THE PRESCRIPTION DRUG USER FEE ACT

For nearly 30 years, the Prescription Drug User Fee Act (PDUFA) has played a critical role in strengthening the U.S. Food and Drug Administration's (FDA) ability to help ensure the availability of safe and effective medicines. First created in response to a bottleneck in the drug approval system that left patients waiting for years for an under-staffed and under-funded FDA to review new drug applications, PDUFA has helped meet urgent patient needs for more timely review and approval of life-saving medicines.

Because of PDUFA, the United States now leads the world in the introduction of new medicines,[1] and the FDA human drug review program is the global gold standard for regulatory review and approval.

The History of PDUFA

Before PDUFA, it often took the FDA more than two years to review new medicines,[2] and more than 70% of medicines were first approved outside of the United States. In 1992, Congress passed the first PDUFA and now, nearly 30 years later, the average approval time for a new medicine is just 10 months and over the last five years, approximately 75% of novel drugs were approved in the United States before any other country.[3] In addition, FDA has several expedited programs for serious conditions that can accelerate not only review but also development timelines.[4]

How does PDUFA Benefit Patients?

PDUFA helps ensure patients have timely access to life-saving medicines. Specifically, PDUFA:

- Fosters development and accelerates approval of medicines for patients with serious and life-threatening diseases while maintaining the Agency's high standards for scientific rigor and patient safety.

To help make the review process more efficient and predictable, biopharmaceutical companies pay two different user fees[5] under PDUFA:

- **Application fee:** Fee due when a sponsor submits a New Drug Application (NDA) or Biologics License Application (BLA).
- **Program fee:** Annual fee for most approved prescription drug products without an approved generic.

The latest reauthorization of PDUFA (PDUFA VI) was signed into law on August 18, 2017 and took effect on October 1, 2017. It expires September 30, 2022.

Enhances FDA's scientific and medical expertise to facilitate drug review reflecting patient perspectives, including information from patient-focused drug development meetings and patient-reported outcomes.

Advances incorporation of the patient perspective into the structured approach that informs regulatory decisions on whether the benefits of a medicine outweighs its risks.

Strengthens the FDA's focus on ensuring patient safety through increased investment in the Sentinel drug safety surveillance system.

How is PDUFA VI Enhancing the Drug Development Process?

- **Innovative Clinical Trial Designs:** Establishing processes to facilitate appropriate use of innovative clinical trial designs and novel statistical methods, which help to enhance their review and acceptance in the drug review process.
- **Patient-Focused Drug Development:** Boosting the FDA's expertise and capacity to advance the science of patient input and to incorporate patient perspectives in drug development and review processes.
- **Real-World Evidence (RWE):** Providing additional resources to the FDA to help develop a better understanding of how RWE can be used in regulatory decision-making.
- **Breakthrough Therapies and Medicines for Rare Diseases:** Providing dedicated resources to prioritize the development and availability of breakthrough medicines for patients with serious and life-threatening diseases and helping FDA to advance the development and approval of medicines for rare diseases, including pediatric rare diseases.
- **Post-Market Safety:** Providing significant resources to enhance the FDA's ability to review, track and communicate important post-market safety information, including investments to expand the Sentinel system's capabilities and enhance the communication process with stakeholders on the use of Sentinel data.

New developments in medical and fundamental science — including immunotherapies and cell and gene therapies — hold the promise of treating debilitating diseases such as Alzheimer's disease, cancer, diabetes and many rare disorders. Fulfilling this promise depends on a modern regulatory framework, that PDUFA facilitates, that can serve patients by providing timely, science-based regulatory decisions.

PDUFA Supports COVID-19 Pandemic Response

PDUFA is also helping FDA fulfill its central mission of protecting the public health as the Agency responds to the COVID-19 pandemic. Since the start of the pandemic, FDA has been working with biopharmaceutical companies to accelerate the development of safe and effective COVID-19 therapeutics and vaccines. FDA has provided timely recommendations, regulatory guidance and technical assistance to developers of potential treatments and vaccines for COVID-19, including on the use of novel approaches to clinical trials and drug review. FDA has also provided clarity to biopharmaceutical sponsors on how to continue to advance the development of new medicines during the current pandemic. PDUFA has helped support this engagement by ensuring FDA's human drug review program has the infrastructure, scientific expertise and experience with cutting-edge drug development approaches needed to support continued innovation and inform efficient regulatory decision-making.

Endnotes

1. https://www.fda.gov/media/144982/download
2. https://wayback.archive-it.org/7993/20170406002629/https:/www.fda.gov/AboutFDA/ReportsManualsForms/Reports/UserFeeReports/PerformanceReports/ucm117257.htm#DISCUSSIONOFFY95PERFORMANCE
3. https://www.fda.gov/drugs/development-approval-process-drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products
4. https://www.fda.gov/media/86377/download
5. https://www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments