BIO Supports Timely Reauthorization of PDUFA to Promote the Development of Innovative Therapies and Speed New Medicines to Patients

FDA Public Meeting on PDUFA V
October 24, 2011

On behalf of the Biotechnology Industry Organization, I thank you for the opportunity to comment on the reauthorization of the Prescription Drug User Fee Act (PDUFA). BIO supports the PDUFA V recommendations as they will enhance the drug development and review process through increased transparency and scientific dialogue, advance regulatory science, and strengthen post-market surveillance. Most importantly, PDUFA V will provide patients and doctors with earlier access to breakthrough 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, thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment.

I. Predictability in FDA Review Processes Supports Innovation

We as a nation need to focus policy discussions on how to unleash the promise of biotechnology so that the American public can realize the benefits it has to offer, and the PDUFA program is a key element of the overall innovation eco-system. A fundamental part of biotechnology companies' ability to innovate and raise private investment is having an FDA with the resources and mechanisms required to effectively and consistently review and approve innovative products in a timely manner based on the best available science. Since 1992 Congress, FDA, and the biopharmaceutical industry have supported a carefully structured user fee program to help fund FDA's human drug review activities, which has contributed to the approval of more than 1,200 new medicines and initially reduced review times for the newest, most innovative drugs by more than a year.

However, the human drug review program has been under considerable stresses in recent years as new regulatory requirements, such as risk Evaluation and Mitigation Strategies (REMS),

increased utilization of advisory committees, and more foreign inspections have been layered on the review process and the scientific complexity of applications has increased. As a result, overall approval times lengthened in the early years of PDUFA IV and patients were forced to wait longer for new therapies.[i]

Unpredictability in the review process, suboptimal communication with sponsors, and decreased FDA performance not only hinders patient access to new treatments, but also negatively impacts the ability of biotechnology companies to raise funding to support clinical development and ongoing innovation. This undermines economic growth in the biotechnology sector as well as biomedical research into key public health priorities.

II. PDUFA V: Getting Back-to-Basics for Patients

In PDUFA V, industry and FDA have agreed upon a set of enhancements that seek to restore FDA's review performance and get back-to-basics for patients. These proposals have also been informed by an unprecedented level of public input through workshops, meetings, and stakeholder outreach, which have further strengthened the technical agreement. Underlying the PDUFA V recommendations are the principles that a science-based, transparent, and well-managed review process that appropriately balances benefits and risks can enhance public trust and increase patient access to new medicines.

- New Molecular Entity (NME) Review Program: Historically, nearly 80% of all applications are ultimately approved, but less than half of the products submitted to the FDA are approved on the first submission.[ii] We can and must do better for patients. By strengthening scientific dialogue and transparency between FDA and the Sponsor under the proposed review program for novel drugs or biologics, we can minimize the potential review issues that can delay patient access to needed treatments. Increased FDA-Sponsor scientific dialogue and transparency, such as a mid-cycle communication, exchange of discipline review letters and advisory committee information, and a significant new late-cycle meeting, will help to identify and resolve issues earlier in the review. This represents a significant paradigm-shift in FDA's review process. Coupled with an additional two month validation period during the review period and a robust third-party evaluation, we expect that this program will lead to fewer review cycles leading to shorter overall approval times and earlier patient access to needed treatment.

  Enhanced Communication during Drug Development: To help advance American innovation and promote the development of the next generation of modern medicines, FDA has also committed to a philosophy under PDUFA V that timely, interactive communication with biotechnology and life science companies during drug development is a core Agency activity.

  FDA's recent report on driving biomedical innovation highlights that "the private sector is the engine of innovation, and much of this innovation begins with small business."[iii] Indeed, many small biotechnology companies operate on the cutting edge of biomedical science to develop new therapies for devastating diseases. Yet we must acknowledge that

the scientific method does not operate in a vacuum, and it is critical to promote interactive, scientist-to-scientist communication between FDA and Sponsors. In the course of drug development, Sponsors sometimes have simple or clarifying questions, the responses to which could have a significant impact on the development program, but which are not extensive enough to warrant formal meetings. To obtain timely responses to such questions, Sponsors currently often have to engage in a lengthy exchange of multiple formal letters with FDA, which is an inefficient use of both FDA's and the Sponsor's time. For small biotechnology companies reliant on limited venture capital, these delays can create significant impediments to development programs.

