

What is PDUFA?

The **Prescription Drug User Fee Act (PDUFA)** is a landmark, bipartisan piece of legislation which transformed the Food and Drug Administration's (FDA) ability to assure the safety and efficacy of therapeutic drugs. The law, originally passed in 1992, establishes a well-defined regulatory process to assess new therapeutics in a timely and transparent way.

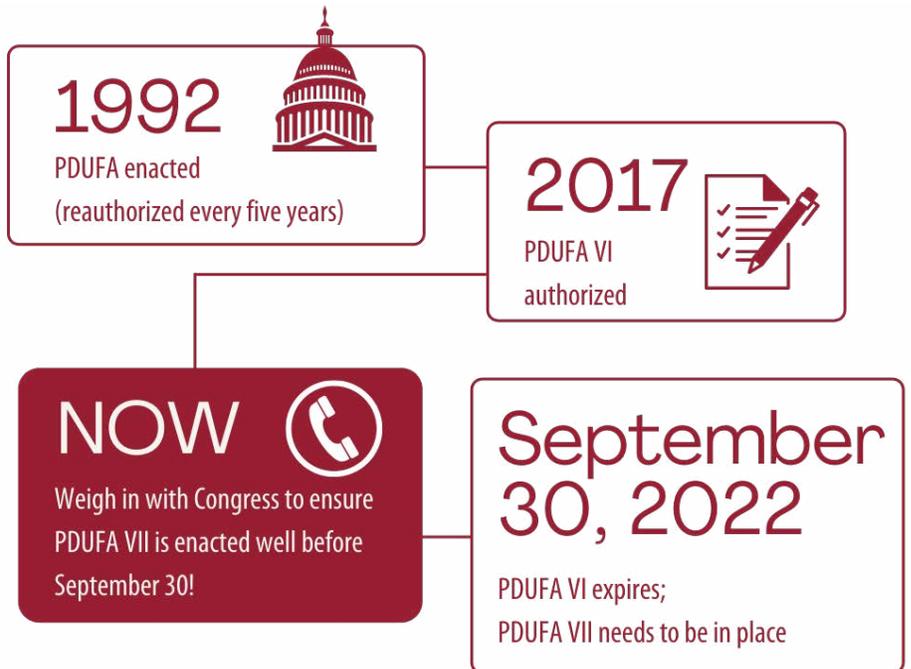
Before the enactment of PDUFA, it took the FDA, on average, 29 months to complete a drug application review. With the benefit of enhanced revenues from the law's user fees, the median approval time for new medicines is just 10 months for standard applications and 8 months for priority applications.

The **law must be reauthorized every five years** following a complex negotiation between the agency and industry to produce an agreement that is then sent to Congress for consideration and approval. FDA public meetings with patient advocacy organizations, professional societies, and other third party stakeholders are also part of the agreement development process. Based on the success of the PDUFA model, FDA user fees have been adopted for related sectors. These statutes include MDUFA (medical devices), GDUFA (generic drugs), and BsUFA (biosimilars). The authorization of all four statutes expires on September 30, 2022.

At present, just over half of the FDA's budget is funded through annual appropriations while the remainder is funded through user fees paid by industry. In 2021, 54%, or \$3.3 billion, of FDA's budget was funded through annual appropriations. The remaining 46%, or \$2.8 billion, was funded by industry user fees.

What's Next for PDUFA?

On August 23, 2021, the FDA released its commitment letter for PDUFA VII, the next reauthorization of the law. The letter outlines performance goals and procedures the FDA proposes to implement in PDUFA VII based on input from public stakeholders, industry, and the FDA.



The PDUFA VII commitment letter continues the practice begun in the first PDUFA by committing to the safe and effective review of medical products in a timely, transparent, and efficient manner. The commitment letter sets a goal of FDA review and action on new drug applications of 10 months for standard drugs and 6 months for priority drugs.

Other important provisions in the commitment letter include enhancements to the FDA's Patient-Focused Drug Development (PFDD) initiative begun in PDUFA V. PDUFA VII incorporates PFDD in the development of cell and gene-based therapies and includes several other metrics to enhance the focus on patient input. The letter also proposes an increase in FDA staffing to accommodate the

expansion of submissions for cell and gene therapy products. It includes provisions to incorporate real-world, evidence-based strategies; to advance rare disease drug development; and to strengthen post-marketing requirements.

Congress is well underway in considering the PDUFA VII agreement. Both committees of jurisdiction (the Senate Health, Education, Labor and Pensions Committee and the House Energy and Commerce Committee) have held hearings and mark-ups are expected to begin soon. Energy and Commerce released their bill on a bipartisan basis on May 4, 2022. The reauthorization process has in the past been used as a vehicle to attach other FDA-related provisions, and that will likely be the case this year. Topics arising during the authorization process include: the use of real-world evidence; changes in the accelerated approval process; concrete steps to enhance clinical trial diversity and incorporation of lessons learned from COVID-19.

PDUFA VI expires on September 30, 2022, and reauthorization is regarded as “must-pass” since the authority to collect user fees expires on that date. Ideally, it will be completed by the August recess. Failure to enact PDUFA VII in a timely manner would greatly impact FDA’s ability to operate and put patient innovation at risk.

Resources

- For more information on PDUFA, visit <https://bit.ly/FDA-PDUFA>.
- For ongoing coverage of PDUFA VII progress, visit <https://www.raps.org/news-and-articles>.

