Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Premarket Subgroup

December 4, 2025 | 1:00 pm–3:00 pm

FDA White Oak Campus, Silver Spring, MD

MEETING PURPOSE

To confirm the approach to FDA's Model-Informed Drug Development (MIDD) proposal and continue negotiating Industry's Incorporate Regulatory Science into Regulatory Decision-Making along with FDA's Meetings Management proposals.

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 confirmed their approach to FDA's MIDD proposal and negotiated Industry's Incorporate Regulatory Science into Regulatory Decision-Making proposal. FDA and Industry continued negotiating FDA's Meetings Management proposal.

Confirm Approach to FDA's MIDD Proposal

FDA expressed that the Agency felt they aligned with Industry during their December 2nd discussion on MIDD. FDA summarized that Industry raised questions about resourcing, and that both sides agreed to defer discussion of this matter to the Finance subgroup. FDA asked if Industry had any outstanding questions and whether they were aligned on next steps.

Industry agreed on the value of MIDD and expressed that they support the proposal. Industry also agreed with deferring discussion of resourcing to the Finance subgroup. FDA and Industry agreed to draft PDUFA VIII commitment letter language for MIDD in the meantime.

Approach to Incorporate Regulatory Science into Regulatory Decision-Making Proposal

Industry presented their perspective on translating the Advancing Real-World Evidence (RWE) Pilot Program into practice. Industry highlighted that some of the PDUFA VII Advancing RWE commitments are still outstanding, and that they want FDA to continue those commitments through to completion. For PDUFA VIII, Industry presented the position that they want to leverage the outcomes from the PDUFA VII commitments and memorialize learnings, as appropriate, through updated guidances, Manuals of Policies and Procedures (MaPPs), Standard Operating Procedures and policies (SoPPs), and reviewer training programs.

Additionally, Industry proposed to retain and enhance FDA staff capacity to review RWE-based proposals and support socialization of concepts among review teams. Industry reiterated that the goal is for FDA to use RWE in regulatory decision-making, and they have not seen improvements in this area coming out of the Advancing RWE Pilot Program. In addition, Industry proposed eliminating the separate RWE pilot meeting structure and having sponsors use traditional PDUFA meetings to gain review division feedback on RWE-based approaches. The purpose of this proposal was to ensure that RWE subject matter experts (SMEs) are available to consult with review teams and that the process enables shared decision making led by the SME and endorsed by the review team leading to binding decisions. Industry also proposed redirecting previously provided PDUFA resources for RWE to ensure they support the discussions occurring under traditional PDUFA meeting types. Lastly, Industry proposed creating a new section in the action package documenting how FDA considered the RWE-based approach, the strengths and limitations of the proposed approach, and what (if any) regulatory decisions were informed and reflected in labeling. This documentation would inform aggregate Performance Reporting on FDA's use of RWE in regulatory decision-making and in labeling. Industry stated the intent of this proposal was to help enable greater use of RWE for regulatory decision-making and understanding how it is being used in regulatory decision making.

FDA asked clarifying questions about Industry's understanding of retaining and enhancing staff capacity and training to support RWE and Industry's perspective on how to redirect resources for RWE. Industry responded that they want to ensure the right staff are trained on RWE and deferred to FDA on the appropriate approach to distribute the existing resources to advance the

use of RWE for regulatory decision-making and transition the RWE program into standard review practice.

Industry concluded their presentation by sharing proposed next steps for the Rare Disease Endpoint Advancement (RDEA) Pilot Program, the Complex Innovative Design (CID) Program, and the MIDD Program. For RDEA, Industry suggested completing the discussion on the Pilot Program as well as on the Rare Disease Innovation Hub in a future meeting. Industry reaffirmed that FDA would draft commitment letter language for CID and MIDD for Industry's review.

FDA presented their position on translating pilots and programs into practice, noting that apart from RDEA and RWE, all the other programs (MIDD, CID, Benefit Risk Assessment, Patient Focused Drug Development, and Drug Development Tools, including biomarker qualifications) are already established. In turn, FDA asked whether Industry was suggesting the language for these programs be removed from the commitment letter. FDA and Industry agreed to consider whether to remove language referencing these programs from the commitment letter and this would be a topic for discussion at a future negotiation meeting.

FDA also asked clarifying questions about Industry's proposal that FDA include a new section on innovative approaches and tools used in regulatory decision-making in the action package, train all CDER and CBER review staff on using innovative approaches, and provide a performance report on use of these approaches. FDA agreed to provide their perspectives on Industry's proposal to incorporate learnings from innovative approaches, while Industry agreed to provide more detail on how to incorporate innovative approaches into the action package.

Approach to FDA Meetings Management Proposal

FDA presented data on Type C Surrogate Endpoint meeting volume in response to Industry's November 20th request, sharing that there had been few requests and suggesting that discussion of Surrogate Endpoints could take place in regular Type C meetings. Industry asked how the Type C Surrogate Endpoint meeting type would be continued if it was moved to the general Type C meeting. Additionally, Industry expressed that many sponsors want to discuss Surrogate Endpoints with nonclinical disciplines and emphasized wanting to receive more specialized feedback. FDA agreed to consider the questions Industry raised and discuss them at a subsequent negotiation.

Industry presented a counterproposal, sharing that sponsor evidence generation timelines would be delayed if FDA meeting timelines change, potentially delaying their clinical programs by a month or more. Industry also expressed a pain point that there are still issues on both sides regarding "white space" between meetings, stating that sponsors plan for the submission of a meeting request based on the availability of relevant data, and that FDA's proposal to require the meeting package be submitted at the time of a meeting request could cause unnecessary delays. FDA agreed to consider a revised meetings management proposal in response to the counterproposal Industry presented. Industry also agreed to share experiences on Type D

meeting requests being converted to a Type C meeting. Lastly, FDA agreed to share their feedback on Industry's Improve FDA-Sponsor Interactions proposal.

Next Steps

The goals for the next meeting on December 9th will be to continue discussing the Advancing RWE, Rare Disease and Facilitate First Cycle Reviews proposals.