Prescription Drug User Fee Act (PDUFA) Reauthorization

Stakeholder Meeting with FDA

February 2, 2026 | 9:00 am - 11:00 am

FDA White Oak Campus, Silver Spring, MD and Virtual Format

MEETING PURPOSE

To gather stakeholder perspectives on key PDUFA VIII topics, including FDA's Rare Disease Innovation Hub, first cycle approvals and complete responses (CRs), and PDUFA resourcing and finances.

MEETING SUMMARY

This meeting was the fourth in a series of monthly PDUFA VIII reauthorization stakeholder consultation meetings held at FDA's White Oak Campus with a virtual participation option. Representatives from over 30 patient and consumer advocacy groups attended the meeting. The meeting covered three topics: the Rare Disease Innovation Hub, first cycle approvals and complete responses, and PDUFA resourcing and finances.

Rare Disease Innovation Hub

The Rare Disease Innovation Hub[1] was established in late 2024 to serve as a centralized focal point and coordination mechanism for FDA's rare disease programs, making it easier for stakeholders to navigate engagement pathways and enhancing product coordination across programs. The Hub focuses on policy and process issues rather than individual product reviews. FDA highlighted the success of the Rare disease Innovation, Science, Exploration (RISE) workshop series, which attracted approximately 2,400 attendees with roughly 50% from industry. For 2026, the Hub planned additional RISE workshops, Rare Disease Day activities, and an educational materials review initiative.

Stakeholders asked questions about dedicated staffing and resources for the Hub. Other questions focused on how the Hub facilitates collaboration between rare disease teams and its role in knowledge dissemination beyond workshop attendees. Stakeholders expressed strong support for the Hub's potential to streamline engagement pathways and facilitate cross-center collaboration, with one stakeholder suggesting new approach methodologies for rare diseases as a workshop topic. However, concerns were raised about whether the Hub has sufficient dedicated

[1] https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/fda-rare-disease-innovation-hub

staff to fulfill its mission effectively and ensure robust knowledge dissemination to the broader FDA review workforce.

First Cycle Approvals and Complete Responses

FDA presented data showing that the first cycle complete response (CR) rates[2] for original New Drug Applications (NDA) and Biologics License Applications (BLA), indicate an overall declining trend from fiscal year 2008 to fiscal year 2024 and have hovered, on average, around 30% in recent years with some year-to-year variability. FDA clarified that PDUFA performance goals focus on completing timely scientific reviews, not approval rates, which depend, in large part, on application quality. FDA shared that most applications that receive first cycle CRs ultimately achieve approval in subsequent cycles, with approximately 15-20% more applications approved after additional review cycles. FDA shared that an internal review of 150 CR letters for original applications received in fiscal years 2020-2024 revealed that approximately 95% of the letters identified inspections, clinical, and/or product quality issues as the reason(s) for receiving a CR.

Stakeholders asked whether FDA could provide greater transparency about reasons for CRs through a centralized public resource and whether CR statistics could be broken down by division or therapeutic category, particularly for orphan drugs. Stakeholders appreciated FDA's data presentation and highlighted the observation that most applications eventually achieve approval, which indicates that FDA is appropriately upholding safety standards. However, some stakeholders expressed concerns about the impact of CR timelines on patients with rare and serious diseases, noting that additional review cycles can result in irreversible disease progression and cost patient lives. Multiple stakeholders noted that even if FDA's data doesn't show increasing CR rates, media reports about changing review standards are eroding trust in FDA and creating uncertainty in the patient and consumer community. Consumer advocates also voiced concerns about potential erosion of FDA’s safety and efficacy standards, particularly given staff reductions and increased use of expedited pathways. Stakeholders emphasized the need for sufficient FDA resources to ensure rigorous oversight and adequate follow-through on postmarketing requirements.

