U.S. FOOD & DRUG ADMINISTRATION

WELCOME

We will begin the meeting promptly at 9:00am.

U.S. FOOD & DRUG ADMINISTRATION

Public Meeting on the Reauthorization of the Prescription Drug User Fee Act (PDUFA)

Monday, July 14th, 2025

Nana Adjeiwaa-Manu

Center for Drug Evaluation and Research, FDA

Meeting Host, Program Evaluation and Implementation Staff, Office of Program and Strategic Analysis, Office of Strategic Programs

AGENDA

- Welcome and Introduction
- Opening Remarks
- PDUFA Background and Reauthorization Process
- Industry Comments
- **Break**
- Public Comment
- Closing Remarks

Submit your public comments by August 14!

You can access the docket via:

- FDA's public meeting webpage

  (https://www.fda.gov/industry/public-meeting-reauthorization-prescription-drug-user-fee-act-pdufa-07142025)

- Directly through the Federal Register

  (https://www.federalregister.gov/documents/2025/05/19/2025-08824/reauthorization-of-the-prescription-drug-user-fee-act-public-meeting-request-for-comments)

Marty Makary

FDA Commissioner

PDUFA
Background and Reauthorization Process

Andrew Kish

Center for Drug Evaluation and Research, FDA

Director, Office of Program and Strategic Analysis, Office of Strategic Programs

Outline for this briefing

- PDUFA Background
- Review Program and Performance
- Financial Background and Fee Structure
- Reauthorization Process Overview

PDUFA Background

Before 1992, timeliness of FDA drug review was a big concern.

PDUFA I

- User fees added resources for more review staff to eliminate the backlog of overdue applications and improve review timeliness
- FDA agreed to meet specific performance goals

Result:

- More predictable, streamlined process
- Patients gained earlier access to new drugs and biologics approved since 1992
- Overall, clinical development time and average time to approval dropped since 1992

PDUFA introduced fees to help enhance the review process.

- Fee funds are added to appropriated funds and are intended to increase staffing and other resources to speed and enhance review process
- User fees pay for services that directly benefit fee payers*
- Fee discussions with industry focus on desired enhancements in terms of specific aspects of activities in "process for the review of human drugs"
  - What new or enhanced process will the FDA want or industry seek to include in the next 5 years?
  - What is technically feasible?
  - What resources are required to implement and sustain these enhancements?
  - No discussion of policy.
- Experience: *Devil is in the Details*

** OMB Circular A-25; direct benefit distinguishes user fees from tax*

Performance commitments and fee funding have evolved since 1992.

PDUFA | *1993-1997*
Added funds for pre-market review; reduced backlog and set predictable timelines (goals) for review action

PDUFA II (FDAMA) | *1998-2002*
Shortened review timelines, added review goals; added process and procedure goals; added some funding

PDUFA III (BT Preparedness & Response Act) | *2003-2007*
Significantly added funding; increased interaction in first review cycle (GRMPs); allowed limited support for post-market safety

PDUFA IV (FDAAA) | *2008-2012*
Increased and stabilized base funding; enhanced pre-market review; modernized post-market safety system

PDUFA V (FDASIA) | *2013-2017*
Small increase to base funding; review enhancements increased communication with sponsors; strengthened regulatory science & post-market safety; set electronic data standards

PDUFA VI (FDARA) | *2018-2022*
Modernized the user fee structure; focused on HR and financial management improvement; enhanced use of regulatory tools and staffing for breakthrough therapy reviews; focused on communication with industry; explored RWE in regulatory decision-making

PDUFA VII (FDAUFRA | 2023-2027):

- Enhanced staffing for cell and gene therapy products
- Incorporated allergenics
- Introduced timelines and performance goals for pre-approval review of post-marketing requirements, use-related risk analysis, and human factor protocol submissions
- Introduced new meeting types for focused discussion
- Introduced new pilot programs related to rare diseases, real-world evidence, manufacturing, and drugs for unmet therapeutic areas
- Enhanced product quality reviews; chemistry, manufacturing, and controls approaches; and utilization of innovative manufacturing techniques
- Modernized information technology systems, bioinformatics support, and use of digital health technologies

The following slides describe enhancements in specific areas.

PDUFA VII includes over 250 performance enhancement commitments.

Performance enhancement commitments are commitments that are not review goals.

The 250+ performance enhancement commitments in PDUFA VII include:

- 70+ new or updated pilots, programs or processes
- 50+ data/list postings to the public website
- 20+ public meetings or public workshops
- 30+ new or revised guidances
- 30+ public reports
- 30+ other commitments

CBER Review Support

Goal: Enhance CBER's capacity to guide development and review innovative products such as cell and gene therapies

Enhancements:

- Increase in staff capacity and capability to review cell and gene therapy products
- Support for stakeholder meetings and guidance related to patient perspectives and novel methods in cell and gene therapy
- Incorporation of allergenic extract products into PDUFA program

Pre-Market Review

Goal: Introduce new approaches to improve efficiency and expand communication in the human drugs review program

Enhancements:

- New performance goals and timelines for review of post-marketing requirements
- New meeting types to discuss focused issues, opportunities to follow-up on clarifying questions, and documents updated to incorporate best practices for meetings
- New procedures, timelines, and guidance for use-related risk analysis and human factor validation study protocols
- 3 new pilot programs to:
    - Expedite review of efficacy supplements to treat serious conditions with unmet medical needs (STAR)
    - Advance rare disease drug development by providing support for efficacy endpoint development (RDEA)
    - Improve the quality and acceptability of real-world evidence (RWE)

Regulatory Decision Tools

Goal: Continue application of innovative methods and tools to enhance regulatory decision-making

Enhancements:

- Continued support for patient-focused drug development methods, including workshops, guidance, and stakeholder outreach
- Advancement of the Model-Informed Drug Development (MIDD) and Complex Innovative Designs (CID) pilots into programs

Post-Market Safety

Goal: Ensure safe use of medicines through continued enhancements to our drug safety system

Enhancements:

- New REMS review performance goals for review of methodological approaches and study protocols for REMS assessments
- New and updated processes and guidance relevant to REMS assessment
- Continued support for the Sentinel Initiative
- Support for assessing outcomes of pregnancies in women exposed to drugs and biological products
- Support for developing new methods to support causal inference for product safety questions and advance our understanding of how real-world evidence may be used for studying effectiveness

Chemistry, Manufacturing, and Controls

Goal: Facilitate manufacturing readiness and use of innovative manufacturing technologies

Enhancements:

- Improved communication during review, including updated processes, a third-party assessment, and goals for notifying sponsors of inspections
- Guidance on the use of alternative tools to assess manufacturing facilities named in pending applications
- Pilot program (CDRP) to facilitate CMC readiness for products with accelerated clinical development timelines
- Public workshop and strategy document on the utilization of innovative manufacturing technologies

Digital Health and Informatics

Goal: Invest in modern technology and bioinformatics to support enhanced and streamlined drug development and review

Enhancements:

- Expanding capacity to advise the development and implementation of digital health technologies (DHTs) and to evaluate their outputs
- New DHT framework document to guide the use of DHT-derived data in regulatory decision-making
- Resources to modernize FDA's information technology systems, including the Electronic Submission Gateway and cloud-based technologies
- Continued transparency and collaboration with Industry via regular meetings and a modernization strategy

Finance, Hiring, and Retention

Goal: Enhance financial management and transparency and focus on the strategic hiring and retention of world-class technical and scientific staff

Enhancements:

- Mature the capacity planning function by publishing an implementation plan and annual updates
- Continue to promote transparency by publishing financial plans, holding public meetings, posting hiring updates, and conducting an assessment of hiring and retention practices
- Modifications to fee adjustments to provide funding for highly qualified technical staff and to manage financial risks to the program

Performance data and completed deliverables are available to the public.

