Prescription Drug User Fee Act (PDUFA) Reauthorization

Stakeholder Meeting with FDA

January 9, 2026 | 9:00 am - 12:00 pm

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

MEETING PURPOSE

To gather stakeholder perspectives on key PDUFA VIII topics, including Patient-Focused Drug Development (PFDD), post-market safety, and orphan drug exceptions and exemptions.

MEETING SUMMARY

The third PDUFA VIII Reauthorization Public Consultation Meeting was held on January 9, 2026. The meeting focused on gathering stakeholder perspectives on key PDUFA programs and initiatives. Patient and consumer advocacy groups participated in discussions covering Patient-Focused Drug Development, post-market safety initiatives including Sentinel, Risk Evaluation and Mitigation Strategies (REMS) and Postmarketing Requirements (PMRs), and orphan drug fee exceptions and exemptions. The meeting captured stakeholder input via verbal question-and-answer sessions and collaborative whiteboards. Participants emphasized the importance of patient voice integration, transparency in regulatory processes, and maintaining incentives for rare disease drug development.

PFDD: Patient-Focused Drug Development

Patient-Focused Drug Development is a systematic approach designed to ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation processes. This initiative recognizes that effective patient-centered drug development cannot be accomplished by FDA alone and requires collaboration between patient organizations, FDA, and drug developers.

FDA's presentation highlighted the evolution of PFDD from its inception through current PDUFA VII commitments, including the development of methodological guidance documents and work on patient preference guidelines with ICH (International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use). FDA emphasized that patient input is most effective when incorporated early in development rather than late in the process. FDA described how the Agency dedicates a portion of FDA statisticians' time to PFDD work, conducts monthly cross-divisional meetings, and has successfully identified and corrected misinterpretations of FDA guidance that could have diminished clinical trial impact.

Stakeholders expressed strong support for PFDD. Some participants commended FDA for elevating the patient voice and hoped to see continued commitment to PFDD in PDUFA VIII. Others noted that PFDD is essential to understanding what matters to patients, citing recent meetings where patients had very different needs than drug developers or reviewers knew about. Participants emphasized that PFDD adds value by ensuring patient experience data is incorporated into drug development in a systematic, scientifically credible manner.

Stakeholders also raised several important questions and concerns during the discussion. Consumer advocacy groups inquired about conflict of interest considerations in patient data collection. Healthcare professional organizations asked about the relative importance of patient experience data in drug approvals, particularly in diseases where subjective and objective measures may not align. Academic organizations questioned FDA's approach to continuing validation of patient experience data endpoints. Participants emphasized the need for patient experience data to be treated as a design principle rather than an afterthought, called for more understanding about how PFDD influences regulatory decisions, and requested information about program resources including full-time equivalents (FTEs) and budget allocations. Some stakeholders also raised concerns about financial barriers that prevent smaller patient advocacy organizations from meaningfully participating in PFDD sessions, noting that these sessions require significant resources to access experts and generate reports.

Post-Market Safety: Sentinel Initiative

The Sentinel Initiative is a post-market risk identification and analysis system launched to meet statutory requirements for monitoring medical product safety using diverse healthcare data sources. The system is designed to help FDA determine whether its Active Risk Identification and Analysis (ARIA) capabilities are sufficient to assess serious safety risks before requiring sponsors to conduct post-market observational studies.

FDA's presentation outlined the evolution of the Sentinel Initiative to the proposed Sentinel 3.0, through which FDA aims to create a more efficient, centralized ecosystem serving CDER, CBER, and CDRH. Since implementation, Sentinel has completed hundreds of analyses that have informed regulatory activities including advisory committee presentations, labeling changes, and NDA/BLA reviews. The Biologics Effectiveness and Safety (BEST) Initiative, launched as part of Sentinel, has contributed significantly to biologics and vaccine safety monitoring. Current PDUFA VII commitments focus on pregnancy safety demonstration projects and negative control methodology development.

Stakeholders acknowledged Sentinel's value in post-market safety surveillance and expressed support for continued patient engagement within the system. Stakeholder questions centered on data access, transparency, and public engagement. Multiple participants emphasized the need for consistent public involvement across diverse populations and expressed disappointment with the limited actionable results from Sentinel. Participants also questioned the allocation of PDUFA funds to post-market safety initiatives (compared to pre-market work) and the cost-effectiveness of Sentinel (compared to other post-market surveillance methods). Participants

pressed for greater data transparency and public access to Sentinel data for independent verification. FDA explained that data use agreements with partners currently limit access to FDA only, though FDA noted that much of the underlying Medicare and Medicaid data is available through Research Data Assistance Center (ResDAC)[1]. Participants requested more transparency about when ARIA is used, as opposed to PMR studies, and asked about potential integration with FDA Adverse Event Reporting System (FAERS) dashboard capabilities. One stakeholder also emphasized the need for Sentinel data to be representative of all patient populations, including developmentally appropriate age subgroups and data on health conditions, race, sex, sexuality, and gender groups.

