THE PRESCRIPTION DRUG USER FEE ACT (PDUFA VII)

The Prescription Drug User Fee Act (PDUFA) has helped the U.S. Food and Drug Administration (FDA or Agency) fulfill its central mission – to help protect and advance the public health – by allowing the Agency to keep pace with the number and complexity of innovative drugs and biologics entering the review pipeline. In large part because of PDUFA, the United States now leads the world in the introduction of new medicines,[1] and the FDA's human drug review program is the global gold standard for regulatory review and approval.

Since 1992 when the program was enacted, PDFUA has provided more timely access to more than 1,700 new drugs and biologics including treatments for cancer, rare diseases, cardiovascular, neurological and infectious diseases. PDUFA continues to play a critical role in strengthening the FDA's ability to review human drug applications. The program also provides biopharmaceutical companies with greater regulatory predictability, which fosters industry investment in research and development. At each five-year reauthorization of PDUFA, FDA and the biopharmaceutical industry have the opportunity to advance initiatives that further support innovation and enhance the regulatory review process.

The History of PDUFA

Before PDUFA, it often took the FDA more than two years to review new medicines,[2] and the vast majority of new medicines were first launched outside of the United States.[3] In 1992, Congress passed the first PDUFA as a bipartisan solution to help provide the FDA human drug review program with stable and predictable funding by supplementing Congressional appropriations. Now, the median approval time for a new medicine is 10 months for standard applications and 8 months for priority review[4] applications. Over the last five years, approximately 75% of novel drugs were approved in the United States before any other country.[5]

To help make the review process more efficient and predictable, biopharmaceutical companies pay two different user fees under PDUFA[6]:

- **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.

A Closer Look at PDUFA VII: Modernizing the U.S. Regulatory and Drug Development Paradigm

The PDUFA VII agreement will strengthen review fundamentals at FDA while enhancing accountability and transparency and ensuring stable growth of the program:

Strengthen Scientific Dialogue and Advance Innovation

- Expanding opportunities for obtaining FDA feedback throughout the drug development process.
- Establishing a new Split Real Time Application Review (STAR) pilot program to shorten time to the action date for certain novel uses of approved therapies.
- Continuing to advance product development for rare diseases, including rare diseases in children.

Enhance Patient-Centric Drug Review and Support Safety Monitoring

- Advancing the incorporation of patient-centric data, including patient preference information, into drug development and regulatory reviews.
- Providing significant resources to support the Agency's ability to review, track and communicate important post-market safety information.
- Supporting implementation of a standardized process for determining necessity and type of pregnancy postmarketing studies.

Support the Next Wave of Advanced Biological Therapies

- Substantially strengthening the Center for Biologics Evaluation and Research (CBER)'s staff capacity and capability to support the development and review of cell and gene therapies.
- Facilitating broader use of novel trial designs and approaches for rare and ultra-rare diseases and evaluating their application to more common diseases.
- Expanding understanding of patient perspectives on gene therapy products.

Modernize Regulatory Evidence Generation and Drug Development Tools

- Advancing the use of real-world evidence (RWE) for regulatory decision-making, including for approval of new indications or to satisfy postmarketing study requirements.
- Facilitating further use of complex adaptive and other novel clinical trial designs, including use of Bayesian approaches.
- Advancing consistency and predictability around the use of modeling and simulations, including model-informed drug development (MIDD) approaches, in regulatory decision-making.

Enhance Innovation in Manufacturing and Product Quality Reviews

- Facilitating the use of innovative manufacturing technologies for both products in development and those commercially available.
- Facilitating Chemistry, Manufacturing & Controls (CMC) readiness for products with accelerated clinical development timelines.
- Advancing use of alternative tools to assess manufacturing facilities named in pending applications, including incorporation of best practices from the use of such tools during the COVID-19 pandemic.

Advance Digital Technologies and Information Technology (IT) Infrastructure

- Directing FDA to establish a framework to facilitate the adoption of innovative digital health technologies (DHTs).
- Advancing the use of DHTs in decentralized clinical trials, including the potential for DHTs to increase diverse patient populations in clinical trials.
- Modernizing data and IT capacity and capabilities, including adoption of cloud-based technologies.

Enhance FDA Hiring, Retention and Financial Management

- Building on the foundational work started in PDUFA VI to modernize financial and staff resource management, accountability, and transparency.

New developments in medical and fundamental science—including immunotherapies and cell and gene therapies—hold the promise of treating debilitating diseases such as 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.

It is critical that Congress reauthorizes the PDUFA program before its expiration in September 2022.

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/media/73625/download
4. https://www.fda.gov/media/138325/download
5. https://www.fda.gov/drugs/development-approval-process-drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products
6. https://www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments