Prescription Drug User Fee Act (PDUFA)

Background

The Prescription Drug User Fee Act (PDUFA) was first approved in 1992 in response to prolonged review times for new drug applications at the U.S. Food and Drug Administration (FDA). PDUFA authorized FDA to collect user fees from drug sponsors to support the additional personnel needed to conduct drug reviews. The fees were authorized on a five-year basis. FDA was required to achieve certain performance goals with respect to review times and responsiveness. The program has been reauthorized five times, with the current PDUFA (VI) in place until September 2022.[1] As the major source of funding for drug reviews, this program is critical for ensuring that cancer patients have timely access to the newest therapies.

Over the years, changes were made to the types and amount of certain fees that were required. Currently, sponsors (typically manufacturers) are responsible for paying an application fee (submitted with a new application or biologics license application) and program fee (an annual fee submitted for each approved prescription drug application). The program fees make up 80 percent of the total prescription drug user fee revenue, while the application fees make up 20 percent.[2] PDUFA fees account for 72 percent of the drug review division's operating budget, with federally appropriated funds making up the rest.[2] The cost of submitting a PDUFA drug application is approximately 2.4 million dollars.[2]

The terms of each PDUFA agreement is negotiated between industry and FDA, with the final agreement submitted to Congress for approval. Historically additional scope has been added to each PDUFA agreement. For example, in the last PDUFA reauthorization, funding was included to support the creation of patient-focused drug development guidance documents. While some new responsibilities are sometimes added within each new negotiated PDUFA agreement, the must-pass nature of the reauthorization provides a vehicle for additional FDA-related policy changes to be added alongside the core PDUFA legislation, and this is where advocacy organizations typically try to influence FDA policy.

PDUFA VII Reauthorization Timeline[3]

As of June 2019, this is the timeline.

- Late Spring 2020: Federal Register notice for initial public meeting
- Summer 2020: public meeting
- Late Summer 2020: docket closes; analyze comments
- September 2020-March 2021: FDA industry discussions
    - September 2020: initiation of FDA/industry technical negotiations and FDA/stakeholder consultation meetings
    - March 2021: finalization of draft of PDUFA VII Performance Goals Letter
- April 2021-September 2021: clearance process
    - Submit draft PDUFA VII Performance Goals Letter for the U.S. Department of Health and Human Services (HHS) and the Office of Management and Budget (OMB) clearance
    - July 2021: HHS and OMB clearance
      Federal Register notice for final public meeting
- October 2021-September 2022: legislative process
    - August 2021: final public meeting
    - January 2022: deadline for Administration to Transmit Proposed PDUFA VII Performance Goals Letter to Congress
    - September 30, 2022: PDUFA VI expires

Other User Fees Programs[2]

There are other user fee programs (all synchronized on the same schedule), which FDA is authorized to undertake including:

- Medical Device User Fee Amendments (MDUFA) include medical devices used to diagnose, treat, monitor, or prevent a disease or condition (e.g. tongue depressors, bedpans, pacemakers, laser surgical devices).
    - Initiated in 2002, the MDUFA fee program is currently on its fourth cycle and accounts for 36 percent of the operating budget. On average, the cost of submitting a MDUFA device application is $310,764.
- Generic Drug User Fee Amendments (GDUFA) includes the use of generic drugs.
    - Initiated in 2012, the GDUFA fee program is currently on its second cycle and accounts for 76 percent of the operating budget. On average, submitting an application to the GDUFA program costs $171,823.
- Biosimilar User Fee Amendments (BsUFA) includes biologic drugs that are similar to brand-name biologic drugs, but not structurally identical.
    - Also initiated in 2012 and on its second cycle, the BsUFA fee program accounts for 29 percent of the operating budget. On average, the cost of submitting an application to the BsUFA drug program is $227,213.

ACS CAN Priorities

In past PDUFA cycles ACS CAN's priority was strengthening the patient representative program. This program – managed by the Advisory Committee Oversight and Management Staff (ACOMS) within the Office of the Commissioner – is comprised of over 200 patients and primary caregivers, who serve as patient representatives. The goal of this program is to ensure patient input to the benefit-risk analysis by providing a process by which patients could be present during FDA-sponsor meetings and advisory committees. Patients bring their specific disease experiences to these discussions, but they also act as general advocates for patient-focused research design and regulatory decisions.[4]

During PDUFA V negotiations ACS CAN led a successful effort to codify the then-optional patient representative program. ACS CAN would like continued support of the patient representative program as part of the new agreement and request additional reporting and evaluation of the program performance. ACS CAN's other priorities in the upcoming PDUFA negotiations include:

- Ensuring that patient stakeholders are engaged in drug development and the science of patient-focused drug development is advanced;
- Ensuring that resources and personnel are directed to the Oncology Center of Excellence where appropriate; and
- Promoting the development of policies that would allow greater use of decentralized clinical trials.

[1] U.S. Food and Drug Administration. Prescription Drug User Fee Amendments. Updated December 16, 2019. Accessed January 2020. https://www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments
[2] Congressional Research Service. FDA Human Medical Product User Fee Programs: In Brief. Published March 2017.
[3] Biotechnology Innovation Organization. PDUFA VII Reauthorization
[4] U.S. Food and Drug Administration. Learn About FDA Patient Engagement. Updated May 8, 2019. Accessed January 2020. https://www.fda.gov/patients/learn-about-fda-patient-engagement