Association for Accessible Medicines

Your Generics & Biosimilars Industry

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# FDA USER FEE AGREEMENTS: IMPROVING MEDICAL PRODUCT REGULATION AND INNOVATION FOR PATIENTS, PART II

**COMMITTEE ON HEALTH, EDUCATION, LABOR AND PENSIONS**

Good morning Chairman Alexander, Ranking Member Murray, and Members of the Committee on Health, Education, Labor and Pensions. First, let me thank you for asking me to participate in this timely and important hearing.

I am David Gaugh, Senior Vice President for Sciences and Regulatory Affairs at the Association for Accessible Medicines (AAM), formerly GPhA, and the Biosimilars Council (Council) and a licensed pharmacist. AAM represents the manufacturers and distributors of finished generic pharmaceuticals, bulk pharmaceutical chemicals, and the suppliers of other goods and services to the generic industry. Generics represent greater than 89% of all prescriptions dispensed in the U.S., but only 27% of expenditures on prescription drugs.

The Biosimilars Council, a Division of AAM, works to ensure a positive regulatory and policy environment for biosimilar products, and educates the public and patients about the safety and effectiveness of biosimilars. We are deeply committed to accessible, affordable and high quality medicines.

***Introduction***

I would like to begin today by commending the Committee for your continued focus on the important issues we will examine today. As someone who has worked in and around the generic drug industry for more than two decades, I have witnessed firsthand the industry's remarkable growth and the vital role it plays in the lives of Americans every day, by providing access to affordable generic medicines.

As for the biosimilars industry, I have been engaged in this industry for over a decade and have seen American ingenuity take this science to new levels. Today we have a growing and thriving biosimilars industry – creating jobs and leading the world with our innovative science.

This growth in both the generic and biosimilar industries has served to underscore the critically important role of the Food and Drug Administration (FDA). As I will highlight, the level of cooperation between industry and the FDA has never been greater, and it is our hope that this collaboration will continue throughout all of our interactions with the agency.

However, the agency remains underfunded, and the responsibility of ensuring access to safe, effective and affordable medicines is a shared one with the entire pharmaceutical industry. That is why the generic and biosimilar industries have once again committed to provide the FDA with additional user fee resources to address the ongoing challenges caused by an increasingly global drug supply-chain.

***Generic User Fee Amendment***

I am here to discuss AAM's conviction that the best way of achieving the goal of providing patients access to generic alternatives is through the development of policies that promote robust, competitive markets.

Generic manufacturers make complex and highly confidential analysis when selecting which products to pursue. This analysis can include assessing the complexity in reverse engineering, the state of the intellectual property, the size of the market, the likely number of competitors, the product development and manufacturing capabilities and costs.

Because of these complexities, AAM believes that the best way to control drug costs generally, is through policies that incentivize competition and the Generic Drug User Fee Amendment (GDUFA II) does just that.

GDUFA II builds on the experiences – both the successes and shortcomings – of GDUFA I. The priority of the generic drug industry in the GDUFA II negotiations was to achieve a more effective and transparent generic drug review program. We believe that accomplishing this goal will improve the rate of first-cycle approvals on the earliest legally eligible date through greater transparency and communication during the review process. Greater communication and cooperation between FDA and generic drug sponsors benefits both parties by sharing knowledge and experiences throughout the review process. Our industry's goal was not merely a faster FDA review timeline, but a more effective review process – that enables more approvals during the first-review cycle. Similar to the goals of the branded drug user fee program, PDUFA, reducing multiple FDA review cycles is a critical component of increasing access to affordable generic alternatives. The fewer review cycles required to get to approval, the sooner

patients and payors can experience the benefits of generic drug competition. We strongly believe GDUFA II is well positioned to achieve this goal.

**A few key areas of focus in GDUFA II include:**

Application Metrics – FDA will review and act on 90 percent of ANDAs within 10 months after the date of submission for standard applications and 8 months for priority applications. This includes the inspection components of the review process. Priority status will be provided by FDA for submissions affirmatively identified as eligible for expedited review pursuant to current CDER Prioritization Policies (MAPP 5240.3 Rev. 2).[1]

- Submissions containing patent certifications pursuant to 21 CFR 314.94(a)(12);
- Submissions related to drug shortages;
- Submissions that are subject to special review programs such as the President's Emergency Plan for AIDS relief;
- Submissions related to public health emergencies;
- Submissions related to certain government purchasing programs;
- Submissions subject to statutory mandates or other legal requirements;
- Supplements for which expedited review is requested under 21 CFR 314.70(b)(4); and
- Submission for "sole-source" drug products.