Completed PDUFA VII deliverables can be found on FDA's website:

https://www.fda.gov/industry/prescription-drug-user-fee-amendments/completed-pdufa-vii-deliverables

FDA released a PDUFA performance dashboard that allows users to view and download current and historical performance data:

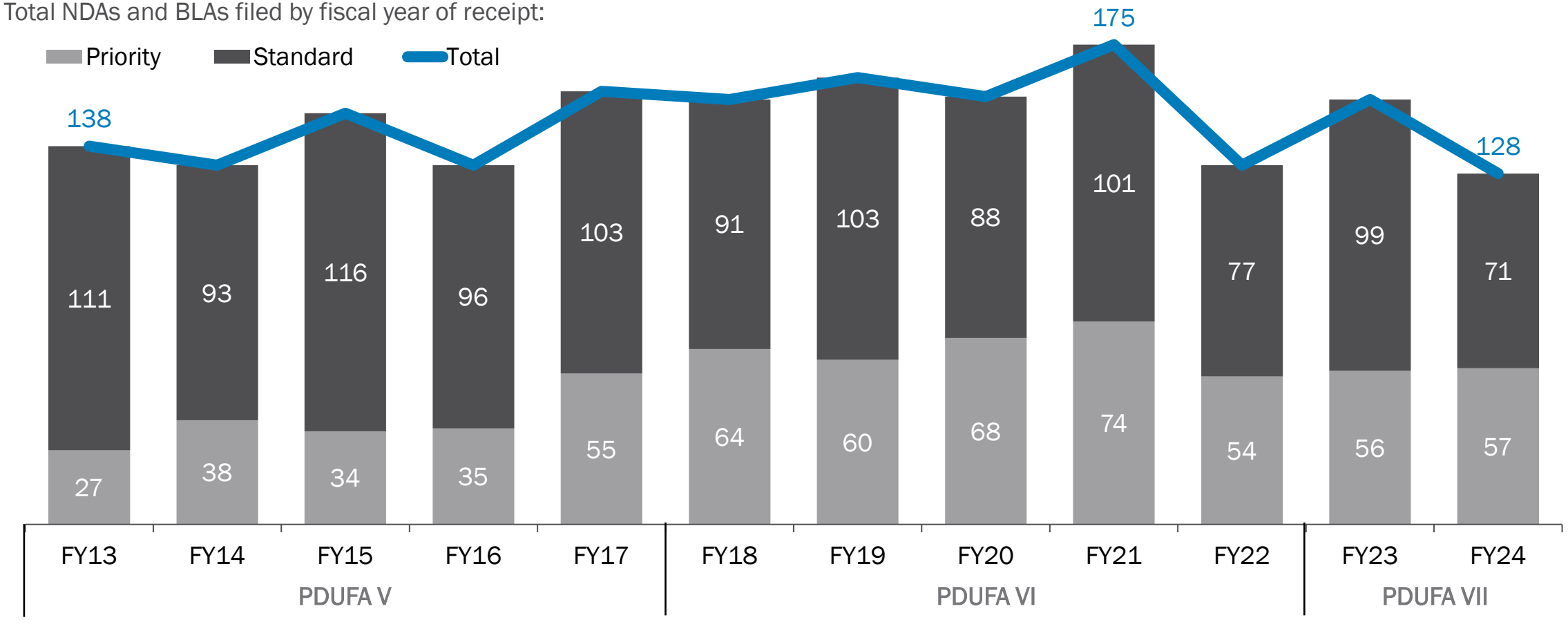
https://www.fda.gov/about-fda/fda-track-agency-wide-program-performance/fda-track-pdufa-performance

Review Program and Performance

FDA filed 128 NDAs and BLAs in FY24.

NDA and BLA submissions increased during PDUFA VI, particularly in FY21. Submission volume was more variable in the past three years.

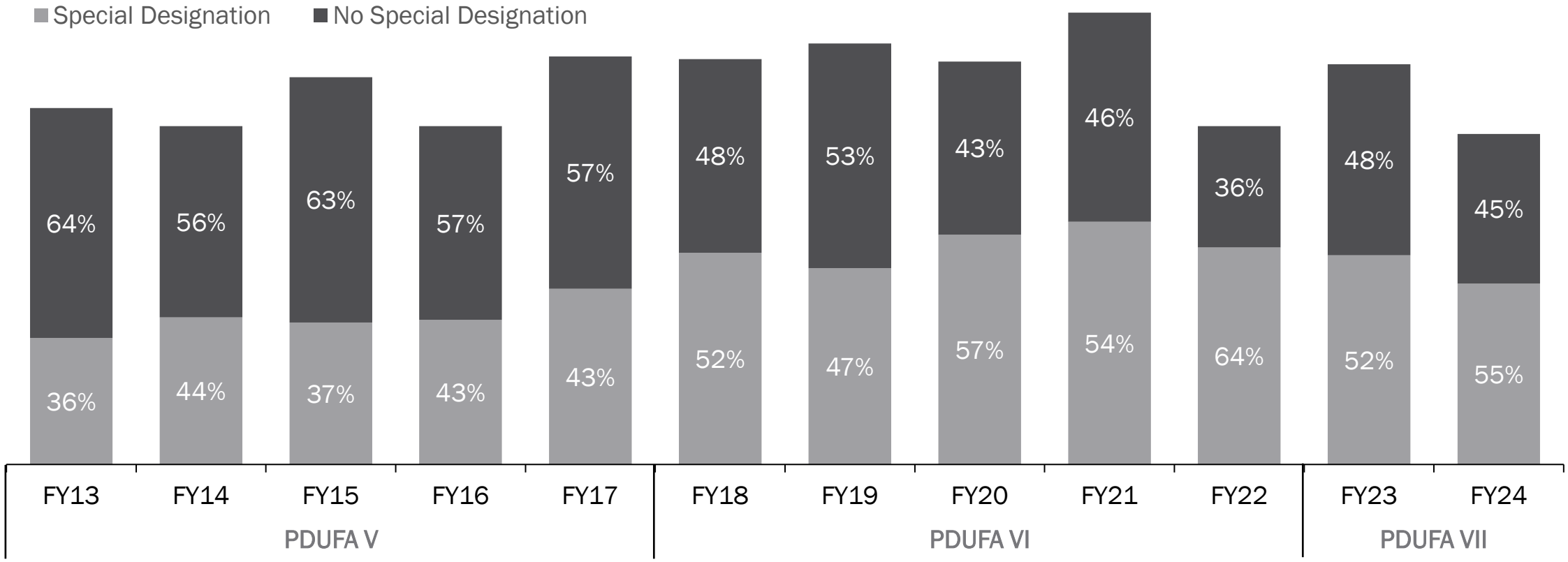
Total NDAs and BLAs filed by fiscal year of receipt:



Since 2020, over half of filed NDAs and BLAs have at least one special designation.

Special designations include Priority, Orphan, Fast Track, Breakthrough Therapy, and Accelerated Approval.

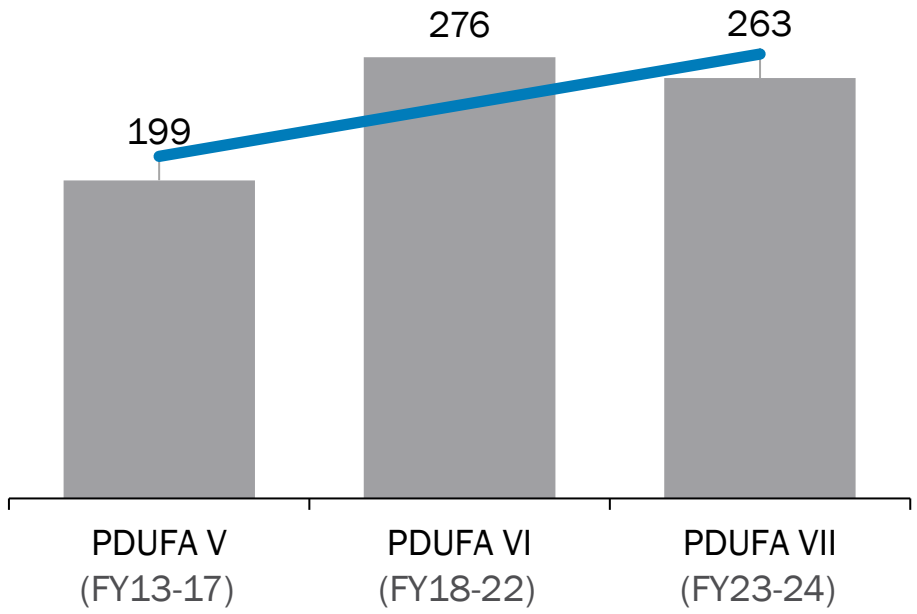
Total NDAs and BLAs filed by fiscal year of receipt:



FDA files over 3,300 efficacy and manufacturing supplement submissions per year under PDUFA VII.