PDUFA Resourcing and Finances

FDA explained that all PDUFA funds collected from fees are used to support statutorily allowable activities and expenses in the program, such as IND reviews, meetings between FDA and sponsors, supplement reviews, post-market safety activities, and policy development. The

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[2] The first cycle refers to the first time FDA conducts a complete review of an application resulting in an approval or complete response (CR). FDA will send the applicant a CR letter if the agency determines that FDA will not approve the application in its present form for one or more of the reasons given in § 314.125 or § 314.127, respectively.

annual target revenue is currently established through adjustments for inflation, strategic hiring and retention, capacity planning, operating reserve (10-14 weeks), and additional direct costs. To collect this revenue, FDA charges two types of fees: 1) application fees, paid when companies submit NDA and BLA applications, account for 20% of the total, and 2) program fees, paid annually by companies with approved products, account for 80% of the total. FDA shared that in recent years at least half of applications do not pay fees due to waivers and exemptions

FDA shared information on recent staffing changes and how that may impact PDUFA expenditures and fee adjustments in future years. Stakeholders requested division-level staffing data and more transparency about hiring, departures, and recusals. Consumer advocates raised concerns that declining staff and reduced revenues could erode safety and efficacy standards and requested information about safeguards in place. Concerns were raised about ensuring sufficient oversight for drugs approved through expedited pathways and whether FDA has adequate resources to handle increased workload from methods requiring more labor-intensive post-market studies. Overall, while stakeholders appreciated the financial transparency, they expressed mixed opinions about resource adequacy, with strong calls for more detailed, publicly available information about staffing levels and safeguards to maintain review standards despite workforce reductions.

Next Steps

FDA will host another stakeholder engagement meeting in March. If negotiation meetings are held with Industry in April, FDA will also host a stakeholder meeting in April.

PARTICIPANTS

STAKEHOLDERS	(* = in person attendee, ** = online attendee)
Alexander Naum**	Generation Patient
Annie Kennedy**	EveryLife Foundation for Rare Diseases
Anthony So**	Yale Collaboration for Regulatory Rigor, Integrity, and Transparency (CRRIT), Johns Hopkins Bloomberg School of Public Health
Brianna Greeno**	Breakthrough T1D
Brittany Avin McKelvey**	LUNGevity Foundation
Courtney Wallin**	LEAD Coalition
Cynthia Bens**	Personalized Medicine Coalition
Diana Zuckerman**	National Center for Health Research
Emily Anderson*	Physicians Committee for Responsible Medicine
Erin O'Quinn**	Parkinson's Foundation
Gavin Glingham**	Alliance for Patient Access
George Eastwood **	Emily Whitehead Foundation
Hoang Nguyen**	Lupus ABC
Ian Kremer**	LEAD Coalition

Irene Ulrich**	Center for Science in the Public Interest
Isabelle Xu**	Center for Science in the Public Interest
Jamie Sullivan*	EveryLife Foundation for Rare Diseases
Janet Krommes**	Doctors for America
Jeanne Ireland*	The diaTribe Foundation
Jeff Allen**	Friends of Cancer Research
Kara Berasi**	Haystack Project
Kaylin Bower**	On a Mission for Multiple Sclerosis LLC
Keith Desserich**	The Cure Starts Now
Mark Fleury**	American Cancer Society, Cancer Action Network
Michael Jones**	n/a
Michael T. Abrams**	Public Citizen
Michelle Adams*	NORD
Naomi Maxwell*	Humane World Action Fund
Nicole Boschi**	National Multiple Sclerosis Society
Pamela Gavin**	National Organization for Rare Disorders
Patricia Kelmar**	U.S. PIRG
Paul Melmeyer*	Muscular Dystrophy Association
Rachel Chon**	The Cure Starts Now
Ryan Fischer**	Foundation for Angelman Syndrome Therapeutics
Shion Chang**	National Health Council
Sophia Phillips**	Doctors of America
Tess Robertson-Neel**	National Center for Health Research
Therese Ziaks**	Yale School of Medicine

FDA

Adam Stamper	OC
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Shirley Seo	CDER