[1] Center For Drug Evaluation And Research, MaPP 5240.3 Rev. 2 , https://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ManualofPoliciesProcedures/UCM407849.pdf

Bridging (No ANDA Left Behind) – In GDUFA I, ANDA applications that were filed with the FDA prior to October 1, 2014, did not receive an official GDUFA I Goal Date. However, during early implementation phases of GDUFA I, the FDA agreed to assign Target Actions Dates (TADs) to those applications. These TADs would allow both the FDA and industry to better track the application status. During GDUFA II negotiations, it was agreed that ALL GDUFA I pending applications would be provided an official GDUFA II Goal Date. Therefore, prior to the completion of GDUFA I, all applications and supplements that have been assigned TADs by FDA will be converted to official GDUFA II Goal Dates. For all applications and supplements that were either (a) previously not assigned a TAD or (b) were previously assigned a TAD and the TAD was missed, at the time of GDUFA II commencement, these pending applications will be assigned a goal date by the FDA that shall not be later than July 31, 2018. This will provide for an official accountability for all pending application.

Complex Products – The GDUFA II agreement creates a pre-ANDA submission communication pathway for a subset of generic drug applications, complex products. Like the Breakthrough Therapies program initiated for certain high priority branded drug application, earlier interaction between the applicant and FDA is expected to enhance industry's ability to understand and anticipate FDA's expectations during the critical research and development phase for complex products. With this new pathway, industry and FDA will be able to engage in Product Development, Pre-Submission, and Mid-Review Cycle meetings for complex products. As captured in the commitment letter, industry will need to meet a high bar in order for FDA to grant a meeting request. The

high bar was deliberately set to allow FDA to staff up in the earlier years, which is reflected in the metrics in GDUFA II. It is industry's belief that this early engagement between industry and FDA will significantly contribute to the applicant's ability to improve the overall submission quality of ANDA's, which in turn will contribute to first-cycle approvals.

ANDA Review Transparency and Communications Enhancements – The agreement includes increased transparency and communication elements between FDA and ANDA applicants throughout the review process through liberal use of Information Requests (IRs) and Division Review Letters (DRLs). These enhancements are intended to decrease the number of review cycles from the 3-4 review cycles experienced today, and move them more towards first-cycle approvals. FDA should consider how it can further enhance communication with generic drug sponsors to improve on its 9% first-cycle approval rate.

Reporting and Accountability – FDA will conduct increased financial and performance reporting to maximize transparency to Congress, industry and the public. The GDUFA II agreement includes several new performance and financial reporting requirements to ensure transparency and efficiencies are maintained. The new reporting requirements will allow Congress, generic drug sponsors and FDA to better assess FDA's resource management planning and processes to ensure the overall success of the GDUFA program. The quarterly and annual reporting requirements will also provide insight into

the financial and performance efficiencies of the FDA, allowing for future program improvements and enhancements.

Small Business Consideration – The GDUFA II agreement supports small business by exempting them from a facility fee until the first ANDA in that facility is approved. The proposal also provides for tiering of the annual ANDA program fees based on small, medium and large companies. This tiering is based on the total number of approved ANDAs for each company.

***Biosimilar User Fee Act***

Biologic medicines are often the only lifesaving treatments for many of the most severe diseases encountered by patients today. In many respects, they represent the future of medicine. Their high price tag, however, can keep them out of reach for many patients.

In October, the FDA reported that over 66 biosimilar programs were under review for development of 20 different biologic products. This was made possible by the BPCIA, and by BsUFA I user fee funding. We learned in BsUFA I, however, that the innovation involved in biosimilar development – the science of understanding what is in a biologic for comparison purposes – is complicated and involves many new skills that the industry and the FDA need to understand. This requires new staff and training to assure high quality and efficient review. Historic FDA staffing cannot meet these needs which depend far less on clinical data, and far more on new innovative scientific techniques

that demonstrate that a biosimilar is highly similar to the reference product and has no clinically meaningful differences.

