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Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Premarket Subgroup

December 11, 2025 | 10:30 am-12:30 pm

Virtual Format

MEETING PURPOSE

To continue discussing FDA's Rare Disease, Industry's Incorporate Regulatory Science into Regulatory Decision Making, and Industry's Enhancing Transparency and Consistency Related to Patient Experience Data (PED) proposals.

PARTICIPANTS

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

MEETING SUMMARY

FDA and Industry continued discussing FDA's Rare Disease proposal and discussed a proposed approach to incorporating Drug Development Tools (including biomarker qualification) along with Benefit-Risk Assessment language in the PDUFA VIII commitment letter. FDA and Industry discussed FDA's counterproposal to Industry's Enhancing Transparency and Consistency Related

to PED proposal. FDA proposed to withdraw its Streamlining Review of Certain Efficacy Supplements proposal from consideration in the user fee negotiations. Industry presented a counterproposal to a portion of its Facilitate First Cycle Reviews proposal.

Approach to Rare Disease Proposal

FDA presented a response to Industry's data request, which inquired about the distribution of the Agency's PDUFA Full-Time Equivalents (FTEs) currently dedicated to rare diseases along with the volume and attendance at the Agency's rare disease training courses. FDA noted that rare disease FTEs have been distributed in offices across CDER and CBER, noting that the Centers work together, and provided a list of the FTEs' specific roles within each Center. The FTEs' roles include providing rare disease consults, working with the Translational Sciences Team (TST), providing Rare Disease Drug Development Design (RD4) consults, and conducting RDEA proposal reviews. PDUFA Rare Disease FTEs also manage the Rare Pediatric Disease Priority Review Vouchers program, the Rare Disease Evidence Principles (RDEP) program, and the Support for Clinical Trials and Advancing Rare Disease Therapeutics (START) program. FDA presented data on rare disease training within the Agency, which FDA stated has been attended by a significant number of reviewers involved in rare disease product review. FDA also stated that the Agency deploys several internal communications to inform rare disease staff of new areas in rare disease drug development, case studies used by external rare disease stakeholders, and of current as well as past trainings held.

FDA presented its resource ask for the Rare Disease proposal, which will support adding the Rare Disease Innovation Hub's (the Hub) Rare Disease Feedback Meetings, additional Rare Disease Innovation, Science, and Exploration (RISE) workshops, and converting the Rare Disease Endpoint Advancement (RDEA) pilot into a full program. FDA also reiterated the purpose of the Hub.[1]

Industry asked questions about the topics FDA covers at Rare Disease Feedback Meetings that help Industry with its drug development and how PDUFA rare disease FTEs are incorporated into rare disease product reviews. Industry asked for confirmation on whether the resources FDA requested would be in addition to what the Agency received in PDUFA VII, how the FTEs requested would be distributed between review and non-review work, and the Agency's current rare disease resource constraints.

Industry also presented the position that they believe in the importance of rare disease efforts, but want to better understand how the learnings from the Hub translate into further supporting orphan disease drug development. Specifically, Industry stated that over half of approvals are for rare disease. Industry also stated that while FDA reviewers may receive training on topics and issues related to rare disease drug development and approaches for review, it is not always clear that the learnings are consistently applied across review divisions. Industry also presented the

[1] See the December 9th meeting summary for details on the Hub.

position that they believe the RDEA pilot program has been working and stated that they would think more about how to move the pilot forward. Industry shared that they would present more detailed perspectives on FDA's rare disease proposal at a subsequent negotiation.

FDA stated that during the Rare Disease Feedback Meetings, the Agency focuses on current topics in the rare disease space and case studies on the challenges and opportunities within rare disease product development. FDA presented the position that the Agency wants to obtain additional feedback from external stakeholders on what topics to cover at the rare disease feedback meetings. FDA also stated that currently, review teams internally decide whether they need rare disease FTE support, and that this process has been working well. FDA confirmed that its PDUFA VIII resource ask would be in addition to what they received in PDUFA VII.

