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November 1, 2010

Dockets Management Branch (HFA-305)
Food and Drug Administration
5600 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: Docket No. Docket No. FDA-2010-N-0506, Request for Comments on the Food and Drug Administration Fiscal Year 2011-2015 Strategic Priorities Document

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to submit comments on the Agency's "Fiscal Year 2011-2015 Strategic Priorities Document." BIO supports FDA's initiative to establish a long-term vision for the Agency based upon guiding principles and we stand ready to work with the Agency to promote biomedical innovation, modernize regulatory science, further secure the global supply chain, and meet the needs of special populations.

BIO represents more than 1,200 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. Accordingly, BIO's diverse membership falls under the jurisdiction of nearly all of FDA regulatory programs, including drugs, biologics, devices, and foods.

I. Promoting the Public Health through Biomedical Innovation:

We are pleased to see the importance that Agency leadership has placed on reinforcing FDA's role as a preeminent public health Agency grounded in sound, science-based decision making. The biotechnology industry shares this vision, and consistent with

FDA's mission of promoting the public health, we view biomedical innovation and the development of novel therapies to target unmet medical needs as the most promising means to advance that goal. Indeed, the Strategic Plan underscores the importance of innovation and promoting the public health by highlighting FDA's mission of "Helping to speed innovations that make medicines and foods safer and more effective" (p.2). Support for the development of new, life-saving therapies is best facilitated through a regulatory environment that is science-based, collaborative, transparent, and accountable, as well as predictable and consistent. We encourage FDA leadership to continue to institutionalize these guiding principles of the Strategic Plan throughout all levels of the Agency.

BIO is committed to working with the Agency to support the development of the next generation of medical therapies and to ensure continued patient access to high-quality, effective, and safe biomedical products. It is important to recognize, however, that by definition prescription drugs carry both benefits and risks that must be carefully and systematically evaluated in the full context of the product's benefit/risk profile, and in the context of the disease severity. These inherent risks must be appropriately mitigated and communicated to patients, their caregivers, and medical providers, which necessitates early and regular coordination between FDA and the Sponsor during drug development, review, and marketing, particularly if a Risk Evaluation and Mitigation Strategy (REMS) is potentially involved.

To promote the advancement of new cures, we must also reiterate the importance of FDA meeting and communicating with companies early in the development/approval process through both formal meetings and ongoing dialogue. This is particularly important for our smaller, emerging companies.

Finally, as Commissioner Hamburg aptly states in the introduction, "It's no secret that FDA's responsibilities have increased significantly over the past several years," due in part to new legislative mandates, advancements in science, and the globalization of markets (p.iii). A strong FDA is essential to our success and as a founding member of the *Alliance for a Stronger FDA*, we will continue to fight for increased Agency funding through the appropriations process.

II. The Biotechnology Industry Can Serve as a Resource to Advance the FDA Mission:

BIO recognizes that improving the efficiency and effectiveness of the drug and biologics evaluation process is a shared responsibility. We hope that you will view BIO and its member biotechnology companies as constructive partners and a resources as you work to modernize the Agency and enhance its effectiveness.

a. Collaboration to Advance Regulatory Science:

BIO has been very supportive of the Agency's efforts to modernize regulatory science and we are pleased to see the Agency's ongoing commitment to the initiative. FDA's scientific knowledge and expertise is essential for evaluating the safety and efficacy of

medical products and biotechnology-derived animal products. More work must be done to advance the development and regulatory acceptance of tools – biomarkers, new clinical trial designs – that make drug and diagnostic development better and more efficient without sacrificing safety. FDA must anticipate regulatory challenges that may be posed by novel therapies, diagnostics, medical devices, and foods, and work collaboratively to design and implement strategies to ensure efficient review.

The Strategic Plan in part defines Regulatory Science as:

> *Before FDA can make a decision to approve a new product, we must determine what it means for that product to be safe and effective. We must develop the* ***appropriate standards and guidance*** *for making approval decisions.* ***The science, the tools, and the standards*** *we need to assess and evaluate the efficacy, quality, and performance of a food or medical product form what we call regulatory science. (P.5, emphasis added).*

Because this opening statement fundamentally defines "regulatory science" for the purposes of the Strategic Plan, we believe it should specifically note that the standards, guidance, and tools FDA intends to develop will be "science-based." In addition, we believe the statement should stipulate that such tools and standards will be developed in collaboration with the best researchers and in partnership with other Agencies to foster global consistency among regulators. We note that subsequent text in this part refers to recruiting outstanding scientists as well as to FDA collaboration with other agencies, but making such outreach an essential feature of "regulatory science" would considerably strengthen the definition.

Indeed, the Strategic Plan indicates that "To make rapid and efficient improvements in public health and drive innovation, we must harness the best ideas from a broad range of stakeholders and leverage resources through collaboration with other federal, state, and local regulatory and public health agencies; non-government organizations; consumer and patient organizations; academic medical centers and research universities; the private sector; and the public." (p.3)

BIO wholeheartedly agrees that strengthening FDA's collaboration with external scientists in academia, the private sector, and other organizations is a key component of advancing regulatory science. BIO has long supported the Critical Path Initiative through public-private partnerships, such as the Biomarkers Consortium and the Clinical Trials Transformation Initiative (CTTI). BIO members stand ready to work with FDA to help develop novel, 21st Century approaches to more effectively and efficiently evaluate the safety, effectiveness, and quality of medical products.