In addition, even more innovation is underway to allow for approval of interchangeable biologics which can be shown to perform the same in any given patient and, when approved, substituted at the pharmacy like generic drugs. This innovation is what makes biosimilars competitive, affordable, safe and effective for patients.
But, these innovations squarely depend on having the critical additional FDA resources funded by BsUFA II.

Innovation was used to craft the BsUFA II Commitment Letter. We took a hard look at the first five years. Not only are new FDA resources needed, more efficient regulatory approaches that use funding more wisely are necessary to accelerate FDA review. Together we included innovations from BsUFA I and PDUFA to enhance the review process and to ensure regulatory clarity. The BsUFA II user fees are now tied to the level of resources needed and adjust with resource demand.

Biosimilars provide a cost-saving alternative for patients. BsUFA II will support the foundations set in BsUFA I and provide FDA with adequate resources to apply consistent regulatory standards to all biosimilars, review new applications as they are filed, and develop important public policy positions. FDA, industry and patients will all benefit from the user fee program by gaining a higher degree of certainty in the timeliness of application reviews.

**BSUFA II includes several important enhancements:**

Review timelines – Industry agreed to shift review timelines from the current 10-month timeframe to 12 months in order to improve and increase opportunities for communications touchpoints between industry and FDA, striving for first-cycle review when possible.

Additional Funding – Funding will focus on hiring additional staff for guidance development, reviewer training, and timely communication. BsUFA II will not be linked to PDUFA fee levels in order to create independent and predictable funding levels based on program needs and resource requirements.

Draft Guidances – FDA also agreed to publish draft and final guidance documents on several critical, outstanding policy positions.

Communications – Enhanced communication and meeting opportunities that eliminate unnecessary delays in development and review. The meeting deadlines were adjusted based on BsUFA I experience to allow for the most effective use of the meetings to accelerate program development. Initial Advisory meetings were accelerated, and Type 2 meetings were extended to allow the Agency to have the time to provide complete answers and better guidance. At the same time an option for written advice was added which could accelerate in many situations the time to receipt of Type 2 meeting advice.

Resource Capacity Planning – Using resource capacity planning to set budgets, staffing levels and fees. The use of capacity resource measurement and planning will help ensure that the level of funding is actually tied to the resources needed and will allow for adjustment of fees up and down as the number of programs fluctuate. This should make the review more efficient, avoid the opportunity cost of delays, and allow for adjustment of fee allocation to the kinds or resources actually needed by the Agency. For example, as the number of marketed products increase, the fees will increase and fees may be reduced on the pre-application development side.

Program Review Models – Adopting the highly effective Program Review Model to increase first-cycle application approvals and training of review teams for greater effectiveness. The Program Review Model was tested in PDUFA and puts in place performance obligations, communication commitments, pre-filing meetings, mid-cycle communication and a late-cycle meeting. Experience shows that the enhanced communication conserves FDA resources and applicant resources and has enabled first-cycle approval more often than when it was not in place. This should accelerate approval of high quality applications.

Dedicated Staff – The agreement makes commitments to dedicate staffing and to issue regulatory guidance to promote best practices and predictability.

Education – The agreement expands biosimilar public education activities.

Each improvement accelerates high quality development and review to help assure that patients have more timely access to life-saving, affordable, safe, and effective biosimilars.

***Summary***

By designing both of these user fee programs to spread fees across multiple stakeholders and sources to keep individual amounts as low as possible, the programs will help assure that patients continue to receive the significant cost savings from generics and biosimilars. It is also important to emphasize that the funding provided by both of these user agreements is in addition to, not a substitute for, Congressional appropriations. Expenditure is contingent, as in the past, on a spending trigger tied to Congressional appropriations.

***Conclusion***

In conclusion, Mr. Chairman, the user fee proposals are the culmination of months of negotiations between FDA and industry, and the final product as transmitted to Congress represents a careful balance among all the stakeholders involved. We respectfully urge the Committee to approve GDUFA and BsUFA as negotiated by FDA and industry, without any changes to the underlying agreements. It is also vital that the agreements be approved in a timely manner so that patients, the FDA, and generic and biosimilar manufacturers can begin to see their many benefits. Nothing is more important to our industries than ensuring patients have access to the safe, effective and

affordable lifesaving medications they require, and these historic agreements provide a critical step toward accomplishing this goal.  Thank you.