Post-Market Safety: Risk Evaluation and Mitigation Strategies (REMS) Assessment & Post Marketing Requirements (PMRs)

REMS are drug safety programs designed to ensure that medication benefits outweigh risks by implementing specific risk mitigation strategies beyond standard labeling. These programs can include medication guides, communication plans, Elements to Assure Safe Use (ETASU), and implementation systems with required assessment timetables.

FDA reported that REMS assessment methodology submissions have increased significantly in recent years, which FDA attributes to new PDUFA VII review timelines. Despite this dramatic increase in submissions, FDA has met its performance goals and achieved high compliance with review targets. Post-Marketing Requirements encompass several types of studies: accelerated approval confirmatory trials, animal efficacy rule studies, pediatric research, and safety studies. FDA has fulfilled its PDUFA VII commitments by establishing processes for communicating anticipated PMRs weeks before the goal dates and creating new procedures for PMR release requests.

Stakeholders recognized the importance of post-market safety programs and acknowledged FDA's efforts to improve REMS assessment processes and meet performance goals despite the significant increase in submissions. However, stakeholder discussions also revealed several concerns about transparency and implementation. Participants questioned the drivers behind increased REMS methodology submissions and highlighted challenges for companies in meeting post-market requirements, particularly for ultra-rare conditions where patient populations are extremely limited. Others asked about the impact of draft safety labeling guidance on REMS requirements and repeatedly pressed for enhanced transparency in REMS assessment processes and scientific justification for changes. Some participants raised concerns about potential conflicts of interest when companies evaluate their own REMS programs, citing opioid training as an example where independent evaluation revealed poor physician completion rates. Regarding PMRs specifically, stakeholders emphasized concerns about long timelines for post-market commitment completion and requested greater transparency around PMR processes and

[1] Research Data Assistance Center (ResDAC) website: https://resdac.org/

requirements. One participant expressed particular concern about drugs approved under accelerated approval, requesting earlier and more structured communication of postmarketing requirements to patients and emphasizing the need for timely completion of confirmatory studies to ensure patients can trust that medicines deliver on their promised benefits.

Orphan Drug Exceptions & Exemptions

FDA presented a PDUFA VIII proposal to address what it characterized as an equity issue in the current orphan drug fee structure. Currently, sponsors can receive application fee exceptions for orphan-designated products and later expand to non-orphan indications without paying application fees and continuing to receive program fee exemptions.

FDA explained that the proposal would require a sponsor of a product approved for orphan-only indications to pay an application fee when they submit a supplement intended to expand to non-orphan indications. FDA voiced that this approach would ensure equity and consistency across the fee structure by ensuring that the orphan exception is not utilized for non-orphan indicated products. The estimated financial impact of the proposal is relatively modest: FDA sees about 2.5 instances per year of approved orphan products later adding non-orphan indications without paying the application fee; only 17 program fees for products with non-orphan indications receive the orphan exemption out of 2,781 total fees paid in FY 2025. FDA initiated this proposal from an equity perspective, as current exceptions result in higher fees for sponsors who are paying their fees.

Stakeholders appreciated FDA's transparency in presenting the proposal and acknowledged the equity concerns FDA is trying to address. Overall, stakeholder feedback was mixed and leaned toward a cautionary approach about the proposal rather than a supportive one. Participants expressed strong concerns about reducing incentives for rare disease drug development, worrying about limiting access to therapies. Suggestion was made to revise the definition of what constitutes an orphan drug indication. Others questioned whether the proposal aligns with Congressional intent and requested statutory analysis. Rare disease advocates emphasized that a significant percentage of Americans live with rare diseases while very few have FDA-approved treatments, and they argued against reducing any incentives when sponsors are already fleeing the rare disease space. Some participants questioned whether the administrative complexity was worth the minimal revenue impact and suggested the proposal would create unnecessary administrative complications. Multiple stakeholders echoed concerns about discouraging innovation in rare diseases. Additionally, some stakeholders requested broader transparency regarding the orphan drug indication program and suggested that if fees are reinstated for products with non-orphan indications, consideration should be given to removing previously granted market exclusivity from the initial waiver approval. Participants noted that the current structure incentivizes companies to pursue rare indications first and worried that the proposed change would reduce this incentive, potentially harming the treatment development environment for rare diseases. Overall, participants needed more time to assess the proposal and what was shared were initial reactions.

Next Steps

FDA will produce and post meeting minutes publicly following the meeting. The next PDUFA VIII Reauthorization Stakeholder Consultation Meeting is scheduled for Monday, February 2, 2026, with topics to be determined. FDA indicated that financial information about PDUFA, which was requested during this stakeholder meeting, may be addressed in future meetings based on stakeholder survey feedback. The Agency emphasized its commitment to incorporating stakeholder perspectives into ongoing PDUFA VIII negotiations with industry while maintaining focus on areas within the scope of user fee reauthorization.

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
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Mark Fleury**	American Cancer Society, Cancer Action Network
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Michael T. Abrams*	Public Citizen

Michelle Adams*	NORD
Naomi Maxwell**	Humane World Action Fund
Nicole Boschi**	National Multiple Sclerosis Society
Nishith Pandya**	American Cancer Society, Cancer Action Network
Pamela Gavin**	National Organization for Rare Disorders
Patricia Kelmar*	U.S. PIRG
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