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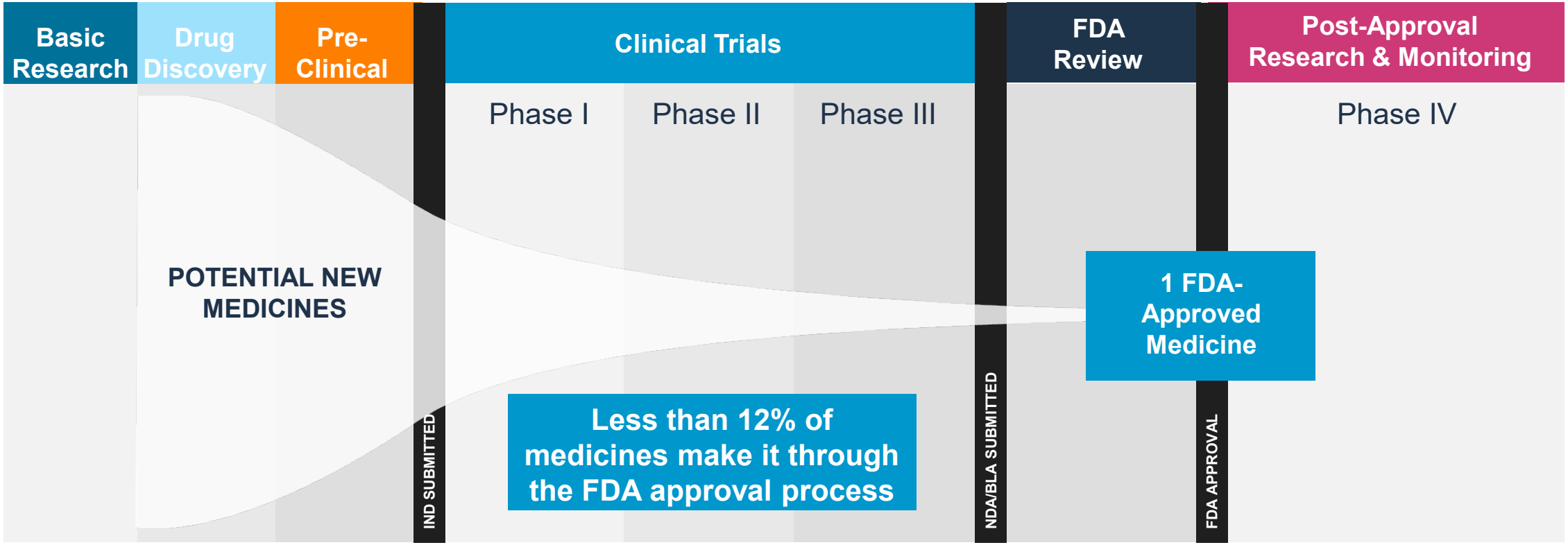
The R&D Process is Lengthy, Costly and Highly Uncertain

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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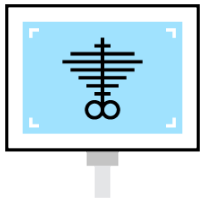
[1] FDA, Novel Drug Approvals 2024 &FDA 2024 Biological License Application Approvals https://www.fda.gov/media/184967/download?attachment; https://www.fda.gov/vaccines-blood-biologics/development-approval-process-cber/2024-biological-license-application-approvals 2 Summary of NDA Approvals & Receipts, 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.