Additionally, independent reports commissioned by FDA have also demonstrated that enhanced communication during drug development ultimately results in higher quality applications, which can enhance efficiency for FDA reviewers.[iv]

BIO fully supports the PDUFA V proposal to promote innovation through enhanced communication between FDA and sponsors during drug development, which will establish best practices for this type of interactive dialogue, train staff on communication practices, and provide the Agency with additional staff capacity to respond to sponsor inquiries in a timely manner.

- Modernizing Regulatory Science: Additionally, the agreement makes new resources available to modernize regulatory science, for example, in the areas of personalized medicine and rare disease drug research. Modern approaches to drug development and evaluation, such as through the application of new tools for rare disease drug development, flexibility with regard to creative study designs and new endpoints, greater utilization of biomarkers and patient reported outcome tools will introduce new efficiencies in the drug development enterprise and provide FDA with additional tools to evaluate the benefits and risks of pharmaceutical products. These proposals will also integrate more structured and systematic approaches to assessing benefits and risks of therapies, and allow FDA to conduct outreach to patients and hold workshops to better understand patient perspectives on disease severity and unmet medical need.

- Robust Drug Safety and Post-Market Surveillance Capacity: PDUFA V continues industry's commitment to a lifecycle approach to product evaluation by strengthening FDA's post-market surveillance and benefit/risk management capacity. Earlier discussion of risk management strategies, standardized approaches to REMS, and further validation of the Sentinel Network will promote patient confidence in drug and biologics.

III. User Fees should Complement a Sound Base of Appropriations:

Under the agreement, industry has reinforced its commitment to a well-funded drug and biologics program that supports sound, science-based regulation consistent with FDA's public health mission. However, user fees are intended to support limited FDA activities around the drug review process and were never intended to supplant a sound base of appropriations. User fees currently account for nearly two-thirds of the cost of human drug review. We urge

Congress to support FDA's mission and fund the Agency at the Administration's FY12 requested levels.

IV. Timely Reauthorization of PDUFA

Finally, it is critical for PDUFA to be reauthorized well in advance of PDUFA IV's expiration in September 2012 in order to avoid a reduction in force at the FDA. Even the threat of a downsizing at the FDA would be devastating to the Agency's public health mission and its ability to review new drugs and biologics.

BIO looks forward to working with Congress and FDA to fully implement these enhancements under PDUFA V.

V. Sponsors and FDA should Begin Planning for PDUFA V in Advance of Reauthorization

Finally, successful implementation of PDUFA V from a practical perspective will require both FDA and individual companies to make changes to their existing regulatory procedures and communication practices. We recognize that preparing for these changes is a shared responsibility. For example, under the NME review program both FDA medical reviewers and industry regulatory affairs professionals will need to be aware of the revised review schedule, be prepared for the key points of interaction during the review such as the mid-cycle communication and late-cycle meeting, and understand their respective expectations and roles. The NME review program will become effective on day one of PDUFA V on October 1, 2012, so to facilitate effective implementation, we encourage both biopharmaceutical Sponsors and FDA to begin internal planning and staff training on new review processes and communication practices well in advance of final passage of the legislation.

VI. Conclusion

Thank you for the opportunity to speak in support of the PDUFA V recommendations and I would be pleased to answer any questions.

Sincerely,

/S/

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[ii] FY10 PDUFA Performance Report, p.4, http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/UserFeeReports/PerformanceReports/PDUFA/UCM243358.pdf

[iii] FDA, *Driving Biomedical Innovation: Initiatives for Improving Products for Patients*, October 2011, http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/UCM274464.pdf

[iv] Booz Allen Hamilton, *Independent Evaluation of FDA's First Cycle Review Performance –- Final Report* July 2008, http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm127117.htm