

# WHAT IS PDUFA VI?

For nearly 25 years, the Prescription Drug User Fee Act (PDUFA) has provided greater consistency, certainty and predictability for the U.S. Food and Drug Administration's (FDA) human drug review program and has helped to bring safe and effective innovative medicines to patients. Because of PDUFA, the United States now leads the world in the introduction of new medicines and the FDA human drug review program is the global gold standard for regulatory review and approval.



PDUFA VI builds upon the successes of previous PDUFA agreements with continued focus on ensuring patient safety, maintaining the FDA's high standards for regulatory review and ultimately promoting timely access to safe and effective innovative medicines, including treatments for patients with rare, serious or life-threatening diseases. PDUFA VI will help ensure the long-term sustainability of the FDA's human drug review program and strengthen agency capabilities by:

- Creating efficiencies that can accelerate the development and availability of new medicines to patients while providing scientific and regulatory predictability that will foster continued biopharmaceutical innovation;
- Facilitating the systematic integration of the patient perspective into the development and regulatory review of innovative medicines;
- Enhancing the FDA's access to the tools, processes and expertise necessary to keep pace with the latest scientific advances in drug development and regulation;
- And helping to ensure the FDA can hire and retain a strong scientific and medical workforce to advance its public health mission.



The FDA released the PDUFA VI performance goals letter in July 2016, and Congress will consider reauthorization of the program before its expiration in September 2017.

## How Does PDUFA VI Benefit Patients?

**Strengthens efforts to incorporate patient perspectives into the drug development and review process:**

- Fosters enhanced development and approval of medicines for patients with serious and life-threatening diseases;
- Enhances FDA's scientific and medical expertise and strengthens efforts to advance the science of patient input and to incorporate patient perspectives into the drug development and review process, including the use of patient reported outcomes ("PROs");
- Advances use of a structured approach to inform regulatory decisions on whether the benefits of a medicine outweighs its risks;
- And strengthens the FDA's focus on ensuring patient safety through increased investment in the Sentinel drug safety surveillance system.

## How Does PDUFA VI Advance Medical Innovation?

### Supports development and application of 21st Century regulatory science:

- Helps to facilitate timely, efficient regulatory review of innovative treatments;
- Enhances the FDA’s biomarker qualification pathway and establishes a dedicated process to improve use of biomarkers as surrogate endpoints in drug development;
- Explores the use of real world evidence (RWE) for regulatory decision-making;
- And facilitates the use of innovative clinical trial approaches to enhance the efficiency of the drug development process.

## How Does PDUFA VI Build a More Effective FDA?

### Enhances predictability and efficiency of the human drug review and approval process:

- Advances development and approval of medicines for rare diseases, including pediatric rare diseases;
- Builds upon the success of the PDUFA V review model for new molecular entity medicines (NME Review Program) and preserves the 8-month (priority) and 12-month (standard) FDA review timeline for new drug and biologics applications;
- Seizes on success of Breakthrough Therapy Program by investing resources to continue prioritizing development of breakthrough medicines for patients with serious and life-threatening diseases;
- And streamlines regulatory review of combination medicines (products composed of a drug or biologic and a device) and improves coordination between FDA review centers.

### Continues to strengthen FDA’s drug safety system:

- Enhances existing FDA tools and technology related to post-marketing safety of approved drugs, including providing additional resources dedicated to expanding the capabilities of the agency’s Sentinel system.

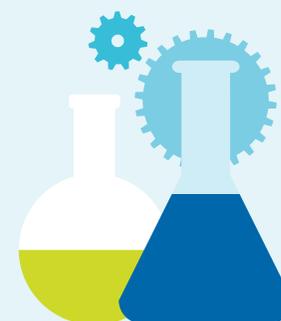
### Promotes long-term stability and sustainability of FDA’s human drug review program:

- Helps to ensure FDA can hire and retain a strong scientific and medical workforce to advance its public health mission;
- And establishes common sense financial reforms that provide greater transparency predictability for FDA’s human drug review program.

## PDUFA IS GOOD FOR..



PATIENTS



MEDICAL INNOVATION



THE FDA