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The new law ensures that FDA will continue to receive a source of stable and consistent funding ... to fulfill its mission to promote and protect public health

PDUFA V

Before enactment of the Prescription Drug User Fee Act (PDUFA) in 1992, the drug review process at FDA was neither timely nor very predictable. This contributed to a delay in the availability of new drug products in the Unites States when compared to other countries.

PDUFA authorized FDA to collect fees from companies that develop and submit certain human drug and biological products for FDA review. These funds are supplementary to FDA's congressional appropriations and are used by FDA to hire additional reviewers and support staff, as well as upgrade information technology systems to improve the efficiency of the FDA review process. In return, FDA commits to review and act on applications within a predictable time frame.

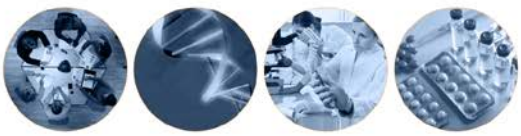
FDA's current goal is to review and act upon 90% of priority review applications within six months, and 90% of standard review applications within ten months. PDUFA's effect has been to dramatically reduce the FDA review time while still maintaining FDA's high standards for demonstrating drug safety, efficacy, and quality. This has allowed the agency to fulfill its mission to protect and promote public health by helping to bring to market important new medicines for patients.

PDUFA V Enhancements PDUFA has undergone authorization five times, most recently as part of the FDA Safety and Innovation Act (FDASIA) signed into law on July 9, 2012. PDUFA V will cover fiscal years 2013-2017 beginning on October 1, 2012. The new law ensures that FDA will continue to receive a source of stable and consistent funding throughout PDUFA V. There are many aspects of PDUFA V that may be of special interest to small business, including:

- Communication: To promote greater transparency and improve communication between the FDA review team and the applicant, FDA will establish a review model (hereafter referred to as "the Program") that will apply to all new molecular entity new drug applications (NME NDAs) and original biologics license applications (BLAs.) This will include applications that are resubmitted following a Refuse-to-File action, received from October 1, 2012, through September 30, 2017. The goal of the Program is to improve the efficiency and effectiveness of the first cycle review process and decrease the number of review cycles necessary for approval, ensuring that patients have timely access to safe, effective, and high quality new drugs and biologics.

- Review Performance Goals: For NME NDA and original BLA submissions that are filed by FDA under the Program described above, the PDUFA review clock will begin at the conclusion of the 60 calendar day filing review period that begins on the date of FDA receipt of the original submission.

- Communication Liaisons: PDUFA V also aims to establish a team of liaisons that will be available as an additional resource to enhance communication between FDA and sponsors during drug development. The liaison staff will serve as a primary point of contact for sponsors who have general questions about drug development or who need clarification on which review division to contact with their questions. The staff will also serve



CDER Small Business Assistance | Division of Drug Information | Office of Communications
10001 New Hampshire Avenue | Hillandale Bldg, 4th Floor | Silver Spring, MD 20993
(866) 405-5367 or (301) 796-6707
CDERSmallBusiness@fda.hhs.gov
www.fda.gov/smallbusinessdrugs

as a secondary point of communication within CDER for sponsors who are encountering problems in communication with the review team for their investigational new drug application (IND.) In addition, the liaison staff will identify and disseminate best practices for enhanced communication, and develop training programs for review staff. They will also collaborate with sponsor stakeholders to develop training for sponsors and receive feedback from sponsors on FDA's programs regarding best practices for communication during drug development.

- Electronic Submissions: Small business should also be aware that Section 1136 of FDASIA includes a requirement that submissions to FDA be in electronic format. This provision applies not only to NDAs and BLAs submitted under the PDUFA program, but also to generic drug and biosimilar applications. Receiving applications in a standardized electronic format is a key factor that allows for an efficient review of an application. To implement this requirement, FDA will issue draft guidance on the standards and format of electronic submission of applications. After consideration of public comments on the guidance, the agency will finalize the guidance. Following issuance of the final guidance on electronic submissions, applicants will have no less than two years to comply with this requirement. More information on the electronic submission requirement for PDUFA applications can be found in the PDUFA performance goals letter.

There are also other enhancements to PDUFA V, including enhancing benefit-risk assessment to integrate a more structured approach in FDA's regulatory decision-making; enhancing the FDA Drug Safety System by initiating a public process to standardize REMS, and more. You may find details in the PDUFA V Fact Sheet and PDUFA V Performance Goals Letter.

User Fees PDUFA authorized three types of user fees - application fees, establishment fees, and product fees. Application fees are a one-time fee, whereas the other two fees are annual fees. The current fee for filing a new NDA or BLA requiring clinical data is approximately $1.9 million. The FY 2013 fees are effective October 1, 2012.

Fee Category	Fee Rates for FY2013
Applications - Requiring clinical data - Not requiring clinical data	---------- $1,958,800 $979,400
- Supplements requiring clinical data	$979,400
Establishments	$526,500
Products	$98,380

Since the beginning of PDUFA, there has been a provision in place to ensure that payment of user fees does not present a hardship for small pharmaceutical companies without a revenue stream. In PDUFA II, this took the form of an application fee waiver for small businesses that are submitting their first human drug application. This provision remains in PDUFA today. Small businesses may also apply for a waiver or reduction of product and establishment fees through other waiver provisions such as the public health or barrier-to-innovation waivers. In an upcoming newsletter I will expand on these additional provisions, in the meantime you may find waiver information on our webpage and in our guidance document.

Stay tuned,

Renu Lal, Pharm.D.
CDER Small Business Assistance

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