



Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Premarket Subgroup

January 6, 2026 | 1:00 pm-3:00 pm

FDA White Oak Campus, Silver Spring, MD

MEETING PURPOSE

To discuss FDA’s Advancing Real-World Evidence (RWE) proposal, FDA’s Rare Disease proposal, and Industry’s Incorporate Regulatory Science into Regulatory Decision-Making proposal.

PARTICIPANTS

FDA

Mary Thanh Hai	CDER
Nana Adjeiwaa-Manu	CDER
Thamar Bailey	CDER
Marie Bradley	CDER
Irene Chan	CDER
Kathleen Davies	CDER
Emily Ewing	CDER
Sonday Kelly	CDER
Andrew Kish	CDER
Mark Levenson	CDER
Rajanikanth Madabushi	CDER
Janet Maynard	CDER
Paul Phillips	CDER
Amy Comstock Rick	CDER
Katie Rivers	CDER
John Scott	CDER

INDUSTRY

Mark Taisey	BIO (Amgen)
Donna Boyce	PhRMA (Pfizer)
Annetta Beauregard	BIO
Rob Berlin	BIO (Vertex)
Steve Berman	BIO
Kelly Goldberg	PhRMA
Kristy Lupejkis	PhRMA
Alison Maloney	PhRMA (Bayer)
Adora Ndu	BIO (Bridge Bio)
Katrin Rupalla	PhRMA (J&J)
Drew Sansone	BIO (Alkermes)
Derek Scholes	BIO
Lucy Vereshchagina	PhRMA

MEETING SUMMARY

FDA and Industry agreed to discuss Industry’s perspective on the Improve FDA-Sponsor Interactions and Meetings Management proposals at the next meeting. FDA and Industry discussed a proposed approach for the Advancing RWE proposal. FDA and Industry continued discussing the Rare Disease Innovation Hub (The Hub) and the Rare Disease Endpoint Advancement (RDEA) pilot. Industry presented proposed draft commitment letter language for

Drug Development Tools (DDT), including biomarker qualification, and Benefit-Risk Assessment.

Approach to Meetings Management and Improve FDA-Sponsor Interactions Proposals

FDA raised the possibility of re-engaging in negotiations of proposals related to improving FDA-Sponsor Interactions and Meetings Management. Industry agreed that these topics should be brought back for discussion at a future meeting.

Approach to Advancing RWE Proposal

FDA presented a summary of the Advancing RWE proposal negotiations thus far, noting that both the Agency and Industry recognize the value of real-world data (RWD) and real-world evidence (RWE) in supporting regulatory decision-making.¹ FDA stated it intends to use its current staff to continue advancing the use of RWE in regulatory decision-making, while removing the Advancing RWE pilot from the PDUFA VIII commitment letter.

FDA also presented draft commitment letter language for Industry's consideration. Industry agreed to review the commitment letter language FDA presented and bring feedback to a subsequent meeting.

Industry asked questions about how the RWE program would train non-RWE Agency staff and how the proposed draft commitment letter language could incorporate new Agency RWE-focused initiatives. FDA responded that the RWE program already trains non-RWE staff across the Agency and would continue to do so in PDUFA VIII. FDA recommended not including new Agency RWE-focused initiatives in the commitment letter since they are early in development.

Industry also asked for clarification on how RWE FTEs are distributed across CDER and CBER. FDA provided initial data on the distribution of RWE FTEs across CDER and CBER. Industry asked if there would be metrics to track how often RWE was used in regulatory decision making. FDA and Industry agreed to continue discussing how the use of RWE could be captured in the regulatory checklist at a subsequent meeting.

Approach to DDT and Benefit Risk Assessment Draft Commitment Letter Language

Industry presented proposed draft PDUFA VIII commitment letter language for DDT, including biomarker qualification, and Benefit Risk Assessment. FDA agreed to review the language internally and bring feedback to the next negotiation.

Approach to Rare Disease Proposal

FDA presented the Hub's internal and external impact. Internally, FDA noted that it focuses on coordinating in-depth rare disease conversations between CDER and CBER reviewers through initiatives such as Rare disease Innovation, Science, and Exploration (RISE) workshops. FDA also presented on its engagement with Capitol Hill, patient and advocacy groups, and other external

¹ See the December 9th meeting summary for details.

stakeholders who expressed their support for rare disease efforts. FDA concluded its discussion of the Hub's impact by noting that public stakeholders recently submitted a letter to FDA requesting adequately resourcing the Hub.

FDA also presented on the Hub's proposed Rare Disease Feedback Meetings. The goal of these meetings is to obtain feedback from industry and other external stakeholders on how FDA can further innovate in the rare disease space.

Industry noted that it is interested in seeing the Agency's proposal clearly articulate how the Hub's work translates into prescription drug review. Industry asked several clarifying questions to understand how reviewers will use information from the Hub in the review process. FDA responded that it is aligned on ensuring the Hub adds value to rare drug review. FDA also stated that the Hub is separate from individual Center review teams and instead focuses on policy, processes, and ensuring consistency between CDER and CBER. Industry indicated the value of workshops on a meaningful scientific question followed by a clear and tangible outcome such as guidance. FDA and Industry will continue discussing the Hub proposal.

Industry presented its RDEA counterproposal, proposing to support enhancing review teams' capacity to review novel endpoints, eliminate the separate RDEA meetings pilot structure, and allow sponsors to use available FDA meeting types to request rare disease novel endpoint-focused meetings. Industry noted that it supports RDEA and wants it to be more widely available for sponsors, given that it has been helpful for companies that have participated thus far. Industry proposed a redirection of previously provided PDUFA resources to support the discussions that will occur under traditional PDUFA meeting types.

FDA responded that the counterproposal would mean a return to a previous process for evaluating endpoints for rare disease drug development, where dedicated subject matter experts unique to the rare disease pilot program were not available to help facilitate endpoint development. Industry cited the importance of making Rare Disease Endpoint Assessment more broadly available (no limit) and across all FDA Centers given half of novel drugs are approved for rare diseases. FDA stated that most of the drugs approved for rare diseases were based on established endpoints for which the RDEA pilot program would not have been necessary. FDA noted that Sponsors can request the RDEA pilot and if denied, Sponsor can pursue the traditional PDUFA meeting route. FDA indicated RDEA was designed for those programs where the science is the most novel and has promising endpoints and utilizes specific subject matter experts to address. FDA is not sure sponsors will receive the same approach if folded into traditional review. FDA agreed to consider Industry's counterproposal and present a response at a subsequent meeting.

Next Steps

The goals for the next meeting on January 8th will be to discuss Industry's Improve FDA-Sponsor Interactions proposal, FDA's Meetings Management proposal, Industry's Incorporate Regulatory Science into Regulatory Decision-Making proposal, and Industry's Facilitate First Cycle Reviews proposal.