CDER
SBIA CHRONICLES

NOVEMBER 28TH, 2017

Listen to our Audio Podcast!

Resources:

1. Guidance for Industry: Assessing User Fees Under the Prescription Drug User Fee Amendments of 2017
2. PDUFA Reauthorization Performance Goals and Procedures FY 2018-2022
3. PDUFA webpage
4. 21st Century Cures Act
5. PDUFA History
6. Dr. Woodcock Testimony

Upcoming Events:

1. SBIA Webinar - REMS Integration Initiative: An Overview – 12/04/2017

PDUFA VI: A Time for Change

October 1st, 2017 marked the date for a host of changes at FDA's Center for Drug Evaluation and Research (CDER) as the new Fiscal Year (2018) took effect. One set of changes comes from the sixth authorization of the Prescription Drug User Fee Act (PDUFA VI).

The Prescription Drug User Fee Act (PDUFA) was created by Congress in 1992 with the cooperation of both FDA and the drug industry. Prior to the first enactment of PDUFA in 1992, FDA's premarket review of drug product applications was understaffed, unpredictable, and slow. In response, Congress passed PDUFA, which authorized the FDA to collect fees to make the drug review process more efficient, without compromising drug safety, efficacy, and quality. Program enhancements have been made with each 5-year reauthorization that incorporate advances in regulatory science into drug development. Enhancements included in PDUFA VI, which support the 21st Century Cures Act, include:

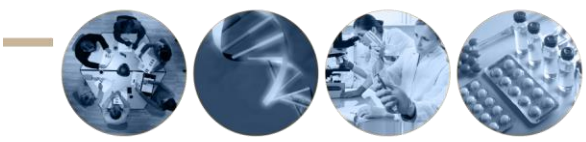
Enhanced Review Transparency and Communication: You may remember "the Program" model from PDUFA V to promote transparency and communication between the FDA review team and the applicant, increase the efficiency and effectiveness of the first cycle review process, and minimize the number of review cycles necessary for approval. FDA is now applying this model to the review of all New Molecular Entity New Drug Applications and original Biologics License Applications, including applications that are resubmitted following a Refuse-to-File decision.

Promoting Innovation through Enhanced Communication: FDA will maintain dedicated drug development communication and training staffs, focused on enhancing communication between FDA and sponsors during drug development.

Ensuring Sustained Success of Breakthrough Therapy Program: Breakthrough therapy designation is intended to expedite the development and review of drug and biological products for serious or life-threatening diseases or conditions when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. PDUFA VI provides funding and resources to this program to enable FDA to continue to work closely with sponsors throughout the designation, development, and review processes.

Early Consultation on the Use of New Surrogate Endpoints: Early consultation between the FDA and sponsors can now occur when the sponsor intends to use a biomarker as a new surrogate endpoint that has never been previously used as the primary basis for product approval in the proposed context of use.

Advancing Development of Drugs for Rare Diseases: CDER's Rare Disease Program staff will provide their expertise on approaches to studying and reviewing such drugs, continue to foster collaborations in the development of tools and data to support rare disease drug development, and facilitate interactions to increase awareness of FDA programs and engagement of patients in the decision-making process.



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Advancing Development of Drug-Device and Biologic-Device Combination Products: PDUFA VI streamlines the process for combination product review and improves the Agency's ability to assess workload and allocate resources to the review of combination products.

Enhancing Use of Real World Evidence (RWE) for Use in Regulatory Decision-Making: PDUFA VI enhances FDA's ability to consider the possibilities of using "real world" data as an important tool in evaluating drug safety and efficacy. FDA will hold public workshops and initiate activities aimed at addressing key outstanding concerns and considerations in the use of RWE for regulatory decision making.

Capturing the Patient Voice in Drug Development: FDA will conduct activities to facilitate the advancement and use of systematic approaches to collect and utilize robust and meaningful patient and caregiver input that can more consistently inform drug development and, as appropriate, regulatory decision making.

Advancing the Use of Complex Innovative Trial Designs and Model Informed Drug Development: FDA will conduct activities to facilitate the development and application of exposure-based, biological, and statistical models derived from preclinical and clinical data sources.

Enhancement and Modernization of the FDA Drug Safety System: FDA will continue to use user fees to enhance and modernize post-marketing drug safety evaluation through the expansion of the Sentinel System and timely and effective evaluation and communication of post-marketing safety findings related to human drugs.

Also of note is the PDUFA VI the fee structure, which is described in FDA's Guidance for Industry: Assessing User Fees Under the Prescription Drug User Fee Amendments of 2017. The new enhanced program fee structure is designed to achieve increased predictability, stability, and efficiency. The one-time application fee is still due upon submission, but FDA has eliminated supplement fees. The post-approval fees (annual payments due after the application is approved), which used to include the product fees and establishment fees, now solely consist of a program fee. In addition, if a company now submits an application in the middle of a fiscal year, it will not be charged a program fee until the next fiscal year.

Under PDUFA VI, a greater proportion of the target revenue allocation has shifted to more predictable fee-paying types (20% to application fees; 80% to Program fees) to provide FDA with a more stable revenue source. PDUFA VI also eliminates a provision under which applicants could apply for a waiver or refund of user fees or "the fees-exceed-costs waiver."

PDUFA V		PDUFA VI	
Application Fee – w/Clinical Data	$2,038,100	Application Fee – w/Clinical Data	$2,421,495
Application Fee – No Clinical Data	$1,019,050	Application Fee – No Clinical Data	$1,210,748
Supplement w/Clinical	$1,019,050	----------	----------
Product	$97,750	Program Fee	$304,162
Establishment	$512,200	----------	----------

FDA has already begun to implement many of these activities and goals. It will be exciting to see how new drug review, the U.S. drug safety system and ultimately the American public benefit in the next five years under PDUFA VI. Find details about PDUFA VI changes and enhancements in the PDUFA Reauthorization Performance Goals and Procedures FY 2018-2022 document.

Cheers,
Renu Lal, Pharm.D.
CDER Small Business and Industry Assistance

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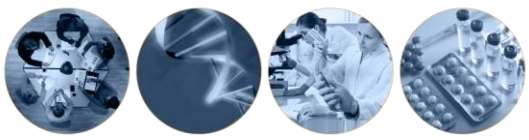

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