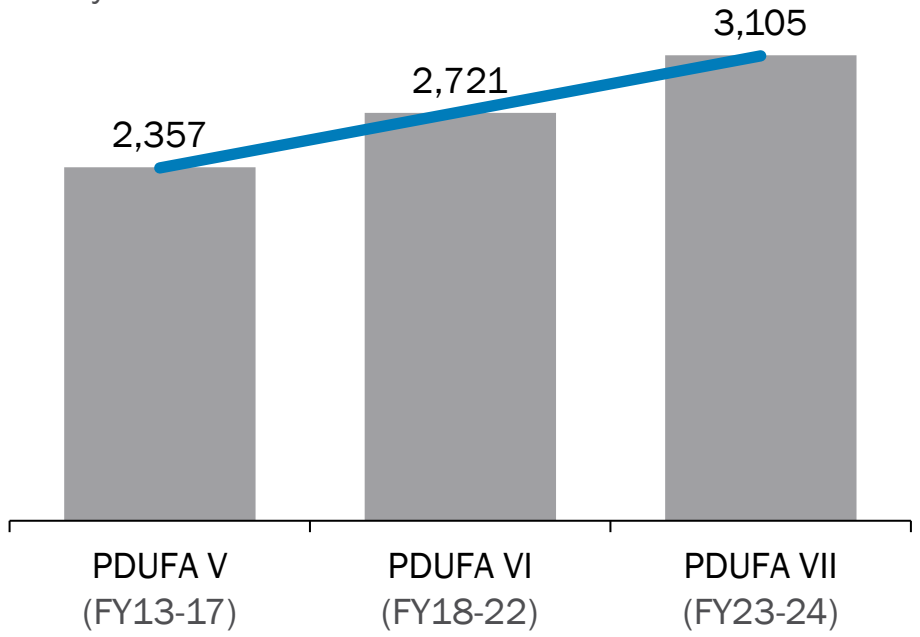
Efficacy supplement filings have **increased by 32%** since PDUFA V.

Average number of efficacy supplements filed per fiscal year:



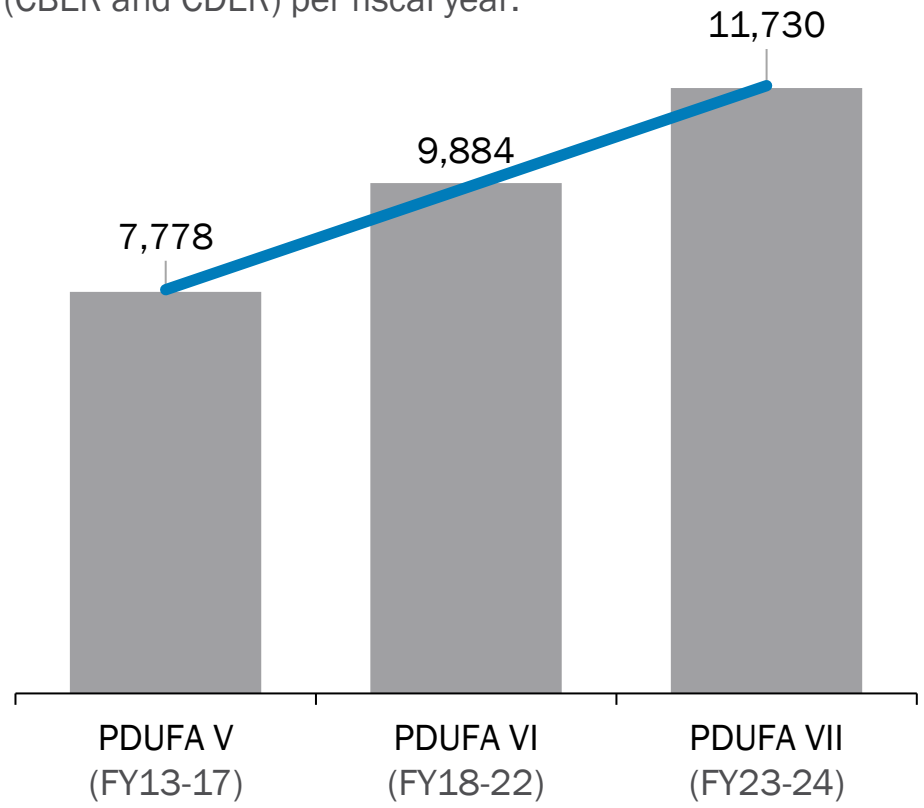
Manufacturing supplement filings have **increased by 32%** since PDUFA V.

Average number of manufacturing supplements filed per fiscal year:



Active commercial INDs average nearly 12,000 per year under PDUFA VII.

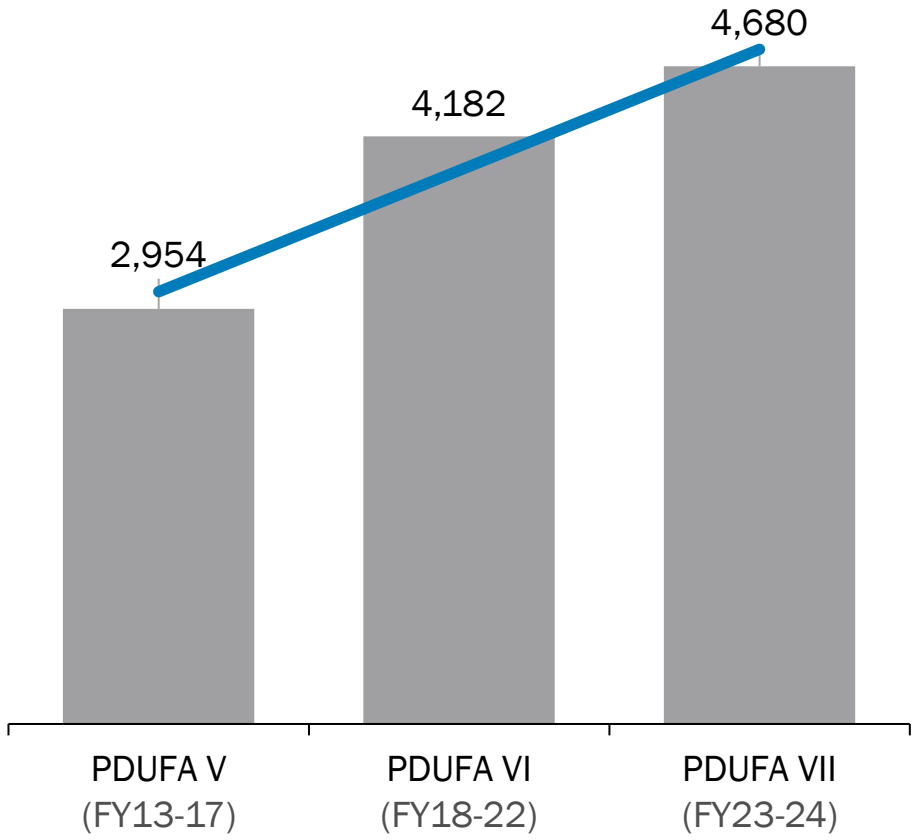
Average number of active commercial INDs (CBER and CDER) per fiscal year:



The average number of active commercial INDs per fiscal year has **increased by 51%** since PDUFA V.

FDA manages over 4,500 meeting requests per year.

Average number of meeting requests per fiscal year (by UFA period):



The average number of meeting requests received per fiscal year has **increased by 58%** in the since PDUFA V.

PDUFA VII includes 42 goals for timely review of these submissions.

