

PDUFA VI: ADVANCING A NEW ERA OF MEDICINE FOR PATIENTS

The Prescription Drug User Fee Act (PDUFA VI) was first created in response to a bottleneck of new medicine approvals that left patients waiting for years for an under-staffed and under-funded U.S. Food and Drug Administration (FDA or Agency) to review new drug applications. Before PDUFA, it often took the FDA more than two years to review new medicines, and more than 70 percent of medicines were first approved outside of the United States.

In 1992, Congress passed the first PDUFA and now, more than 25 years later, the average approval time for a new medicine is just 10 months, and 78 percent of medicines are now first approved in the U.S.

To help make the review process more efficient, biopharmaceutical companies pay two different user fees under PDUFA VI:

- **Application fee:** Fee due when a sponsor submits a New Drug Application (NDA) or Biologics License Application (BLA).
- **Program fee:** Post-approval annual fee for most prescription drug products approved by October 1 of each fiscal year for which no generic drug exists.

The latest reauthorization of PDUFA (PDUFA VI) was signed



PDUFA VI builds upon previous PDUFA reauthorizations by strengthening the FDA's innovative human drug review program and helping the Agency remain the "gold-standard" for review of new medicines.

PDUFA VI helps create the predictable, timely and efficient regulatory review needed to support biopharmaceutical investment and allow the FDA to keep pace with scientific advances while enhancing patient safety.

Key PDUFA VI initiatives include incorporating the patient perspective into drug development, enabling the regulatory acceptance of real-world evidence (RWE) and advancing the use of innovative clinical trial designs.



REAL-WORLD EVIDENCE

Real-world evidence can lead to more efficient drug development programs, provide more robust information about the benefits and risks of new medicines and can ultimately lead to more timely access to innovative, safe and effective medicines for patients.

- Under PDUFA VI and the 21st Century Cures Act, the FDA is conducting public workshops with key stakeholders to inform Agency guidance on how RWE can contribute to the assessment of the safety and effectiveness of medicines.
- PhRMA member companies are working with FDA on pilot programs to establish new scientifically rigorous and valid drug development approaches using RWE that will provide valuable information to shape regulatory decision-making.

[Read more](#) ↓



CLINICAL TRIAL DESIGNS

Innovative clinical trial designs have the potential to enhance the efficiency of drug development and review processes by helping accelerate patient access to safe and effective new medicines. PDUFA VI enhances drug development by establishing processes to facilitate appropriate use of innovative clinical trial approaches, including adaptive clinical trial designs and Bayesian statistical methods.

- Under PDUFA VI, the FDA is developing staff capacity for innovative trial design review and is convening a public workshop and related guidance development to provide clarity to sponsors and external stakeholders on the use of adaptive clinical trials and Bayesian methodologies.
- The FDA also initiated a voluntary pilot program for complex innovative clinical trial designs to validate alternative drug development approaches and review models that will provide valuable information to shape future regulatory actions. This pilot program will develop illustrative examples that provide the foundation for broad, consistent implementation across review divisions.



PATIENT-FOCUSED DRUG DEVELOPMENT (PFDD)

As important stakeholders in the drug development process, patients, family members and caregivers can provide unique and valuable perspectives on their condition and available treatment options.

- Under PDUFA VI, PFDD guidance documents are being developed, so patient perspectives can help inform the evaluation of a medicine's benefits and risks and provide context for FDA's regulatory decision making. By providing their perspective, patients can inform clinical trial endpoints and help researchers identify clinical outcomes that matter most to patients and their caregivers.



PDUFA VI is propelling the new era of medicine by harnessing innovative research and development methods to drive forward groundbreaking therapies for a range of disease states.

