Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Premarket Subgroup

December 2, 2025 | 1:00 pm–3:00 pm

FDA White Oak Campus, Silver Spring, MD

MEETING PURPOSE

To continue negotiating FDA's Model-Informed Drug Development (MIDD) proposal, FDA's Rare Disease proposal, and Industry's Facilitate First Cycle Reviews proposal.

PARTICIPANTS

FDA		INDUSTRY	
Mary Thanh Hai	CDER	Mark Taisey	BIO (Amgen)
Nana Adjeiwaa-Manu	CDER	Donna Boyce	PhRMA (Pfizer)
Marie Bradley	CDER	Annetta Beauregard	BIO
Irene Chan	CDER	Rob Berlin	BIO (Vertex)
Kathleen Davies	CDER	Steve Berman	BIO
Emily Ewing	CDER	Carl Garner	PhRMA (Eli Lilly)
Sonday Kelly	CBER	Kelly Goldberg	PhRMA
Andrew Kish	CDER	Kristy Lupejkis	PhRMA
Mark Levenson	CDER	Alison Maloney	PhRMA (Bayer)
Rajanikanth Madabushi	CDER	Adora Ndu	BIO (Bridge Bio)
Janet Maynard	CDER	Katrin Rupalla	PhRMA (J&J)
Jennifer Mercier	CDER	Drew Sansone	BIO (Alkermes)
Paul Phillips	CDER	Derek Scholes	BIO
Amy Comstock Rick	CDER	Lucy Vereshchagina	PhRMA
Katie Rivers	CBER		
John Scott	CBER		
Issam Zineh	CDER		

MEETING SUMMARY

FDA and Industry discussed FDA's MIDD proposal, began negotiating FDA's Rare Disease proposal, and continued discussing FDA and Industry's perspectives on Industry's Facilitate First Cycle Reviews proposal.

Approach to FDA's MIDD Proposal

FDA presented on the high demand for the MIDD program and shared that FDA has addressed the demand with limited staff capacity. FDA expressed that the MIDD program is seeking resources to expand the program by removing the limit on the number of submissions it can accept. Industry asked about what resources would be needed for the MIDD proposal.

Industry also asked how learnings happen from the MIDD program and the process for making the program a part of standard regulatory practice. FDA stated that the proposal is not resource neutral and that the MIDD program is already part of standard regulatory practice and over the course of several years, has accepted more than the 8 per year cap across many review divisions. FDA also shared that MIDD staff review each submission carefully, considering the specific context, regulatory questions posed, and whether FDA has regulatory familiarity with the topic. FDA noted that the MIDD program staff consults with review disciplines to disseminate learnings from the program.

FDA and Industry tentatively agreed on the value of the MIDD program and will move discussion of resources to the Finance subgroup. Industry also expressed an interest in making MIDD a part of standard regulatory practice and noted MIDD is a good example of translating pilot to practice. Additionally, Industry noted they want to make the Advancing Real-World Evidence program a part of standard regulatory practice as well.

Approach to FDA's Rare Disease Proposal

FDA presented a recap of the Rare Disease proposal for PDUFA VIII, stating that in PDUFA VIII, FDA is interested in incorporating external feedback into FDA rare disease training and programming, facilitating science-based workshops, and continuing support for endpoint development. FDA presented an overview of current PDUFA VII rare disease commitments, which comprise integrating rare disease staff in CDER and CBER into review teams and consultations, as needed, providing training to review staff, the Rare Disease Endpoint Advancement (RDEA) program, and engaging in external outreach with Industry, patient groups, and other stakeholders. FDA also presented selected rare disease accomplishments in CDER and CBER. FDA asked for Industry's feedback on FDA's work under the current PDUFA VII commitments.

Industry commended FDA for its rare disease work and expressed an interest in understanding how the Agency's rare disease work could be translated into standard review practice. Industry asked questions about how rare disease staff are integrated into review teams, whether there is a pathway for sponsors to receive feedback from rare disease experts, how rare disease learnings are shared in CDER and CBER, how FDA plans to incorporate external feedback into rare disease training and programming, and resources for the proposal. Industry expressed frustration in differences across review divisions. FDA responded that CDER and CBER each have subject matter experts across multiple disciplines that support rare disease issues in their respective

Centers. FDA also stated that review teams or sponsors can request that these subject matter experts with experience in rare diseases be involved in meetings.

FDA noted that there are several rare disease issue areas the Agency wants to enhance and presented the position that the Rare Disease Innovation Hub ("the Hub") is one way that FDA will consider and incorporate external feedback into review processes across Centers. Industry asked questions about how the Hub is involved in regulatory reviews. FDA responded that the Hub is not engaged at product specific review level, rather the Hub receives external feedback from rare disease stakeholders for training and programming and connects the rare disease work that CDER and CBER perform. FDA and Industry agreed to continue discussing the rare disease proposal and what the Hub provides, and the resources needed for the rare disease proposal.