Example review categories and associated goals:

EXAMPLE	GOAL
NMEs & Original BLAs	90% of priority applications within 8 months (6 months of filing date) 90% of standard applications within 12 months (10 months of filing date)
Original non-NME NDAs and Original Efficacy Supplements	90% of priority applications within 6 months 90% of standard applications within 10 months
NDA/BLA Efficacy Supplement Resubmissions	90% of Class 1 resubmissions within 2 months 90% of Class 2 resubmissions within 6 months
Manufacturing Supplements	90% of prior approval supplements within 4 months 90% of non-prior approval supplements within 6 months
Special Protocol Assessments (SPA)	90% of SPAs within 45 days of receipt
Clinical Hold Response	90% of clinical hold responses within 30 days of receipt
Meeting Scheduling	90% of Type A/B/C meetings within 30/60/75 days of receiving request

Historically, FDA has met most review goals.

	Performance Against 90% Goal By Fiscal Year						
Review Goal (90% on time)	*FY2018*	*FY2019*	*FY2020*	*FY2021*	*FY2022*	*FY2023*	*FY2024**
Original Priority NMEs and BLAs	100%	100%	92%	98%	100%	92%	100%
Original Standard NMEs and BLAs	90%	100%	85%	92%	100%	100%	100%
Original Priority Non-NME NDAs	94%	100%	100%	91%	91%	89%	100%
Original Standard Non-NME NDAs	97%	99%	95%	93%	98%	96%	100%
Class 1 Resubmitted NDAs and BLAs	89%	88%	100%	80%	100%	93%	96%
Class 2 Resubmitted NDAs and BLAs	96%	90%	89%	94%	97%		
Priority NDA and BLA Efficacy Supplements	99%	96%	99%	90%	92%	96%	100%
Standard NDA and BLA Efficacy Supplements	97%	91%	93%	93%	95%	96%	95%
Class 1 Resubmitted NDA and BLA Efficacy Supplements	100%	100%	100%	100%	100%	83%	100%
Class 2 Resubmitted NDA and BLA Efficacy Supplements	91%	100%	95%	100%	100%		
NDA and BLA Manufacturing Supplements Requiring Prior Approval	97%	98%	97%	96%	96%	96%	96%
NDA and BLA Manufacturing Supplements Not Requiring Prior Approval	99%	99%	98%	96%	99%	97%	99%

** This column is current performance as of 9/30/24 and does not include pending FY 2024 submissions/actions at that time.*

Meeting management continues to be a challenge.

	Performance Against 90% Goal By Fiscal Year						
Meeting Management Goal (90% on time)	*FY2018*	*FY2019*	*FY2020*	*FY2021*	*FY2022*	*FY2023*	*FY2024**
Type A Meeting Response to Requests	93%	90%	91%	93%	92%	94%	89%
Type B Meeting Response to Requests	90%	91%	92%	90%	91%	92%	96%
Type B(EOP) Meeting Response to Requests	80%	82%	83%	85%	88%	87%	91%
Type C Meeting Response to Requests	92%	89%	89%	91%	90%	94%	95%
Type A Meetings Scheduled	75%	74%	76%	80%	73%	74%	76%
Type B Meetings Scheduled	63%	65%	75%	80%	80%	80%	78%
Type B(EOP) Meetings Scheduled	74%	76%	80%	81%	85%	80%	82%
Type C Meetings Scheduled	75%	76%	78%	83%	84%	84%	86%
Type A Written Response	67%	67%	77%	82%	84%	83%	56%
Type B Written Response	77%	81%	82%	70%	65%	75%	88%
Type B(EOP) Written Response	57%	73%	83%	53%	76%	90%	100%
Type C Written Response	84%	81%	79%	75%	76%	84%	89%
Type B(EOP) Preliminary Response	85%	87%	82%	83%	90%	91%	90%
Meeting Minutes for All Meeting Types	91%	92%	93%	93%	94%	93%	94%

** This column is current performance as of 9/30/24 and does not include pending FY 2024 submissions/actions at that time.*

Meeting management continues to be a challenge.

	Performance Against 90% Goal By Fiscal Year*						
Meeting Management Goal (90% on time)	*FY2018*	*FY2019*	*FY2020*	*FY2021*	*FY2022*	*FY2023*	*FY2024***
Type D Meeting Response to Requests	--	--	--	--	--	88%	93%
INTERACT Meeting Response to Requests	--	--	--	--	--	95%	90%
Type D Meetings Scheduled	--	--	--	--	--	84%	85%
INTERACT Meetings Scheduled	--	--	--	--	--	93%	96%
Type D Written Response	--	--	--	--	--	87%	91%
INTERACT Written Response	--	--	--	--	--	94%	96%
Type D Preliminary Response	--	--	--	--	--	88%	93%
INTERACT Preliminary Response	--	--	--	--	--	86%	90%

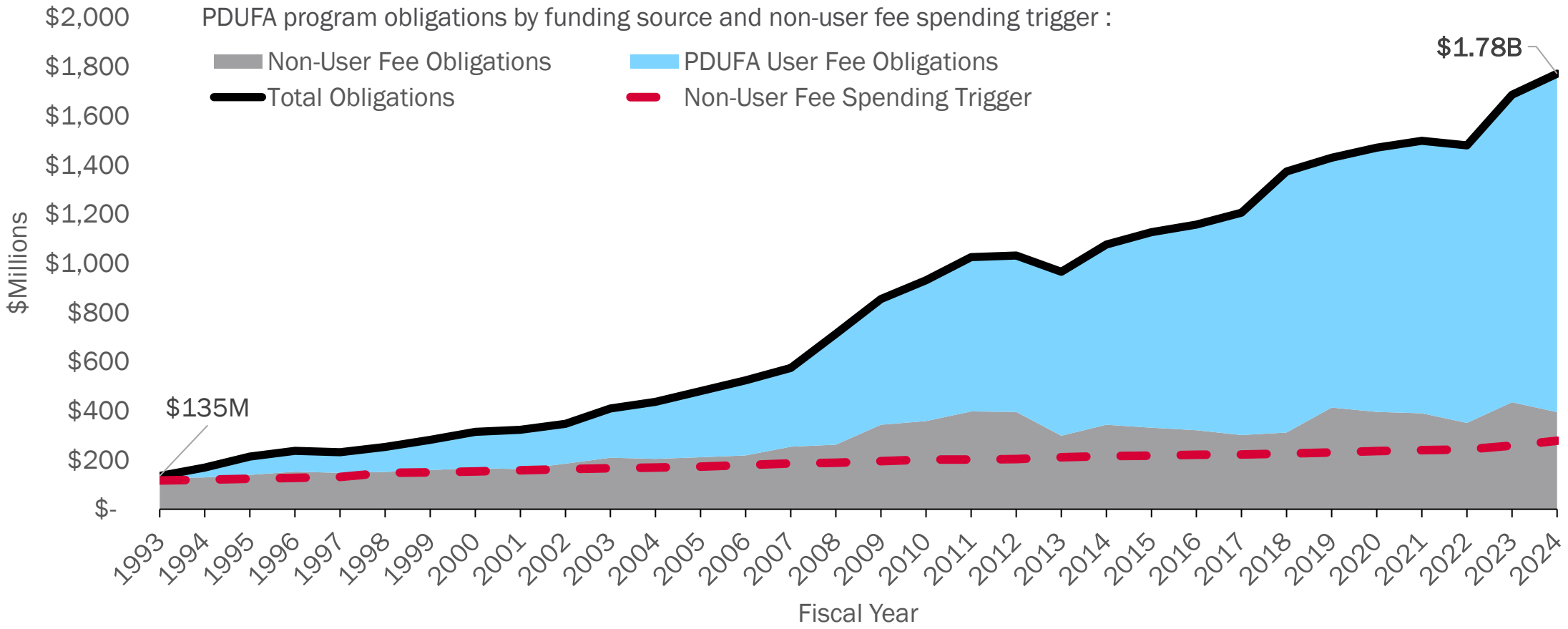
**The performance goal is to meet the specified timeline for 90% of applications, with the exception of meetings scheduled and written responses for Type D and INTERACT, for which the FY2023 goal is 50% and the FY2024 goal is 60%.*

*** This column is current performance as of 9/30/24 and does not include pending FY 2024 submissions/actions at that time.*

Financial Background and Fee Structure

User fee revenue is critical to the program.

