Prescription Drug User Fee Act (PDUFA) Reauthorization

Stakeholder Meeting with FDA

April 20, 2026 | 9:00 am - 11:00 am

Virtual Format

MEETING PURPOSE

To provide an overview of the proposed PDUFA VIII agreement and highlight opportunities for public engagement in the clearance process.

MEETING SUMMARY

The sixth PDUFA VIII Reauthorization Public Stakeholder Consultation Meeting was held on April 20, 2026, bringing together FDA officials and consumer advocacy groups to provide an overview of the proposed PDUFA VIII agreement. Stakeholders expressed appreciation for innovative approaches to patient safety and product quality while raising concerns about public health outcomes, transparency, and the need for independent researcher access to data. The FDA emphasized that the proposed changes represent refinements to existing practices rather than entirely new programs, with a focus on improving efficiency without compromising safety and efficacy standards. The meeting concluded with an overview of the reauthorization process timeline and opportunities for continued public engagement through formal comment periods and public meetings.

Pre-Market

FDA shared that the pre-market part of the proposed agreement focuses on review activities, communication in the human drugs review program, and transitioning regulatory science pilots and programs. New initiatives include prioritizing feedback on pivotal protocols and introducing multi-divisional meetings for sponsors developing products across multiple therapeutic areas. The section also creates new processes for requesting face-to-face meetings and advances drugs for rare diseases through up to ten Rare Disease Innovation, Science, and Exploration (RISE) workshops and transitioning the Rare Disease Endpoint Advancement (RDEA) pilot into a program.

Stakeholders asked whether RISE workshops would remain open to the public, and FDA confirmed their continued public accessibility. Questions emerged about goals and metrics for complete response letters and whether specific percentage thresholds were being targeted. Multiple participants emphasized the importance of public interest goals related to safety and

efficacy, not just efficiency improvements, with particular focus on endpoint validation and transparency in FDA's evaluation processes.

Participants shared support for innovative approaches to rare disease development and appreciation for maintaining public access to workshops. Participants raised concerns about the emphasis on speed and efficiency potentially overshadowing safety considerations. A participant observed that there were no specific new commitments for cell and gene therapy beyond staff training, and questions were raised about FDA's capacity to handle additional deliverables given existing resource constraints. The patient community expressed hopes for more documentation of patient experience data in regulatory decisions and labeling, welcoming case studies but seeking broader implementation.

Post-Market

FDA shared that the post-market section of the proposed agreement promotes safe use of medicines through continued enhancements to FDA's drug safety system, with primary focus on optimization of the Sentinel Initiative. FDA's presentation outlined commitments to maintain high-quality data infrastructure, advance implementation of the Sentinel 3.0 operating model, and streamline annual reporting with simplified metrics. The agreement also introduces biannual technical meetings between FDA and regulated industry to discuss Sentinel 3.0 plans and progress and explores new processes for sponsors to submit proposed approaches to postmarket studies before product approval.

Feedback from stakeholders highlighted concerns about transparency and inclusivity. Multiple participants criticized the exclusion of independent researchers from FDA-industry meetings, arguing that access should not be limited when discussing marketed products. Concerns were expressed about the lack of opportunities for substantive questions and input at current Sentinel stakeholder meetings, with participants noting that these forums do not provide adequate venues for addressing important safety questions about drugs used during pregnancy, psychotropic medications, and rare disease treatments. Stakeholders acknowledged the value of maintaining robust data infrastructure and expressed interest in enhanced communication about postmarket study approaches.

Manufacturing

FDA shared that the manufacturing section of the proposed agreement introduces enhanced engagement, preparedness, and transparency to mitigate facility risks through the new Chemistry, Manufacturing, and Controls (CMC) Facility Lifecycle Program. FDA's presentation described new options for CMC Facility Pre-submission meetings to discuss manufacturing facilities before evaluation and inspection, along with Post-Pre-Approval Inspection (Post-PAI) or Post-Pre-License Inspection (Post-PLI) meetings to address inspection findings. The program

aims to reduce deficiencies that could lead to multiple review cycles, and the agreement includes commitments to publish draft guidance and conduct an assessment of the new program.

Stakeholders inquired about the safety and efficacy implications of manufacturing changes for the public, including the evaluation of in-person versus remote inspections and their relative effectiveness in identifying problems. Participants also inquired about budget implications, commitments to hire additional staff through user fees, and how these changes might relate to America First initiatives for domestic manufacturing. Feedback was overall positive, with appreciation expressed for comprehensive approaches to ensuring product quality throughout the manufacturing lifecycle and emphasis on early engagement to help establish new facilities in the United States. Stakeholders raised concerns about resource allocation and whether FDA has adequate capacity to implement these enhanced programs effectively, with some stakeholders seeking assurance that enhanced efficiency would not compromise safety standards.

Finance

FDA shared that the finance section of the proposed agreement includes enhancements to financial transparency and modifications to the fee structure to incentivize sponsors to conduct clinical trials in the United States. Key proposed changes include a 50% reduction in the application fees for sponsors who start phase 1 clinical trials in the United States, introduction of fees for the first non-orphan supplement to orphan-only products, and limiting small business waiver eligibility to companies based in the United States. Additional proposed modifications include introduction of enterprise performance assessments to identify operational efficiencies.

