

# The Prescription Drug User Fee Act (PDUFA) Overview & PDUFA VII Process & Timeline



# PDUFA was First Enacted in 1992 as a Bipartisan Solution to Increase the Efficiency of Prescription Drug Review at FDA



PDUFA has been reauthorized five times since 1992 and has consistently enjoyed strong bipartisan support

## WHY



- The emerging AIDS epidemic in the 1980s sparked demand for faster review times
- AIDS activist group ACT UP! closed down the FDA to protest the slow process of drug approval

## WHO



- FDA, Congress and Industry worked together to ensure FDA is resourced appropriately to support the regulatory review process for new medicines

## HOW



- PDUFA increased FDA resources by authorizing the FDA to collect user fees from the biopharmaceutical industry
- PDUFA also established certain performance goals for FDA related to application review, drug safety, and the drug development process

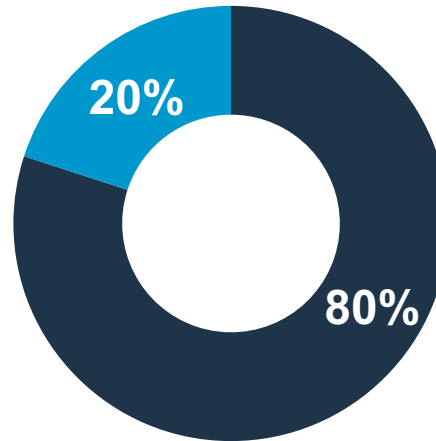
# Biopharmaceutical Companies Pay Two Different User Fees Under PDUFA

PDUFA user fees **supplement** funding received by FDA through congressional appropriations

- Enforced through limiting conditions included in the authorizing law

**Application Fee** - Fee due when a sponsor submits a New Drug Application (NDA) or Biologics License Application (BLA)

**20%** of the total prescription drug user fee revenue comes from application fees



**Program Fee** - Annual fee for most approved prescription drug products without an approved generic

**80%** of the total prescription drug user fee revenue comes from program fees

Having the majority of PDUFA user fees revenue come from **program fees** ensures a stable funding source for FDA activities

# PDUFA is an Important Tool with Defined Parameters

The PDUFA program provides additional resources to FDA for the review of new drugs, while also providing sponsors with greater predictability and enhanced engagement with the Agency during drug development

The PDUFA provisions are limited to improving FDA performance in certain areas or allocating resources to new regulatory review areas and functions

- The agreement cannot be used to change regulatory policy or alter FDA statutory requirements
- In exchange for paying user fees, Industry receives from FDA a commitment to meet certain performance goals
- Prior to each five-year reauthorization cycle, FDA and industry negotiate the performance goals, which are finalized in a written agreement

PDUFA is reauthorized by Congress every five years, providing the biopharmaceutical industry and FDA with the opportunity to make changes or add new provisions that will further improve the program