In 1993, PDUFA user fees funded 7% of the program. In 2024, PDUFA user fees funded 78% of the program.



More information about the Non-User Fee Spending Trigger can be found in the PDUFA Financial Reports: https://www.fda.gov/about-fda/user-fee-financial-reports/pdufa-financial-reports

The current fee structure includes program and application fees.

- PDUFA VI modernized the user fee structure to improve program funding predictability, stability, and administrative efficiency.
- The current fee structure derives 20% of the target revenue from application fees and 80% from annual program fees. The PDUFA program fee is a more predictable and stable funding source compared to the application fee.
- FY 2025 target revenue is $1,478,740,000.
  - 20% collected from applications ($295,748,000 collected from ~69 fee paying full application equivalents)
  - 80% collected from the PDUFA programs ($1,182,992,000 collected from 2,929 program fees)

Fee Type	FY2025 Fee Amount
Applications with clinical data	$4,310,002
Applications without clinical data	$2,155,001
PDUFA program fee	$403,889

FDA offers fee waivers for special circumstances.

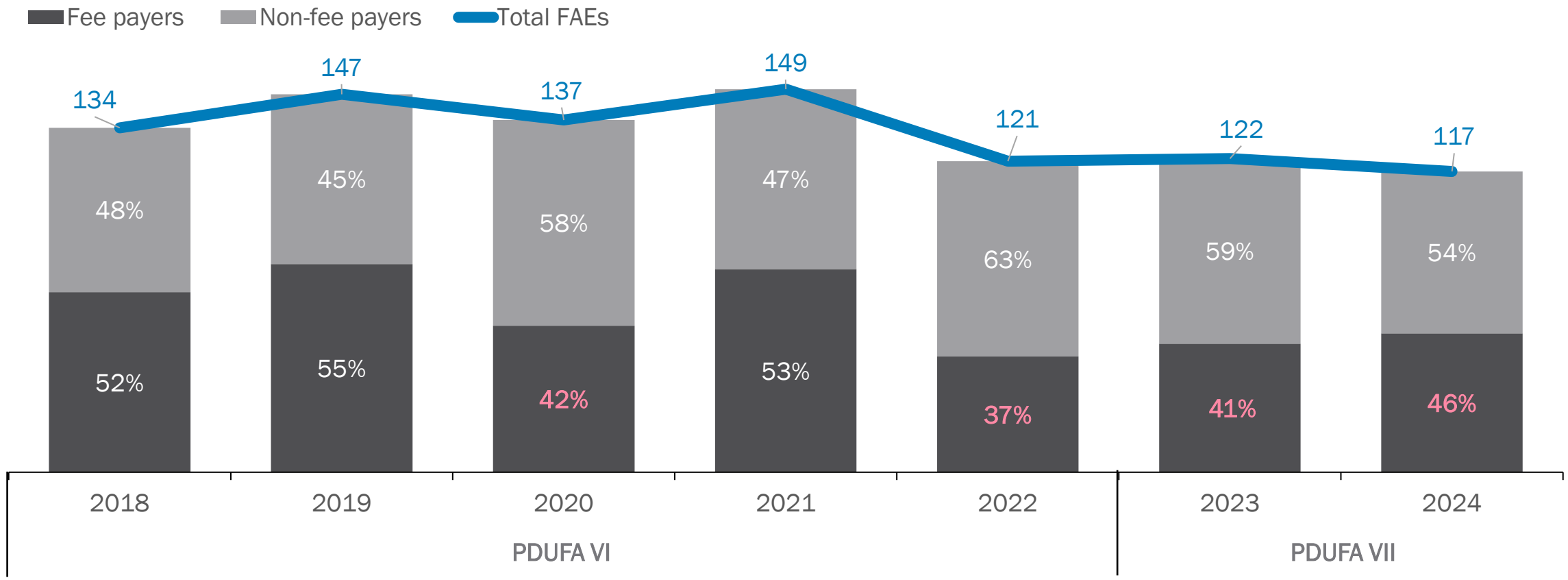
Types of waivers or qualifications for reductions in fees:

Type	Qualifies if…
Public Health	The product protects public health, and the waiver is necessary to protect public health
Barrier to Innovation	Fees would present a significant barrier to innovation because of limited resources available to the applicant or other circumstances
Small Business	The applicant is a small business submitting its first human drug application to the Agency for review and does not have another product approved
Orphan Designation	The product has been designated as a drug for a rare disease or condition
State or Federal Government Entity	The application is submitted by a State or Federal government entity for a drug that is not distributed commercially
No Substantial Work	No substantial work was performed on the application

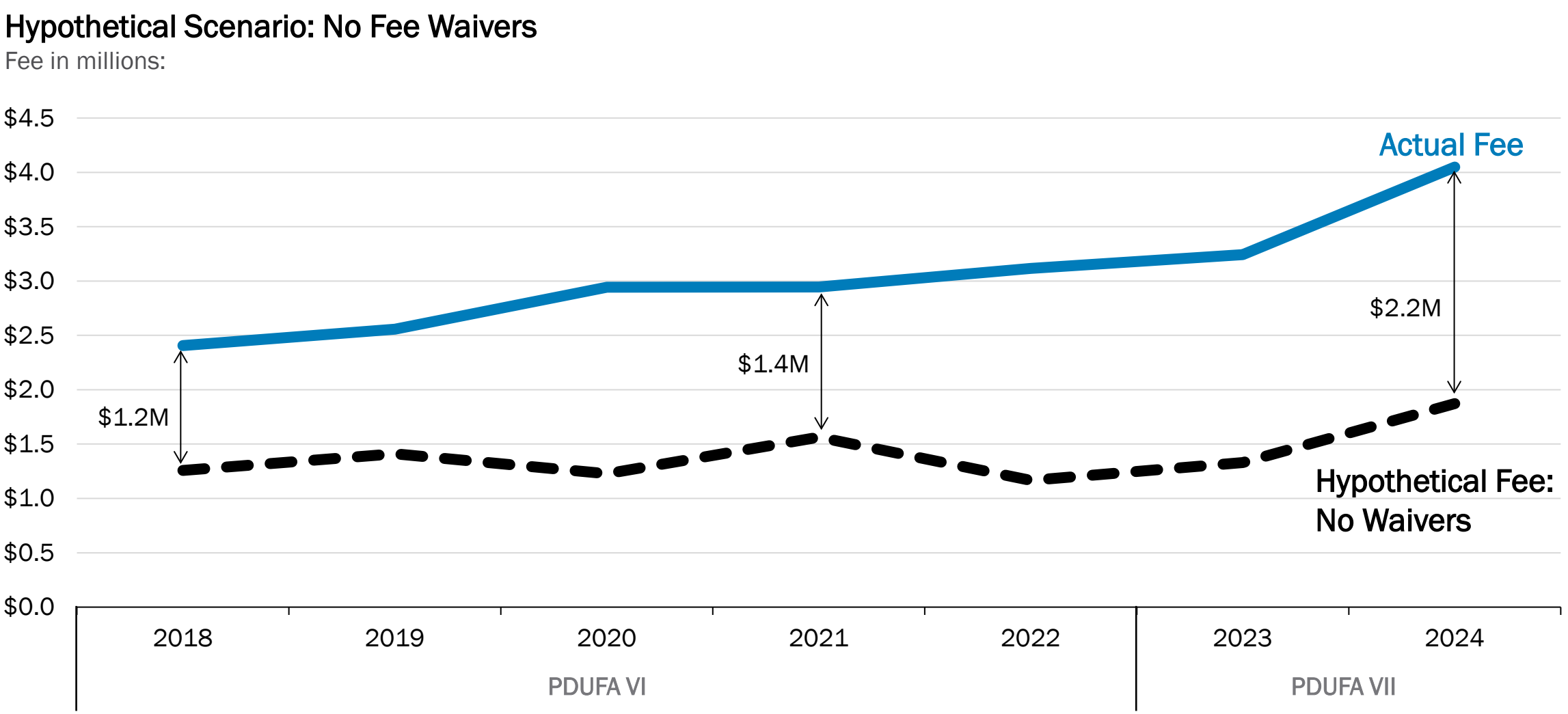
See "Prescription Drug User Fee Act Waivers, Reductions, and Refunds for Drug and Biological Products: Guidance for Industry" for more details.

