670 Maine Avenue, SW • Suite 1000, Washington, DC 20024 • PHRMA.ORG

August 14, 2025

Dockets Management Staff (HFA–305)
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Docket No. FDA-2025-N-0816: Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments[1]

To Whom it May Concern:

The Pharmaceutical Research and Manufacturers of America (PhRMA) is pleased to submit these comments in response to the Food and Drug Administration's (FDA or the Agency) Public Meeting on the Reauthorization of the Prescription Drug User Fee Act (PDUFA).[2] PhRMA thanks the Agency for holding this public meeting to facilitate stakeholder engagement and public comment on PDUFA reauthorization.

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. Together, we are fighting for solutions to ensure patients can access and afford medicines that prevent, treat and cure disease. PhRMA member companies have invested more than $850 billion in the search for new treatments and cures over the last decade, supporting nearly five million jobs in the United States.

I. GENERAL COMMENTS

Since its inception in 1992, PhRMA has been a supporter of, and participant in, the PDUFA program, and we appreciate FDA's commitment to engaging stakeholders on the reauthorization of PDUFA for fiscal years (FYs) 2028 through 2032.

America's biopharmaceutical companies are at the heart of a robust research and development (R&D) ecosystem that develops more innovative medicines than any other country in the world. In recent years, rapid advances in scientific discovery have ushered in a new era of medicine, transforming our ability to treat, and in some cases, cure some of the most challenging diseases – including many cancers, rare diseases, and autoimmune conditions. These advancements are due to the productivity of the United States (U.S.) biomedical R&D ecosystem, which is sustained by a policy framework that is designed to support and advance America's leadership in the innovation of new medicines.

At the same time, medicine development is a long, resource-intensive, and highly uncertain process, and the path to new treatments is rarely straightforward. Fewer than 12% of drug candidates that make it to Phase I clinical trials are eventually approved by the FDA and on average it takes 10-15 years and $2.6 billion to bring a new medicine to market.[3] Additionally,

[1] 90 Fed. Reg. 21315 (May 19, 2025). Available at: www.govinfo.gov/content/pkg/FR-2025-05-19/pdf/2025-08824.pdf.
[2] U.S. Food and Drug Administration (FDA), Public Meeting on the Reauthorization of the Prescription Drug User Fee Act (PDUFA), July 14, 2025. Available at: www.federalregister.gov/documents/2025/05/19/2025-08824/reauthorization-of-the-prescription-drug-user-fee-act-public-meeting-request-for-comments.
[3] Joseph A DiMasi, Henry G Grabowski, Ronald W Hansen, Innovation in the pharmaceutical industry: New estimates of R&D costs, 47 Journal of Health Economics, 20-33 (2016). Available at: https://pubmed.ncbi.nlm.nih.gov/26928437/.

FDA approval of a medicine is rarely where innovation ends, and continuous learning about approved products is always part of the process.

The scientific advancements made by America's biopharmaceutical industry and its global leadership in innovating new medicines is sustained by a robust and predictable regulatory environment. To maintain this leadership and ultimately to better serve patients, the U.S. needs a modern regulatory paradigm that can provide timely, science-based regulatory decisions.

That is why PhRMA and our member companies support a strong, appropriately staffed, and science-based FDA resourced through a combination of Congressionally appropriated funds and user fees from the regulated industry. For more than 30 years, PDUFA has helped the FDA fulfill its central mission – to help protect and promote the public health by ensuring the safety, efficacy, and security of drugs – by allowing the Agency to keep pace with the rapid increase in the number and complexity of innovative drugs and biologics entering the review pipeline.

America's predictable regulatory environment helps provide patients with more medicine choices than people living anywhere else in the world. However, before PDUFA was enacted in 1992, more than 70% of medicines were first approved outside of the United States.[4] Patients' demand for faster review times for new treatments and therapies helped push FDA, Congress, and the biopharmaceutical industry to work together to shorten review times.

PDUFA was created in recognition of this need for faster review of new drugs. Since the first PDUFA, the majority of new medicines are now approved first in the US – including close to 70% of new medicines in 2024.[5] Overall, PDUFA has helped enable timely access to more than 1,000 novel new drugs and biologics, including treatments for conditions like cancer, cardiovascular, and rare diseases.[6]

The user fee funding provided through PDUFA, when combined with sufficient Congressional appropriations, allows FDA to pursue its public health mission to ensure safe and effective medicines for patients, while helping support FDA as the global gold standard for regulatory review. By helping support efficiency, accountability, and scientific rigor in regulatory review, PDUFA has enabled FDA to keep pace with the rapid increase in number and complexity of applications, as well as the rapid scientific advances within the US biomedical enterprise.

