Biotechnology Industry Organization

July 31, 2014

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

Re: Docket No. FDA–2014–N–0833: Office of the Commissioner; Request for Comments on the Food and Drug Administration Fiscal Year 2014–2018 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 "The Food and Drug Administration Fiscal Year 2014-2018 Strategic Priorities."

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.

BIO believes the Strategic Plan is thoughtful and well-crafted, and we appreciate the forward-looking transparency that the document offers to both the public and regulated industry. BIO is also pleased to see that FDA's priorities are aligned with many of those of the biotechnology industry. For example, FDA's goals and priorities reflect the Agency's intent to make the regulatory process more consistent, transparent, and predictable—goals that are especially important to BIO member companies, who rely on investment capital that is highly influenced by the predictability of regulatory outcomes.

FDA's plan also focuses on other priorities our members have identified as critically important, such as the application of 21st Century science and technology, including biomarkers, surrogate endpoints, and adaptive trial design, to help speed the development of medicines. FDA's discussion of "smart regulation," which both encourages and speeds innovation, seems to recognize that accelerating and facilitating patient access to new medicines is a fundamental element of promoting and protecting the public health.

Finally, we note that throughout this strategic priorities document, FDA emphasizes the importance of enhancing communication between the Agency and Sponsors to improve the efficiency of drug development—one of BIO's highest priorities over many years.

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There are several additional areas that we recommend the Agency further clarify to strengthen the final Strategic Plan.

A. Implementation Plans and Program Measurement

BIO appreciates the Strategic Plan's section on Implementation (lines 132-144) and we agree that successful enactment of the plan will require alignment between the annual budget priority setting and program level activities. We are pleased to see that "At the program level, each FDA product center and major office will implement program-specific actions and monitor key metrics for progress toward achieving our stated strategic objectives and strategies." We recommend that each of these Center- and Office-specific implementation plans be appended to the final Strategic Plan, or posted on the FDA website when finalized. While it may be challenging to include these specific details in an overarching plan that encompasses diverse regulated products—from medical products to tobacco to foods—these program-specific implementation plans will provide greater public understanding of each Center or Office's strategic priorities over the next four years.

We also appreciate the Strategic Plan's discussion of how progress will be evaluated through metrics, such as user fee program performance goals and the FDA-Track system. As part of the Agency's ongoing strategic planning, we encourage the Agency to further refine and expand the capacity of FDA-Track. BIO's members fully support the underlying intent of FDA-Track to promote transparency and accountability, but our experience is that the data often lags months behind and the metrics tracked tend to be procedural, rather than reflecting meaningful real-world outcomes, thereby undermining the overall utility of the system.

B. International Harmonization and Coordination

BIO supports the Strategic Plan's emphasis on coordination and cooperation between mature international regulatory authorities and sharing of data and compliance information. We believe this will help to best leverage limited resources across regions, for example through mutual reliance of inspection and compliance information.

However, the plan does not adequately address efforts to harmonize the underlying regulatory requirements to minimize inconsistent or redundant regulatory burdens. Regulatory convergence is a win-win for regulators and industry that can increase efficiency and result in greater coordination between regulatory authorities, improved industry compliance, and enhanced regulatory oversight. Indeed, streamlined compliance costs in industry can help to redistribute resources towards biomedical research to the benefit patients and public health.

Significant progress has been made through the International Conference for Harmonisation (ICH) and additional international initiatives are under discussion in the context of trade agreements such as the TransAtlantic Trade and Investment Partnership (TTIP) and the TransPacific Trade Partnership (TTP). We encourage FDA to highlight these and other related efforts in the plan and continue to work towards convergence

and harmonization of regulatory requirements for the conduct of global clinical trials, mutual reliance of inspections and compliance requirements, and pediatric drug development strategies.

In addition, BIO recommends that FDA's definition of "smart regulation" also include the principle of international regulatory convergence as part of the goal to minimize unnecessary regulatory burden.