The number of full application equivalents (FAEs) is dropping, as is the proportion that pays fees.

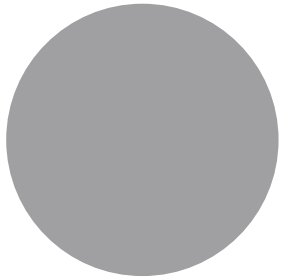
Percent of full application equivalents and total FAEs:



The amount of fee payers affects the fee rate.

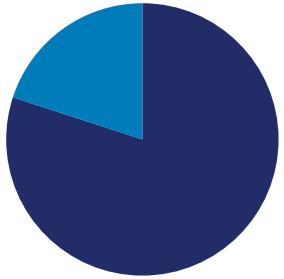


Fewer fee payers means those who do pay, pay more.

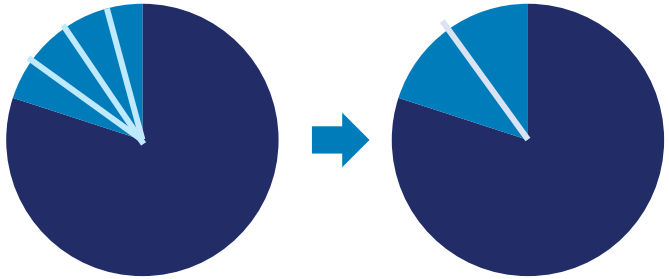


The revenue-setting process establishes the target revenue amount.

Annual Target Revenue = Base Revenue + Adjustments



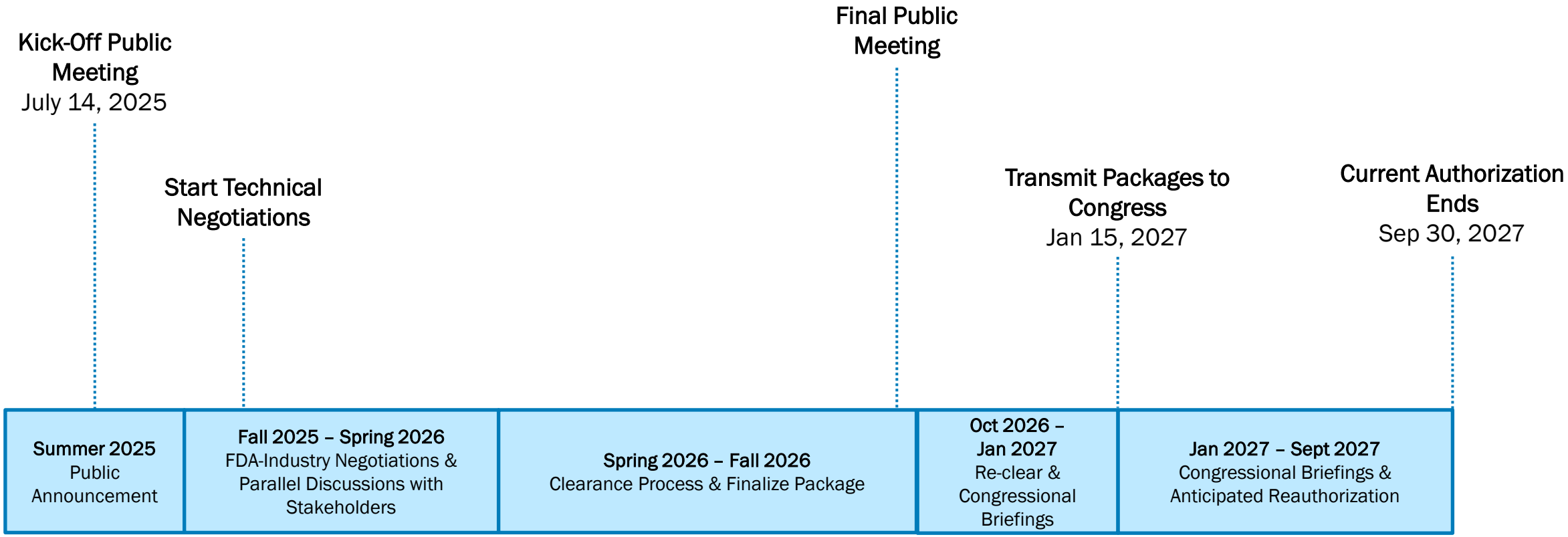
The target revenue is split: 20% from application fees and 80% from program fees.



The 20% from application fees is then divided by the estimated number of fee-paying full application equivalents.
The fewer fee-paying equivalents, the larger each of their slices of pie.

Reauthorization Process Overview

Overview of the Reauthorization Process



PDUFA reauthorization involves significant consultation with stakeholders.

PDUFA REAUTHORIZATION and REPORTING REQUIREMENTS.

(f) REAUTHORIZATION.—

***(1) CONSULTATION.**—In developing recommendations to present to the Congress with respect to the goals, and plans for meeting the goals, for the process for the review of human drug applications for the first 5 fiscal years after fiscal year 2027, and for the reauthorization of this subpart for such fiscal years, the Secretary shall consult with— (A) the Committee on Energy and Commerce of the House of Representatives; (B) the Committee on Health, Education, Labor, and Pensions of the Senate; (C) scientific and academic experts; (D) health care professionals; (E) representatives of patient and consumer advocacy groups; and (F) the regulated industry.*

***(2) PRIOR PUBLIC INPUT.**—Prior to beginning negotiations with the regulated industry on the reauthorization of this part, the Secretary shall—*

(A) publish a notice in the Federal Register requesting public input on the reauthorization; (B) hold a public meeting at which the public may present its views on the reauthorization, including specific suggestions for changes to the goals referred to in subsection (a); (C) provide a period of 30 days after the public meeting to obtain written comments from the public suggesting changes to this part; and (D) publish the comments on the Food and Drug Administration's Internet Web site.

***(3) PERIODIC CONSULTATION.**—Not less frequently than once every month during negotiations with the regulated industry, the Secretary shall hold discussions with representatives of patient and consumer advocacy groups to continue discussions of their views on the reauthorization and their suggestions for changes to this part as expressed under paragraph (2).*

***(4) UPDATES TO CONGRESS. —**The Secretary, in consultation with regulated industry, shall provide regular updates on negotiations on the reauthorization of this subpart to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives.*

(4)PUBLIC REVIEW OF RECOMMENDATIONS.*—After negotiations with the regulated industry, the Secretary shall— (A) present the recommendations developed under paragraph (1) to the Congressional committees specified in such paragraph; (B) publish such recommendations in the Federal Register; (C) provide for a period of 30 days for the public to provide written comments on such recommendations; (D) hold a meeting at which the public may present its views on such recommendations; and (E) after consideration of such public views and comments, revise such recommendations as necessary.*

***(5) TRANSMITTAL OF RECOMMENDATIONS.—**Not later than January 15, 2027, the Secretary shall transmit to the Congress the revised recommendations under paragraph (4), a summary of the views and comments received under such paragraph, and any changes made to the recommendations in response to such views and comments.*

(6) MINUTES OF NEGOTIATION MEETINGS.—

***(A) PUBLIC AVAILABILITY.—**The Secretary shall make publicly available, on the public Web site of the Food and Drug Administration, minutes of all negotiation meetings conducted under this subsection between the Food and Drug Administration and the regulated industry, not later than 30 days after each such negotiation meeting..*

***(B) CONTENT.**—The minutes described under subparagraph (A) shall summarize, in sufficient detail, any substantive proposal made by any party to the negotiations as well as significant controversies or differences of opinion during the negotiations and their resolution.*

Today's meeting begins the stakeholder consultation process.

