CDER reviewers. Without these two key steps, any improvements will not have a true impact on regulatory decisions.

CONCLUSION:

BIO appreciates this opportunity to comment on the draft report, "Identifying the Center for Drug Evaluation and Research's Science and Research Needs." 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)

[2] FDA, Proposed PDUFA Reauthorization Performance Goals and Procedures: Fiscal Years 2013-2017, http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM270412.pdf