# PDUFA Helps Ensure More Timely Patient Access to Safe and Effective New Medicines

CDER's Annual Novel Drug Approvals



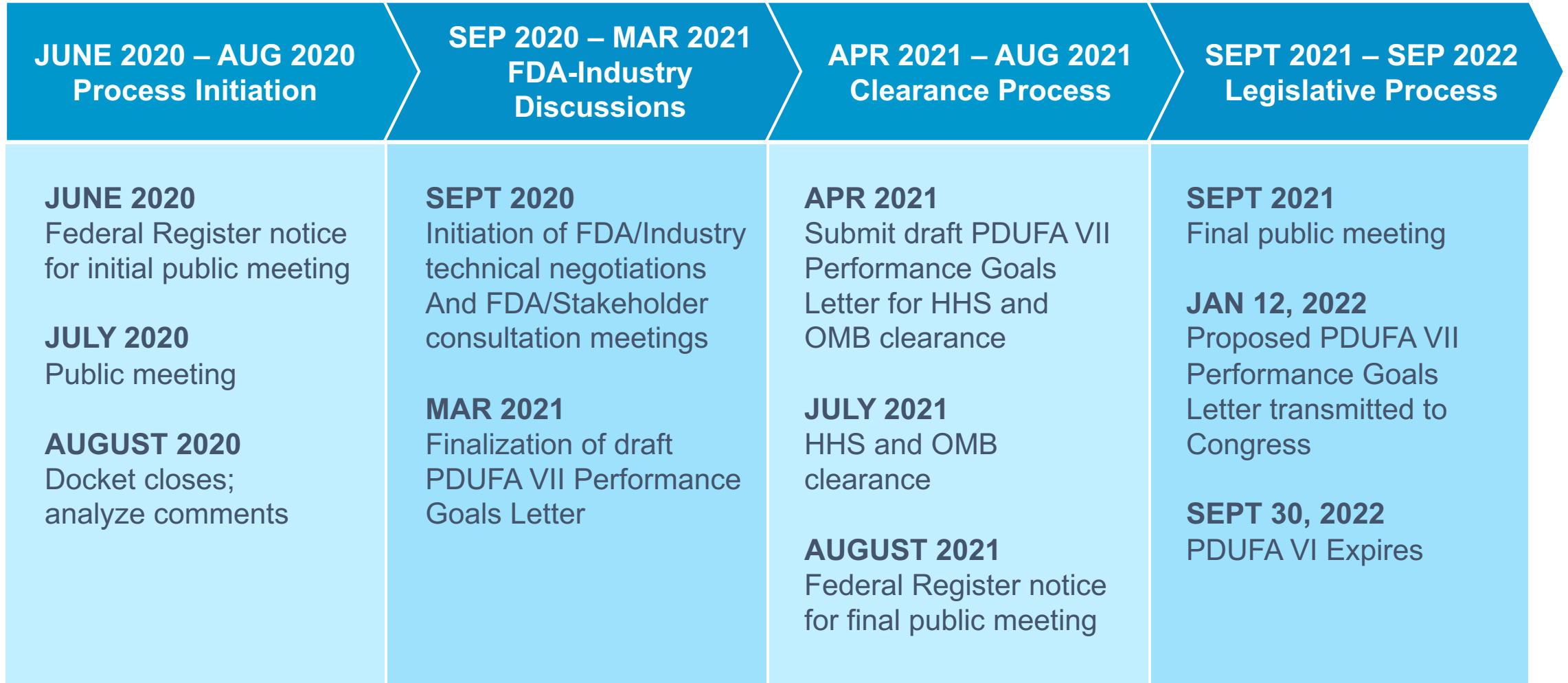
Before PDUFA, it often took FDA **more than two years** to review new medicines

The median approval time for a new medicine is now just **10 months for standard applications and 8 months for priority**

**In 2021, CDER approved 50 novel drugs:**

- 86% were approved on the first cycle
- 76% were approved in the US before any other country
- 52% were for rare or orphan diseases
- 54% were First-in-Class

# PDUFA's Reauthorization Includes Several Phases



# PDUFA VII Commitment Letter

# PDUFA VII will Play a Critical Role in Continuing to Advance an Effective, Science-based Regulatory Review Program

The overarching PDUFA VII efforts are aimed at modernizing the U.S. regulatory and drug development paradigm and addressing new areas such as digital health technologies, cell and gene therapies, and manufacturing. PDUFA VII will:

Strengthen scientific dialogue and advance innovation

Enhance patient-centric drug review and support safety monitoring

Support the next wave of advanced biological therapies

Modernize regulatory evidence generation and drug development tools

Advance digital technologies and IT infrastructure

Enhance innovation in manufacturing and product quality reviews

Build on PDUFA VI efforts to modernize FDA financial and staff resource management



# PDUFA VII will Strengthen Scientific Dialogue and Advance Innovation

Establishing a Split Real Time Application Review (STAR) pilot program to shorten the time from the date of complete submission to the action date in order to allow earlier access to therapies that address an unmet need.

- Seeks to expedite patient access to novel uses for existing therapies by supporting initiation of review earlier than would otherwise occur and therefore allowing earlier approval for qualified efficacy supplements.
- Will apply across all therapeutic areas and review disciplines for applications that meet specific criteria

Establishing new formal meeting types between FDA and sponsor for focused discussion on narrow set of issues about innovative approaches and early engagement before IND submission.

Establishing a Rare Disease Endpoint Advancement pilot program to support development for drugs to treat rare diseases and offering additional engagement opportunities for development programs.

# PDUFA VII will Enhance Patient-Centric Drug Review and Support Safety Monitoring

Continuing to incorporate patient voice in drug development and use of patient preference information to support regulatory decision making.

Expanding safety monitoring program to encourage access to distributed data network to conduct safety surveillance.

# PDUFA VII will Support the Next Wave of Advanced Biological Therapies

Providing FDA with resources to strengthen staff capacity and capability by augmenting the Cell and Gene Therapy Program to address growing workload.

Streamlining and harmonizing procedures, processes and interactions by enhancing and improving best practices for communication for Cell and Gene Therapy Products, including use of novel trial designs.