However, in both the Strategic Plan and in FDA's recently published "Advancing Regulatory Science for Public Health: A Framework for FDA's Regulatory Science Initiative",[1] there is little discussion of how the private sector can best collaborate with

[1] U.S. Food and Drug Administration, Office of the Chief Scientist, "Advancing Regulatory Science for Public Health: A Framework for FDA's Regulatory Science Initiative", October 2010, http://www.fda.gov/downloads/ScienceResearch/SpecialTopics/RegulatoryScience/UCM228444.pdf

FDA to leverage scientific expertise and infrastructure to advance regulatory science projects to fruition. Consequently, biotechnology companies often find it challenging to determine how and when to engage FDA on regulatory science opportunities.

We encourage FDA to elaborate upon the Collaborative Implementation Framework to identify a formal mechanism for external stakeholders, including biotechnology companies, to suggest regulatory science opportunities or to collaborate with existing regulatory science initiatives. BIO would be interested in participating in discussions with the Office of the Chief Scientist to help design this framework. We understand that the FDA Science Board has a responsibility for general strategic oversight of external collaborations; however at the present time we do not believe the current Science Board membership has the necessary expertise to identify and help shape the course of the many complex industry partnerships that are vital to the success of the Agency's regulatory science initiative. As a first step in considering how the collaborative framework could be constituted, we suggest that the Office of the Chief Scientist convene an ad hoc subcommittee of the Science Board, which would include representatives from the various industry trade associations, to develop recommendations for the design of the network with respect to industry partnerships.

We also suggest that FDA articulate a research agenda within each of the broad topics announced in the regulatory science plan. This should be done with stakeholders through public meetings and public comment through the Federal Register. Most importantly, each research item should be linked back to a regulatory decision that it will help inform. Additionally, we suggest the development of a publicly available inventory of all such regulatory science activities that FDA is currently engaged across all its centers through private-public partnerships, CRADAs, and memorandums of understanding, which would enhance transparency and coordination.

b. Further Strengthening the Global Supply Chain:

BIO fully recognizes the challenges posed by the need to regulate products being developed, manufactured, and distributed across the world in an increasingly globalized economy. The U.S. drug supply chain is the strongest in the world, but recent episodes of contamination of FDA-regulated products have underscored the vulnerability of the current system to economically motivated adulteration and criminal counterfeiting. BIO is committed to collaborating closely with FDA to better assure the safety, purity, and potency of both imported drugs and biologics and ensure that Agency funding keeps pace with the FDA's new international responsibilities.

As part of FDA's efforts to combat counterfeit drugs and secure the domestic pharmaceutical supply chain, we encourage the Agency to support the adoption of a uniform national standard for product pedigrees or track-and-trace to authenticate drug products in the domestic market to deter and combat counterfeiting and intentional adulteration.

c. Addressing the Needs of Special Populations:

Finally, BIO agrees with FDA's efforts to meet the needs of special populations, such as pediatric populations or patients suffering from rare diseases.

BIO members recognize the importance of minimizing barriers to, maintaining incentives for, and communicating the value of robust drug and biologic research in pediatric populations. During the last 15 years since enactment, the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) working together have been widely acknowledged as successful for promoting pediatric drug research. Making these laws permanent and eliminating any ambiguity about their continuation is critical to the continued success of pediatric development programs and the infrastructure that has been built to support them. Establishing these laws permanently would also allow FDA to put in place clear guidance documents for industry.

Another key element of ongoing success in pediatric research involves international harmonization of pediatric research requirements. BIO suggests that the strategic plan include "global harmonization of pediatric requirements" in addition to their collaboration with the other agencies (p.9). BIO believes that in addition to a more harmonized approach to timing of pediatric submissions and discussions with the European Medicines Agency and FDA, there is the need for the harmonization of some scientific requirements, for example, pediatric age ranges and definitions and corresponding formulations, indication interpretations, and generally the number and types of studies needed to support pediatric indications. BIO's Pediatrics Committee is developing a White Paper on International Harmonization and we would like to discuss this in greater detail with FDA so that an appropriate venue could be determined for scientific discussion and collaboration on current barriers to global pediatric plans and how to facilitate policy changes that would foster additional success in pediatric drug development. Also, we suggest that the plan highlight the importance of government grants or training opportunities for pediatric investigators to encourage institutions to participate in clinical trials.

BIO companies also believe that FDA has made great strides to make sure that safe and effective orphan products reach patients as soon as possible. However, more can be done to make the regulatory pathway more predictable and to encourage collaboration to bring new products to treat rare diseases to market. [2] We urge FDA to publish additional guidance regarding orphan drug development that provides interpretation of current regulations and to review use of its standards for demonstrating efficacy of a rare disease product. Moreover, BIO believes FDA can improve communications processes for rare disease stakeholders and supports greater transparency at the Agency including more meeting opportunities, and greater consistency among FDA's review divisions. The challenges of developing rare disease products require new regulatory approaches.

[2] Biotechnology Industry Organization, "FDA, Rare Diseases, Orphan Drug Act: Food and Drug Administration Review and Regulation of Articles for the Treatment of Rare Diseases" May 31, 2010, http://bio.org/letters/20100531.pdf

CONCLUSION:

BIO appreciates this opportunity to comment on FDA's strategic priorities for fiscal years 2011-2015. 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)