Stakeholders inquired about implications for FDA from the 50% fee reduction and whether this change would lead to an increase in applications. Questions also emerged about FDA's ability to hire and retain staff at adequate rates, and whether the commitment letter addressed staffing challenges. Participants also inquired about transparency in the use of artificial intelligence (AI) and whether assessments of AI efficiency would be publicly available. Participants sought clarification regarding changes to orphan fees and annual program fees.

Some participants expressed appreciation for incentivizing clinical trials based in the United States. Questions were raised about the emphasis on phase 1 trials in the United States versus phase 2 and 3 trials, with some stakeholders suggesting that later-phase trials might be more important for patient access. Participants expressed support for maintaining scientific development opportunities in the United States and ensuring American patients can participate in the drug development ecosystem. Participants also restated their concerns about the lack of public health outcome metrics in the proposed agreement that assess whether the user fee investments are producing faster, safer drugs for patients.

Hiring and Retention and Information Technology

FDA shared that the proposed hiring and retention section focuses on targeted hiring and retention of technical and scientific staff through enhanced transparency and strategic workforce

planning. FDA's presentation outlined commitments to continue public webpages with quarterly hiring updates and net CDER and CBER hires, along with specific updates on restaffing of the PDUFA program. Priority areas for staffing include review functions and specific PDUFA VIII enhancements such as the Model-Informed Drug Development, Rare Diseases, Facility Lifecycle, and Sentinel programs. The section also addressed information technology transparency and collaboration through quarterly meetings with regulated industry and engagement in pilot testing of enterprise-wide systems.

Stakeholder feedback was generally supportive of transparency initiatives and strategic workforce planning, though concerns persisted about FDA's ability to attract and retain qualified staff in the current environment. Participants appreciated commitments to public reporting on hiring progress and prioritization of critical review functions. However, underlying concerns about overall agency capacity and resource constraints were evident throughout the meeting discussions, with stakeholders questioning whether proposed enhancements could be effectively implemented without adequate staffing levels. The emphasis on transparency in hiring and retention was viewed positively as a mechanism for public accountability and oversight of program implementation.

Next Steps

The meeting concluded with an overview of the reauthorization process timeline and opportunities for continued public engagement, including a public docket and public meeting. FDA staff indicated that negotiations are nearing completion and thanked stakeholders for their participation in this series.

PARTICIPANTS

STAKEHOLDERS

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
Brian Koffman	CLL Society
Brianna Greeno	Breakthrough T1D
Brittany McKelvey	LUNGevity Foundation
Chanel Press	Breakthrough T1D
Cynthia Bens	Personalized Medicine Coalition
Diana Zuckerman	National Center for Health Research
Emily Anderson	Physicians Committee for Responsible Medicine
Erfan Taherifard	Yale School of Medicine
Erin O'Quinn	Parkinson's Foundation
George Eastwood	Emily Whitehead Foundation

Hoang Nguyen	Lupus Research Alliance
Ian Kremer	LEAD Coalition
Isabelle Xu	Center for Science in the Public Interest
Jamie Sullivan	EveryLife Foundation for Rare Diseases
Janet Krommes	Doctors for America
Jason Schwartz	Yale School of Public Health
Jeanne Ireland	The diaTribe Foundation
Kara Berasi	Haystack Project
Kathryn "Taylor" Livelli	1Day Sooner
Kaylin Bower	On a Mission for Multiple Sclerosis LLC
Keith Desserich	The Cure Starts Now Foundation
Mark Fleury	American Cancer Society, Cancer Action Network
Mary Hilley	Humane World for Animals
Michael Jones	n/a
Michael T. Abrams	Public Citizen
Michelle Adams	NORD
Naomi Maxwell	Humane World Action Fund
Nicole Boschi	National Multiple Sclerosis Society
Patricia Kelmar	U.S. PIRG
Paul Melmeyer	Muscular Dystrophy Association
Reshma Ramachandran	Yale Collaboration for Regulatory Rigor, Integrity, and Transparency (CRRIT); Yale School of Medicine
Ryan Fischer	Foundation for Angelman Syndrome Therapeutics
Shion Chang	National Health Council
Sneha Dave	Generation Patient
Sophia Phillips	Doctors of America
Sudhir Sivakumaran	Lewy Body Dementia Association
Tess Robertson-Neel	National Center for Health Research
Therese Ziaks	Yale School of Medicine

FDA

Amy Ramanadham	CDER
Andrew Kish	CDER
Angela Granum	CDER
Christine Hunt	OGC
Emily Ewing	CDER
Issam Zineh	CDER
KaLonna Maull	CDER
Kate Greenwood	OGC
Kristopher Hoover	CDER
Mary Thanh Hai	CDER
Neha Gada	CDER

Pamela Acero	CDER
Jason Bunting	CDER
Sara Abdollahi	CDER
Sau Lee	CDER