# PDUFA VII will Modernize Regulatory Evidence Generation and Drug Development Tools

## Real World Evidence (RWE)

- FDA will provide earlier and increased advice and improve the quality and acceptability of RWE-based approaches in support of new intended labeling claims, including approval of new indications or to satisfy post-approval study requirements
- FDA will establish an RWE pilot program to advance the use of RWE for regulatory decision-making
- FDA will convene a public workshop and issue revised or new draft guidance on RWE

**Model-informed Drug Development (MIDD):** FDA will facilitate the advancement and use of MIDD approaches through the continuation of the PDUFA VI paired meeting program and through the issuance of a Request for Information (RFI) to elicit input for identifying focus areas for future policy or guidance development and stakeholder engagement.

## Complex Innovative Trial Designs

- FDA will facilitate the advancement and use of complex adaptive, Bayesian, and other novel clinical trial designs, through building on existing efforts in the PDUFA VI Complex Innovative Design (CID) pilot and continuing to develop Center for Drug Evaluation and Research (CDER) and CBER staff capacity
- FDA will convene a workshop to discuss aspects of complex adaptive, Bayesian, and other novel clinical trial designs and publish draft guidance on Bayesian approaches

# PDUFA VII will Advance Digital Technologies and IT Infrastructure

## IT Modernization

- FDA will enhance transparency on IT activities and modernization plans to support the PDUFA program. FDA will continue to engage quarterly and annually with industry on challenges, emerging needs, and progress on initiatives relevant to PDUFA and provide an opportunity for industry input
- FDA will establish a strategy on data-driven regulatory initiatives
- FDA will modernize CBER data and IT capacity and capabilities
- FDA will complete Electronic Submissions Gateway (ESG) transition to cloud
- FDA will initiate demonstration projects to enable adoption of cloud-based technologies

**Bioinformatics:** PDUFA VII will provide bioinformatics and computational biology capacity to strengthen FDA's ability to conduct and support reviews of submissions containing a variety of biological data such as Next Generation Sequencing

## Digital Health Technologies (DHTs)

- FDA will expand capacity and advance a digital health technologies framework that will promote regulatory consistency and coordination across FDA on digital health
- FDA will establish a committee to promote consistency across centers regarding DHT-based policy, procedure, and analytic tool development
- FDA will convene public workshops on the use of DHTs in regulatory decision-making; identify demonstration projects to inform methodologies for DHT evaluation; and issue DHT-related guidances
- FDA will enhance its IT capabilities to support the review of DHT-generated data

# PDUFA VII will Enhance Innovation in Manufacturing and Product Quality Reviews

FDA will issue a draft guidance on the use of alternative tools to assess manufacturing facilities named in pending applications, including the utilization of new or existing technology platforms, as appropriate.

FDA is committed to a series of deliverables (Manual of Policies and Procedures (MAPP), Pilot, Public Workshop, Strategy Document) intended to facilitate CMC readiness for products with accelerated clinical development.

The Pilot will incorporate contemporary learnings and the use of science- and risk-based approaches and submission strategies and will feature increased communication between FDA and sponsors.

FDA will advance utilization and implementation of innovative manufacturing technologies by conducting a public workshop and issuing a draft strategy document that will outline the specific actions the Agency will take to facilitate their adoption.

# PDUFA VII will Build on PDUFA VI Efforts to Modernize FDA Financial and Staff Resource Management

## Hiring and Resource Management and Accountability

- FDA will publish an updated implementation plan that will describe how the Agency's resource capacity planning (RCP) function and time reporting will continue to be implemented during PDUFA VII
- FDA will conduct a third-party assessment of the capacity planning adjustment (CPA)
- FDA will publish an updated 5-year financial plan with annual updates and convene an annual public meeting to discuss the 5-year financial plan and the Agency's progress in implementing RCP
- FDA will hire 352 FTEs over the course of PDUFA VII to address the new deliverables outlined in the Commitment Letter, with hiring goals that will be tracked and hiring progress reporting
- FDA will use an independent contractor with expertise in assessing Human Resource (HR) operations to conduct a targeted assessment of the hiring and retention of staff for the human drug review program

**Strategic Hiring and Retention:** FDA will address their hiring and retention capabilities through utilization of their direct hiring and pay authorities granted under the 21<sup>st</sup> Century Cures Act

**Learn more at  
[PhRMA.org/PDUFA](https://PhRMA.org/PDUFA)**