Initial Public Meeting & Public Comment

Today – Public comment period closes August 14, 2025

> ***(2) PRIOR PUBLIC INPUT.***—*Prior to beginning negotiations with the regulated industry on the reauthorization of this part, the Secretary shall—*
>
> *(A) publish a notice in the Federal Register requesting public input on the reauthorization; (B) hold a public meeting at which the public may present its views on the reauthorization, including specific suggestions for changes to the goals referred to in subsection (a); (C) provide a period of 30 days after the public meeting to obtain written comments from the public suggesting changes to this part; and (D) publish the comments on the Food and Drug Administration's Internet Web site.*

Public Stakeholder Meetings

Monthly during negotiations – sign-up by August 4, 2025

> ***(3) PERIODIC CONSULTATION.***—*Not less frequently than once every month during negotiations with the regulated industry, the Secretary shall hold discussions with representatives of patient and consumer advocacy groups to continue discussions of their views on the reauthorization and their suggestions for changes to this part as expressed under paragraph (2).*

Public Minutes from FDA-Industry Negotiation Meetings

Posted within 30 days of each meeting

> *(**A) PUBLIC AVAILABILITY.***—*The Secretary shall make publicly available, on the public Web site of the Food and Drug Administration, minutes of all negotiation meetings conducted under this subsection between the Food and Drug Administration and the regulated industry, not later than 30 days after each such negotiation meeting..*

Industry Comments

Annetta Beauregard

Biotechnology Innovation Organization

Senior Vice President, Science & Regulatory Affairs

Biotechnology Innovation Organization

Reauthorization of the Prescription Drug User Fee Act

Public Meeting

Who is BIO?

BIO is the premier biotechnology advocacy organization representing biotech companies, industry leaders, and state biotech associations in the United States and more than 35 countries around the globe.

BIO members range from biotech start-ups to some of the world's largest biopharmaceutical companies – all united by the same goal: to develop medical and scientific breakthroughs that prevent and fight disease, restore health, and improve patients' lives.

BIO also organizes the BIO International Convention and a series of annual conferences that drive partnerships, investment, and progress within the sector.

Founded	1993
Tax Status	Non-profit trade association
CEO	John F. Crowley
Board Chair	Fritz Bittenbender (Genentech)
Headquarters	Washington, DC
Members	~1,000

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

WHY

- US patients are often the first in the world to receive innovative therapies as a result of PDUFA enabling FDA resources and efficient regulatory processes.

WHO

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

HOW

- Authorizing the FDA to collect user fees from the biopharmaceutical industry
- Establishing certain performance goals for FDA related to the drug development process, application review, and drug safety
  - For example, standard new molecular entity new drug applications now have a goal for review completion within 12 months

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

PDUFA is an Important Tool with Defined Parameters

- The PDUFA program ensures adequate resources for FDA for the review of new drugs, enabling necessary sponsor/FDA engagement during drug development.
  - **For small biotech companies, efficient & predictable review can make a critical difference in innovation reaching patients**

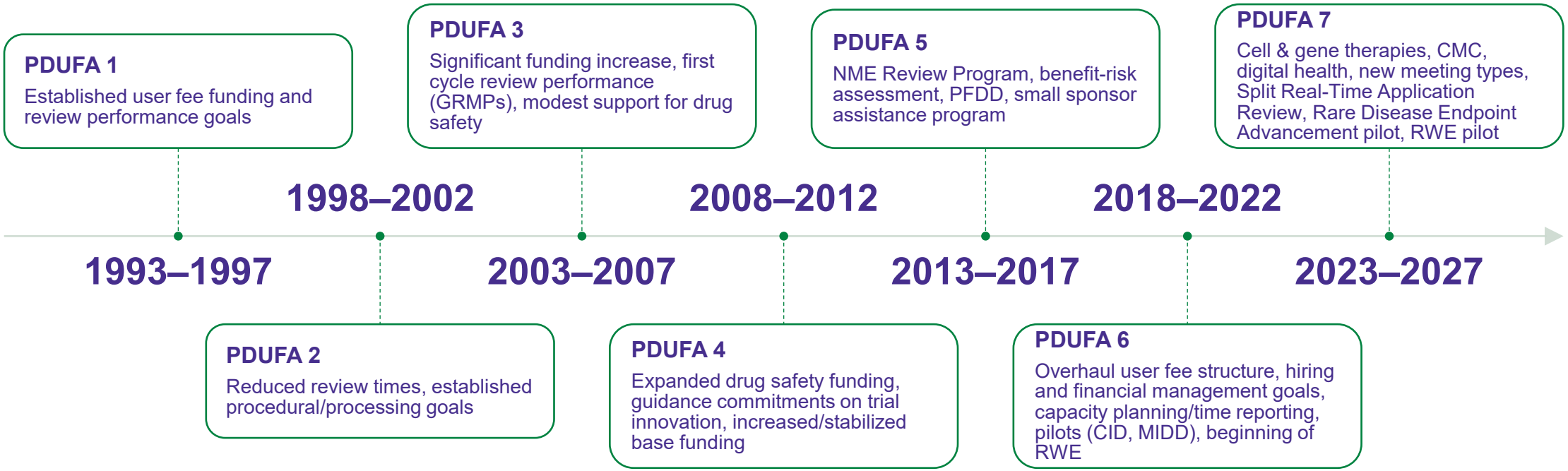
- The PDUFA program is limited to allocating resources/improving FDA performance in regulatory review and associated staffing.
  - The program cannot be used to alter regulatory policy or FDA statutory requirements.
  - As part of PDUFA, industry receives from FDA a commitment to meet certain industry-wide performance goals. Goals are not tied to a specific application under review.

- PDUFA fees are NEVER tied to review outcomes

- FDA maintains 100% scientific independence as a cornerstone principle of PDUFA.

- PDUFA is reauthorized 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. The ultimate goal is to adapt FDA's processes to the latest regulatory science to ensure safe and effective medicines reach patients.

FDA's commitments are additive and have expanded over 30+ years of PDUFA



BIO’s Approach to PDUFA VIII

- BIO recognizes and appreciates Commissioner Makary's commitment to upholding the gold standard of trusted science, increasing transparency, and applying common-sense decision-making.
- BIO looks forward to working collaboratively with FDA, HHS, and other executive and legislative branch stakeholders to ensure a timely reauthorization of this important program.

BIO's Objectives for PDUFA VIII

- Prioritize core review activities to ensure safe and effective biotech innovations reach patients faster.
- Ensure FDA meets its review activity performance commitments
- Increase predictability of the PDUFA review process and financial structure
- Improve FDA communications, transparency, and accountability

Improving FDA Communications and Transparency

- Improving FDA's communications will enable safe and effective drugs to reach patients sooner by adding timely clarity to the regulatory requirements for innovative therapies.
- Additionally, enhancements to how FDA communicates the basis for its decisions will enable greater insights into how America's biotechnology leaders are using the latest science to improve public health.
- These insights will compound into additional benefits for patients and American innovation.

Improving FDA Accountability and Predictability

PDUFA provides a tremendous tool for ensuring that government regulators are accountable for their performance.

- Increasing the accountability for process milestones will add certainty to the drug development ecosystem and spur additional investment in bringing innovative safe and effective therapeutics to patients.
- Having a predictable regulatory environment, including a predictable fee and revenue approach for PDUFA, will enhance biotechnology companies' ability to plan for, and invest in, innovation.
- Industry depends on a stable, well-resourced FDA, staffed by subject matter experts, to conduct the regulatory oversight required to bring safe and effective therapeutics to patients.

Thank you

- BIO appreciates the opportunity to comment on the importance of PDUFA to bringing safe and effective innovations to patients and looks forward to the PDUFA VIII reauthorization process.

Kristy Lupejkis

Pharmaceutical Research and Manufacturers of America

Vice President and Chief of Staff, Science and Regulatory Advocacy

PhRMA
RESEARCH • PROGRESS • HOPE

Public Meeting on Prescription Drug User Fee Act (PDUFA) Reauthorization

July 14, 2025

The Pharmaceutical Research and Manufacturers of America (PhRMA)

- PhRMA represents the country's leading innovative biopharmaceutical research companies, which are focused on developing innovative medicines that transform lives and create a healthier world.
- We believe in the value of biopharmaceutical research and development and the importance of a robust innovation ecosystem supporting scientific advances to the benefit of patients and society.
- For decades, our member companies have pushed the boundaries of science, investing in new technology, research, treatments and vaccines that help advance medicines for patients everywhere.
- These innovators are committed to continuing investment in discovery and research for treatments and vaccines for the many health problems and diseases that threaten our world's wellbeing.