FDA-Industry Discussion on Industry's Facilitate First Cycle Reviews Proposal

FDA presented perspectives on data Industry shared at the November 18th meeting about the rationale for the Facilitate First Cycle Reviews proposals. FDA presented its position that the Agency has successfully incorporated and implemented the Program agreed to in the PDUFA commitment letter[1] based on meeting and, in most cases, exceeding the performance goals for applications reviewed under both the Program and those outside the Program.

In response to Industry's position that data in the FY2024 PDUFA Annual Performance Report shows the first cycle approval rate has decreased (and conversely, the first cycle CRL rate has increased), FDA presented data from the Agency's 2024 Annual Performance Report. Overall, FDA's data presentation supported FDA's conclusion that FDA review performance metrics are at or above agreed-upon goals for NME NDA/BLAs, supplements and resubmissions. Regarding first cycle approval/CRL rates, FDA showed data to support its conclusion that there is no increasing trend in 1st cycle CRLs with the exception of Biologics License Applications (BLAs) between 2020-2022, for which the majority of CRLs were related to manufacturing/facility deficiencies. The Chemistry, Manufacturing, and Controls (CMC) subgroup is discussing ways to solve this issue. FDA noted that the goals letter does not specify any threshold for approval or CRL rate, as the regulatory decision on a marketing application is based on data submitted in the marketing application. FDA must refuse to approve the application based on several reasons stated in statute, including but not limited to data showing that the drug is unsafe for its intended use, there is insufficient information to determine the product is safe for its intended use, and there is lack of substantial evidence of effectiveness.

[1] The Program refers to the model described in the PDUFA VII commitment letter for the review of all New Molecular Entity New Drug Applications (NME NDAs) and original Biologics License Applications (BLAs), including applications that are resubmitted following a Refuse-to-File decision received from October 1, 2022, through September 30, 2027. The Program was established under PDUFA V (2013-2017) and continued under PDUFA VI and VII.

FDA also presented data from the 2024 Annual Performance Report to counter a slide presented by Industry at the November 18th meeting. FDA's data reveal that the median time to approval for all Standard and Priority filed NDAs and BLAs has remained relatively flat and within the review timelines agreed to under the Program.

FDA closed their presentation with its position that the Agency does not agree with Industry's overall proposal to Facilitate First Cycle Reviews, because FDA stated there is no data to support the assertion that FDA has not been meeting the objectives of the Program. FDA raised concerns that the proposal will inhibit the Agency's ability to meet its user fee commitments and ability to deliver on the many new initiatives announced by this Administration. FDA noted that the Agency would work with Industry on a third-party assessment that can inform whether there is need for changes to review and communication practices, in particular reasons for CRLs. However, FDA expressed that the Agency will not accept new performance goals tied to metrics proposed under Industry's Facilitate First Cycle Reviews proposal.

Industry presented their position in response to FDA's perspectives on the Facilitate First Cycle Reviews proposal. Industry expressed that the current CRL rate is not achieving the stated goal of PDUFA VII of promoting the efficiency and effectiveness of the first cycle review process and minimizing the number of review cycles necessary for approval and that Industry is expecting to see more efficiencies in the review process. FDA responded that the language pertaining to the Program in the commitment letter does not specify a threshold for CRL rates and pointed out that since the Program was implemented under PDUFA V, CRL rates decreased by 10% and have remained steady for the past 12 fiscal years. FDA reiterated the reasons why an application may receive a CRL as per the statute. A flat CRL rate may reflect a baseline rate that might include reasons due to a drug development program itself (e.g., Phase 3 trials failed, or drug toxicity identified during NDA/BLA review) that could not be fixed during a first cycle review. Industry expressed that they are trying to avoid delays and unexpected challenges in the first cycle review process due to late-in-the review cycle FDA comments or FDA requests for information that extend the timeline. Industry presented a counterproposal outlining existing metrics that they suggest are most important for tracking, especially considering these are already listed in FDA's MAPPs and SOPPs. Industry also presented an example of sponsors who have received late labeling comments that ended in a CRL. Industry concluded by briefly presenting a list of prioritized Investigational New Drug (IND) protocols that they believe are important to track.

FDA responded that they understood the pain points Industry raised and highlighted the Agency's staffing challenges with an estimated 20% loss of reviewers this past year while the volume of applications and meetings submitted have increased. FDA particularly noted that the number of efficacy supplements received per year exceeds 200 and far exceeds the number of applications submitted under the Program. Despite this volume of workload, FDA showed data where first cycle approval rates have remained steady above 85-90% for standard and priority supplements and the converse, a flat CRL rate of <10-15% over the course of many FYs. FDA again questioned what problem Industry is seeking to solve with its proposal to facilitate first cycle review. Industry responded that Industry is trying to address information requests late in

the review cycle leading to delays, including complete response letters. FDA agreed to review Industry's counterproposal on tracked metrics in further detail and share perspectives at a subsequent meeting.

Next Steps

The goals for the next meeting on December 4th will be to continue discussing Industry's Improve FDA-Sponsor Interactions proposal, FDA's Meetings Management proposal, and Industry's Incorporate Regulatory Science into Regulatory Decision-Making proposal.