C. Cross-Center Collaboration

The future of drug development will increasingly focus on the commercialization of targeted therapies and drug-device combinations. For that reason, it is important that there is clarity in the requirements for the review and approval of companion diagnostics and combination therapies, and improved coordination and communication between the Center for Drug Evaluation and Research (CDER), the Center for Biologics Evaluation and Research (CBER) and the Center for Devices and Radiological Health (CDRH). In light of the varying structures of the prescription drug and medical device user fee programs, reviews that require consultations by multiple Centers can result in a lack of clear expectations for meeting relevant performance goals. We encourage FDA to elaborate in the Strategic Plan on how cross-Center reviews for these innovative products will be conducted.

D. Real-World Evidence and Electronic Health Records

As electronic health records (EHR) are further integrated into the healthcare delivery system, there is great interest in leveraging EHR data for evaluating the safety and efficacy of drugs and biologics. The revolution in "big data" and the use of real-world data can be potentially transformative for medical product development and evaluation of patient outcomes. However, the plan as currently drafted does not adequately address how the Agency plans to engage in health information technology initiatives and how these new national capabilities can be harnessed for drug development. We look forward to working with the FDA and other stakeholders in establishing a roadmap for integrating EHRs into the drug development enterprise to realize the vision of a learning healthcare system.

E. Communicating Benefits and Risks

BIO welcomes the plan's emphasis on providing accurate and easily understood information on the benefits and risks of medical products to the public to help promote their safe use. BIO has long supported FDA's Patient Information Initiative (PMI) and we look forward to seeing FDA enact a single patient-oriented document outlining benefits and risks in a patient-friendly fashion.

BIO also understands that FDA is awaiting Administration clearance of the proposed rule on *Electronic Distribution of Prescribing Information for Human Drugs Including Biological Products.* We suggest that the Strategic Plan further address FDA's plans for

seeking public feedback on the proposed rule and the Agency's longer-term plans for implementation. BIO fully agrees that the electronic distribution of the professional label would speed the availability of new safety information to physicians and minimize the wasteful and environmentally harmful distribution of paper labels.

F. Biosimilars Implementation

BIO strongly supported the enactment of the *Biologics Price Competition and Innovation Act of 2009,* which established an approval framework for biosimilar biological products while balancing incentives for ongoing biomedical innovation. Clear and transparent implementation of the biosimilar approval pathway will help all stakeholders—patients, consumers, providers, payers, and manufacturers—have a clear understanding of the regulatory expectations for these products. We request that the Agency further clarify the process and timing for finalizing the current biosimilars draft guidances and for issuing additional guidance on key topics, such as non-proprietary naming, interchangeability, and extrapolation.

G. Stewardship

BIO appreciates that the plan focuses on maximizing resources and efficiency through effective FDA management. To further support that goal, BIO recommends that the Agency consider further improving its quality management system. The FDA's stated fundamental guiding principles include: science-based decision making, innovation/collaboration, transparency, and accountability – and all are essential attributes of a world-class regulator. To help the Agency achieve these goals, the Agency could improve upon its management system by installing a broadly instituted management process which explicitly defines, measures, analyzes, improves, controls, and validates key processes utilized by its scientists as decisions are made. This system can be the backbone of a consistent and efficient FDA drug review process. Tying an improved quality system back to the FDA guiding principles outlined in the plan would provide the Agency with a key management tool that could help protect and advance the public health by improving the transparency and efficiency of drug review decisions.

Additionally, FDA's strongest asset is its dedicated scientists, medical reviewers, and technical professionals. As part of the Agency's ongoing efforts to recruit and retain a world-class workforce, we encourage FDA elaborate on its human resources strategy at the Center level. For example, the Center for Veterinary Medicine issued its *Strategic Human Capital Plan: FY2012-2016,* while the *CDER Strategic Plan 2013-2017* offers comparatively few details on human resources management, recruitment, and retention, which are key enablers in achieving FDA's public health mission.

Conclusion:

BIO appreciates this opportunity to comment on the "Food and Drug Administration Fiscal Year 2014–2018 Strategic Priorities Document". We would be pleased to provide further input or clarification of our comments, as needed.

Best Regards,

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Biotechnology Industry Organization (BIO)