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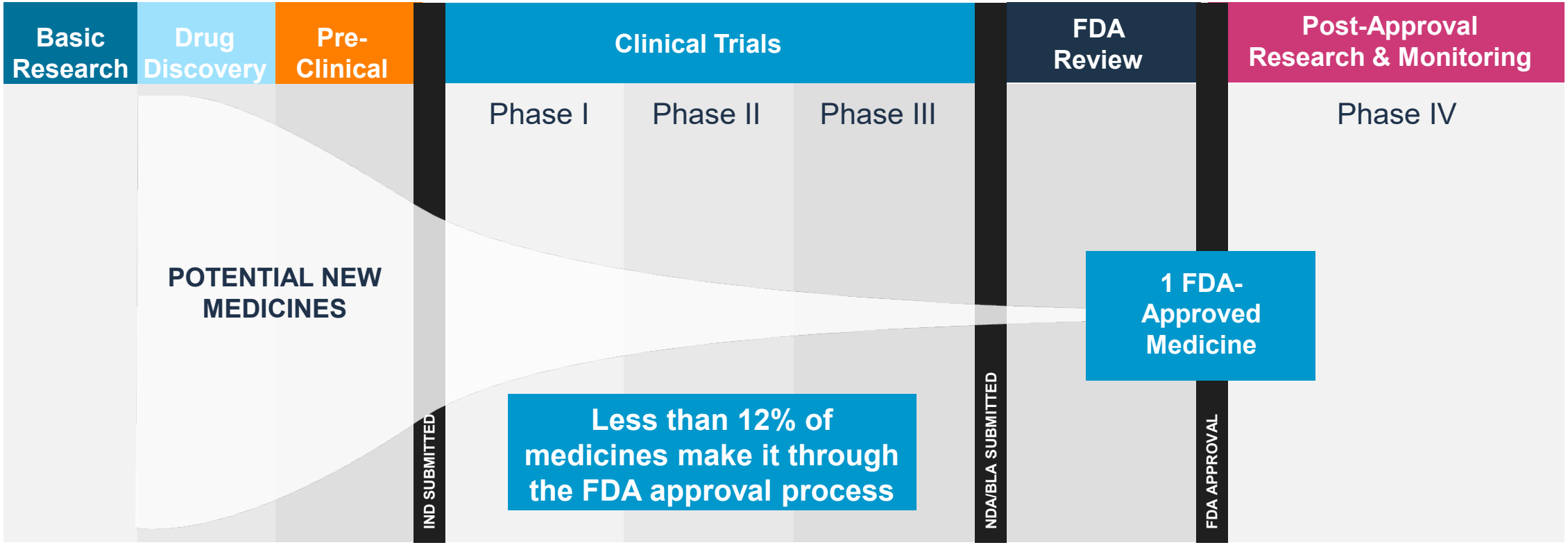
The R&D Process is Lengthy, Costly and Highly Uncertain

Developing a new medicine takes 10 to 15 years and costs an average of $2.6 billion.
Over the last decade, PhRMA member companies have invested more than $800 billion in the search for new treatments and cures.



Key: IND= Investigational New Drug Application, NDA= New Drug Application, BLA= Biologics License Application

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Sources: PhRMA adaptation of DiMasi J. et al; PhRMA member survey.

PDUFA Supports More Timely Patient Access to Safe and Effective New Medicines

America's predictable regulatory environment helps patients have more medicine choices than people living anywhere else in the world

Before PDUFA enactment in 1992, more than 70% of medicines were first approved outside of the United States. Since the first PDUFA, the majority of new medicines are now approved first inside the U.S. including close to 70% of new medicines in 2024 alone[1]

PDUFA has helped **enable timely access to more than 1,000 novel new drugs and biologics**, including treatments for conditions like cancer, cardiovascular, neurological and additional rare diseases[2]

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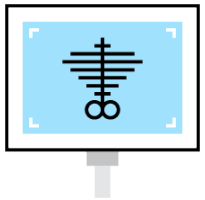
[1] FDA, Novel Drug Approvals 2024 &FDA 2024 Biological License Application Approvals https://www.fda.gov/media/184967/download?attachment; https://www.fda.gov/vaccines-blood-biologics/development-approval-process-cber/2024-biological-license-application-approvals 2 Summary of NDA Approvals & Receipts, 1938 to the present, https://www.fda.gov/about-fda/histories-fda-regulated-products/summary-nda-approvals-receipts-1938-present

PDUFA Helps Ensure FDA is the Global Gold Standard for Regulatory Review



- PDUFA enhances FDA review processes **to keep pace with the number and complexity of innovative medicines entering the review pipeline**

- PDUFA includes **review timelines for new drug and biologic applications**, providing patients and sponsors with greater predictability

- PDUFA supports advancing **regulatory science and strengthening scientific dialogue** between sponsors and the FDA

- PDUFA enables timely and consistent communication between FDA and sponsors during the regulatory review of new medicines, **helping reduce unnecessary delays in patient access to innovative treatments**

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PDUFA VIII Can Support FDA's Vital Role in Medical Product Review and Protecting the Public Health

PDUFA VIII can build on previous PDUFA agreements to help ensure U.S. drug development and review processes are consistent, predictable, transparent, and rooted in sound scientific principles.

- Independent and Science-Based
- Predictable
- Transparent and Credible
- Efficient
- Pro-innovation
- Patient-centric and Engaged
- Accountable

PDUFA VIII Can Help Provide Greater Efficiency, Transparency, and Accountability in the Human Drug Review Program

- PDUFA VIII should support first cycle review of new applications and related performance goals to facilitate timely availability of safe and effective new medicines for patients.
- PDUFA VIII can help FDA optimize staffing and resources by supporting new efficiencies in the human drug review program and streamlining regulatory review processes.
- Prior PDUFA regulatory science initiatives and pilot programs can be incorporated into standard FDA regulatory review practice.

Conclusion

- Ensuring the review of innovative medicines is properly resourced through a combination of Congressional appropriations and industry user fees will help support the timely availability of the next generation of safe and effective new medicines.

- PhRMA looks forward to working with FDA, patient groups and other stakeholders to advance a PDUFA VIII agreement that strengthens America's world-leading innovation ecosystem to address our most pressing health challenges.

U.S. FOOD & DRUG ADMINISTRATION

BREAK

Check the Q&A for our restart time.

Submit your public comments by August 14!

You can access the docket via:

- FDA's public meeting webpage

  (https://www.fda.gov/industry/public-meeting-reauthorization-prescription-drug-user-fee-act-pdufa-07142025)

- Directly through the Federal Register

  (https://www.federalregister.gov/documents/2025/05/19/2025-08824/reauthorization-of-the-prescription-drug-user-fee-act-public-meeting-request-for-comments)

Public Comment

Juliana Reed

Biosimilars Forum

Kaylin Bower

On A MiSsion for Multiple Sclerosis

Irene Ulrich

Center for Science in the Public Interest (CSPI)

Patricia Kelmar

U.S. Public Interest Research Group (PIRG)

Amanda Berhaupt

National Center for Health Research

Mary Hilley

Humane World for Animals

Annie Kennedy

EveryLife Foundation for Rare Diseases

Pamela Gavin

National Organization for Rare Disorders (NORD)

Keith Desserich

The Cure Starts Now Foundation & the Pediatric Brain Tumor Consortium Foundation

Alexander Naum

Generation Patient

Amalia Guinee

NYU Langone MS Center

Janet Krommes

Doctors for America

Dathan Hamann

Saguaro Dermatology and Contact Dermatitis Institute

Zoe Bilis

Biocom California

Curt Hamann

SmartPractice

Marcia Howard

Consumer Healthcare Products Association (CHPA)

Gil Roth

Pharma & Biopharma Outsourcing Association (PBOA)

Reshma Ramachandran

Yale Collaboration for Regulatory Rigor, Integrity, and Transparency (CRRIT) & Yale School of Medicine

Emily Anderson

Physicians Committee for Responsible Medicine

Closing Remarks

U.S. FOOD & DRUG ADMINISTRATION

THANK YOU