Furthermore, by establishing review timelines for marketing applications, supporting issuance of regulatory guidance, and improving communication and engagement and scientific dialogue between FDA and the biopharmaceutical industry, PDUFA has helped enhance regulatory predictability for sponsors. Resources provided through PDUFA for the advancement of new tools and approaches to drug development – including innovative trials design, use of real-world evidence, and digital health tools[7] – have resulted in faster patient access to safe, effective, and transformative medicines.

---

[4] Joseph A DiMasi, Henry G Grabowski, Ronald W Hansen, Innovation in the pharmaceutical industry: New estimates of R&D costs, 47 Journal of Health Economics, 20-33 (2016). Available at: https://pubmed.ncbi.nlm.nih.gov/26928437/.

[5] FDA, Center for Drug Evaluation and Research, Advancing Health Through Innovation: New Drug Therapy Approvals 2024, January 2025. Available at: www.fda.gov/files/drugs/published/new-drug-therapy-2025-annual-report.pdf; Center for Biologics Evaluation and Research, 2024 Biological License Application Approvals, February 2025. Available at: https://www.fda.gov/vaccines-blood-biologics/development-approval-process-cber/2024-biological-license-application-approvals.

[6] FDA, Summary of NDA Approvals & Receipts, 1938 to the present, January 2018. Available at: www.fda.gov/about-fda/histories-fda-regulated-products/summary-nda-approvals-receipts-1938-present.

[7] *See* FDA, PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2023 through 2027. Available at: www.fda.gov/media/151712/download?attachment; PDUFA Reauthorization Performance Goals and Procedures Fiscal Years

PhRMA urges stakeholders from across the biomedical research enterprise to work together and build on the successes of previous PDUFAs to continue supporting FDA's vital role in medical product review and public health. PDUFA VIII will help sustain FDA's gold standard review and enhance a U.S. drug review process that is timely, consistent, predictable, transparent, and grounded in sound science. This includes ensuring that FDA's human drug review program continues to be:

1. **Independent and Science-Based:** FDA should serve as the global gold standard review and the U.S. authority over regulatory decision-making on safety and efficacy of medical products;

2. **Predictable:** FDA should adhere to clear, transparent, established review timelines that meet or exceed PDUFA timelines, prioritizing essential review activities to foster predictability and consistency and ensure sponsors understand regulatory expectations;

3. **Transparent and Credible:** FDA should engage directly and continually with the public and relevant stakeholders to provide proactive, timely and accessible information on regulatory processes and approvals, as well as emerging safety information to maintain the public's trust;

4. **Efficient:** FDA should facilitate the most timely, effective, and efficient reviews, and work with sponsors to help ensure supply chain security and a steady supply of medical products;

5. **Pro-Innovation:** FDA should embrace new methods in medical product development appropriately and consistently across modalities and therapeutic areas to speed up and reduce the cost of development, submission, and review, while applying regulatory flexibilities that align to broad patient needs when weighing the benefits and risks of medical products;

6. **Patient-Centric:** FDA should advance decisions that are patient-centric and evidence-based and take into consideration the full range of the most current scientific knowledge and technological progress; and

7. **Accountable:** FDA should ensure regulatory review is driven by and assessed against metrics-based outcomes, including key performance indicators, and public reporting of relevant review timelines and accountability measures.

PhRMA believes it is critical for PDUFA VIII to build on initiatives included in previous PDUFAs that are intended to help provide greater efficiency, transparency, and accountability in the human drug review program. This includes pulling through prior PDUFA commitments and translating previously funded regulatory science initiatives and pilots into practice.

PhRMA also believes that PDUFA VIII should focus on 1) facilitating first cycle review of new applications and related performance goals to expedite availability of safe and effective new medicines for patients; and 2) helping FDA optimize available staffing and resources by supporting new efficiencies in the human drug review program and streamlining regulatory review processes.

---

through 2018-2022. Available at: www.fda.gov/media/99140/download; PDUFA Reauthorization Performance Goals and Procedures Fiscal Years through 2012-2017. Available at: http://www.fda.gov/media/151712/download.

II. CONCLUSION

Medicines in the biopharmaceutical pipeline provide hope for potential future treatment options for patients who have not found success with existing therapies. The U.S. leads the world in the introduction of new medicines, thanks, in part, to the FDA's human drug review program and the supportive resources provided by both Congressional appropriations and industry user fees through PDUFA.

PhRMA appreciates the opportunity to comment on PDUFA reauthorization and thanks the FDA for holding the corresponding public meeting. We look forward to working with the 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, while supporting the timely availability of next generation safe and effective medicines.

Respectfully submitted,

_____/S/_____
Kristy Lupejkis
Vice President, Science and
Regulatory Advocacy
PhRMA

_____/S/_____
Kelly Falconer Goldberg
Vice President, Law/Senior Counsel
for Biopharmaceutical Innovation
PhRMA