



Summary of Final PDUFA III Legislation

The Prescription Drug User Fee Act was originally enacted in 1992 (PDUFA I), and was reauthorized in 1997 (PDUFA II) as part of the Food and Drug Administration Modernization Act (FDAMA). FDA, BIO and PhRMA worked together to develop a set of recommendations to Congress for a third reauthorization of PDUFA, which was slated to expire September 30, 2002. The recommendations are reflected in the “goals letter” from the Secretary of Health and Human Services and the final PDUFA III reauthorization legislation (included as Title V) in the House-Senate Conference Agreement on the “Bioterrorism” bill – H.R. 3448. The House passed the Conference Agreement on May 22 by a 425-to-1 vote, and the Senate passed it on May 23 by a vote of 98 to 0. The House-Senate Conference Report can be found at http://energycommerce.house.gov/107/pubs/H3448_CON.pdf. The President is expected to sign the legislation into law in the very near future.

New Revenue for FDA

PDUFA III will bring the agency back to sound financial footing by providing substantial increases in user fees and ensuring that FDA’s resources reflect its changing workload.

The PDUFA III legislation:

- Substantially increases revenue from user fees. To bring FDA back to sound financial footing and to support additional review process enhancements, industry has agreed to a near doubling of user fees – from \$133 million in fiscal year 2001 to \$259 million by FY 2006. As part of this increase, FDA would receive \$90 million in new user fees in the first year of the program. This new infusion of resources will allow FDA to hire approximately 458 new FTEs by the end of PDUFA III. The user-fee projections are listed below, in millions:

<u>FY2001</u>	<u>FY2002</u>	<u>FY2003</u>	<u>FY2004</u>	<u>FY2005</u>	<u>FY2006</u>	<u>FY2007</u>
\$133.2	\$177.0 (est)	\$222.9	\$231.0	\$252.0	\$259.3	\$259.3

- Continues the current one-third split among application, product and establishment fees.

- Nearly doubles application fees by fiscal year 2007 and provides a nearly 60 percent increase in the first year of PDUFA III.

	<u>FY2001</u>	<u>FY2003</u>	<u>FY2007</u>
NDA/BLA Application Fee	\$311,000	\$495,333	\$576,222

- Changes the “revenue model” to more accurately adjust to changes in workload. The new workload adjuster – which includes the number of New Drug Applications (NDAs)/Biologics License Applications (BLAs), commercial Investigational New Drug Applications (INDs), efficacy supplements, and manufacturing supplements – ensures against the shortfalls in anticipated revenues that occurred during PDUFA II because of overestimates of the number of NDAs/BLAs and underestimates of waivers/exemptions.
- Modifies the statute slightly to ease administrative burdens at FDA.
- Continues the exemptions from user fees for orphan drugs and small businesses submitting their first applications.

Enhancements to the FDA Review Process

The agreement retains the performance goals of PDUFA II, creates new mechanisms to improve review process enhancements, and establishes several important new goals to be included in the goals letter from HHS to Congress. The provisions will:

- Improve first-cycle review. Assures greater attention to the full review of applications during the time frames set forth in PDUFA II performance goals (10 months for standard applications; six months for priority applications) by (1) developing and implementing “good review management practices”; and (2) assuring early and ongoing communication between FDA and the sponsor.
- Support performance enhancements. Sets aside \$7 million in user-fee funds available through the Commissioner’s office for targeted initiatives to improve the drug review process. This program is intended to improve review efficiency and bring performance at the two review centers into alignment.
- Allow for use of independent consultants. In order to ensure better exposure to cutting-edge science, FDA will consider the appointment of an independent expert, upon the request of a sponsor, to assist in the design of pivotal Phase III clinical protocols. The option applies to products that represent significant advances in the treatment, diagnosis or prevention of a disease or condition, or that have the potential to address an unmet medical need. FDA retains full authority both to choose the independent expert and to serve as the final decision-maker.

- Establish a new risk management system. Establishes a substantial new risk management system to be supported by user fees. Sponsors will now have the option to develop and submit risk management plans as part of the NDA/BLA review. Products with risk management plans will undergo studies for an additional two to three years after approval. The new system will enable the addition of 106 new FTEs.
- Enhance information technology. Provides additional IT resources to maintain the IT infrastructure base, expand and improve electronic submission capability, secure electronic commerce, and support integrity management. FDA agrees to specific milestones; progress on those milestones will be reported to Congress annually and evaluated by an independent contractor.
- Create cumulative marketing application pilots. Initiates pilots to evaluate the cumulative electronic submission of components of an NDA/BLA during the IND phase and FDA formal review of the application.
- Add new efficacy supplement goals. Adds goals for resubmission action dates: two months for Type 1 resubmissions and six months for Type 2 resubmissions.

Other Items

The PDUFA renewal would also:

- Allow for greater consultation during PDUFA IV negotiations. Includes requirements that the Secretary publish in the *Federal Register* joint FDA/industry recommendations and hold a public meeting to discuss the recommendations.
- Amend requirements related to Phase IV commitments. Requires public disclosure of incomplete Phase IV studies and authorizes the Secretary to require sponsors of such studies to notify physicians of the failure to complete the studies and the questions of clinical benefit that remain unanswered.
- Increase spending from appropriations for activities not funded by PDUFA. Requires increased spending from appropriations for post-market surveillance activities and authorizes increased appropriations for prescription-drug advertising enforcement and review of generic drugs.

The Biotechnology Industry Organization (BIO) represents more than 1,000 biotechnology companies, academic institutions, state biotechnology centers and related organizations in all 50 U.S. states and 33 other nations. BIO members are involved in the research and development of health care, agricultural, industrial and environmental biotechnology products. For more information on BIO, visit our website at www.bio.org. For further information, contact Steve Lawton or Wendy Taylor at 202-962-9200.