



James C. Greenwood
President & CEO

April 5, 2017

The Honorable Lamar Alexander
Chairman, Committee on
Health, Education, Labor and Pensions
U.S. Senate
Washington, D.C. 20510

The Honorable Patty Murray
Ranking Member, Committee on
Health, Education, Labor and Pensions
U.S. Senate
Washington, D.C. 20510

The Honorable Greg Walden
Chairman, Committee on
Energy and Commerce
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member, Committee on
Energy and Commerce
U.S. House of Representatives
Washington, D.C. 20515

Dear Chairman Alexander, Ranking Member Murray, Chairman Walden, and Ranking Member Pallone:

Thank you for inquiring about the Biotechnology Innovation Organization's (BIO) views on the authorization of PDUFA VI, the fifth reauthorization of the Prescription Drug User Fee program.

BIO strongly supports the PDUFA VI user fee agreement and its timely authorization. This agreement was carefully crafted through negotiation between FDA and the biopharmaceutical industry, along with input from patient and consumer organizations, health care providers, and other stakeholders - a negotiation that took place according to statutory requirements, over an approximately 18-month period. The agreement Congress is considering reflects a scrupulous assessment of the costs associated with each of the FDA commitments and timelines. PDUFA expenditures and the achievement of the agreed-upon goals will be reported annually, as required by statute.

Like the Congressional deliberations and bipartisan work that achieved the 21st Century Cures Act, the process was lengthy, but the goal is worthy. The provisions of the 21st Century Cures Act will make a positive difference to patients waiting for treatments and cures, and the goals of the PDUFA VI agreement will enhance and complement these promises. Re-negotiating this agreement will delay enactment. Patients need the reforms of PDUFA VI, and they need them now.

This PDUFA agreement maintains the program's nearly 25-year focus on achieving long-standing goals of reviewing Priority applications in 8 months and Standard applications in 12 months. In addition, it enhances the incorporation of drug development into the general program objective of achieving efficiency and predictability. Today, thanks to user fees, the majority of new drugs are available to American patients before patients anywhere else in the world. But, for patients still waiting for treatments or cures, FDA's efficient review times are just the tip of the iceberg; compared with the much longer drug development times of 10 to 12 years prior to review. Significant goals of PDUFA VI are directed at making drug development more efficient by employing novel and newly understood drug development tools as well as new ways of approaching clinical studies and data collection.



PDUFA VI builds on earlier and current efforts in patient-focused drug development. The PDUFA VI commitments will result in incorporating the patient voice early in drug development, including in the design of clinical trials, as well as in the FDA regulatory decision. These activities will result in patient-focused valid evidence that can be included in the drug label and ultimately benefit all prescribers and patients.

Additionally, the PDUFA VI goals include expanding expertise in diverse statistical methods, piloting innovative clinical trial designs and computer modeling and simulation, using biomarkers as surrogate endpoints, and using real world evidence – in other words, bringing 21st century science to bear on drug development. These commitments will transform drug development, while maintaining the statutory gold standard of substantial evidence of safety and effectiveness.

Importantly, PDUFA VI will make improvements to ensure the long-term financial stability of this user fee program and reduce the rate of program growth, as well as making significant changes to improve FDA's ability to estimate PDUFA personnel needs and to hire scientific and medical experts in the numbers needed to carry out its PDUFA goals -- all without jeopardizing the other significant parts of FDA's public health mission. For the first time, PDUFA annual hiring goals are included in the agreement. This allows the public a line of sight into whether goals may fall by the wayside as a result of an inability to hire.

If PDUFA VI is not authorized before the expiration of PDUFA V – September 30, 2017, there will be a significant negative impact on FDA's staffing. User fee staff salaries and benefits are paid for by user fees – not federal tax dollars. Inability of FDA to collect user fees – a consequence of the program not being timely reauthorized – will mean these staff cannot remain employed by FDA. It is impossible to over-state the negative impact of such a situation on the availability of new drugs to patients who need them.

In conclusion and to reiterate, BIO strongly supports this PDUFA VI agreement. We urge Congress to authorize PDUFA VI in a timely way, to avoid the dire consequences of not doing so. We are eager to work with you to achieve this, and are happy to provide any information or assistance you may need.

With Sincerest Regards,

A handwritten signature in black ink that reads "Jim Greenwood". The signature is fluid and cursive, with the first name "Jim" being particularly prominent.

James C. Greenwood
President and CEO