FDA also emphasized its focus on connecting rare disease representatives from CDER and CBER with the Hub through the RISE workshops so that they can apply information from the workshops to supporting medical product development. Industry asked how rare disease subject matter experts (SMEs) are pulled into product reviews. FDA stated that it is clear when rare disease SMEs are needed for product reviews. Industry asked if FDA has had a shortage of rare disease SMEs or how often rare disease SMEs are requested but unable to be provided. FDA stated that it currently is facing resource constraints because of staff loss and increasing responsibilities, many often competing with user fee commitments, and that while the Agency has plans for how the rare disease program can continue to expand, it is challenging to predict what will happen in the future. FDA also stated that it wants all PDUFA rare disease FTEs to support rare disease product development and ensure that the rare disease program has the capacity to attend sponsor meetings as needed. FDA agreed with Industry's approach to present their thinking on the rare disease proposal at a subsequent negotiation.

Approach to Incorporate Regulatory Science into Regulatory Decision-Making Proposal

FDA presented a summary of FDA and Industry's discussion on Industry's Incorporate Regulatory Science into Regulatory Decision-Making proposal, noting that Industry and FDA had agreed to separately review commitment letter language for PDUFA VII regulatory science programs to determine what changes, including deletions, should be made. FDA shared proposed changes to the commitment letter language for Drug Development Tools (DDT), including biomarker qualification, and Benefit Risk Assessment. Industry responded with initial feedback on the proposed changes and agreed to respond with its proposed language at a subsequent negotiation.

Approach to Enhancing Transparency and Consistency Related to PED Proposal

FDA presented perspectives on Industry's Enhancing Transparency and Consistency Related to PED Proposal, sharing areas of alignment, concerns, and a counterproposal. FDA stated that they see a shared commitment to Patient-Focused Drug Development and the value of incorporating patient input throughout drug development and decision making. FDA presented the position that it is challenging to identify what PED has been submitted and where it is in sponsor submissions, stating that 20% of fiscal year 2024 applications included this information as

requested by the electronic Common Technical Document Technical Conformance Guide (eCTD TCG).

FDA stated that sponsors frequently do not articulate how submitted PED should inform specific regulatory decisions, and that often the information considered PED is not collected using appropriate methodological approaches that would support regulatory decision-making. FDA presented the position that Industry's proposal to have FDA document all types of PED in the drug development program and how FDA considered them in its decision making and labeling consideration conflicts with PDUFA VIII goals of streamlining review processes and reducing review timelines. FDA expressed that notwithstanding that FDA must search within the submission for PED, once identified, not all such data are fit-for-purpose in the final regulatory decision or can support labeling. FDA stated that requiring FDA reviewers to document and write a short paragraph about each PED submitted, even in one place in the final review, would create additional burden for reviewers and lengthen written reviews as well as review timelines. FDA also presented additional context that PED informs earlier decisions that are not re-evaluated during NDA and BLA review.

FDA stated that it appreciates the importance of PED and wants to ensure that sponsors submit PED that informs regulatory decisions to the Agency. FDA presented a counterproposal that it stated addresses Industry's ask and provides sponsors with high quality information. FDA proposed to create and publish case studies that demonstrate how PED was considered in specific regulatory decisions across therapeutic areas and to host a public meeting to facilitate broader dialogue on collection, submission, and use of PED in drug development and regulatory review. FDA stated that this proposal would provide Industry with high quality examples that provide insight into FDA thinking and regulatory expectations, avoid slowing down review time by preventing additional burden on FDA review staff, and allow for discussion of how PED informs earlier recommendations throughout the drug development process. FDA stated that the public meeting would provide an opportunity for Industry, patient groups, and other external stakeholders to discuss case studies, ask clarifying questions, and provide feedback on the use of PED.

Industry asked if FDA was thinking of publishing positive and negative case studies, stating that Industry could learn more from negative examples to avoid a false perception of how PED is being used for regulatory decisions. Industry also asked whether FDA would be open to documenting the use of PED in a checklist within the action package. Industry noted that the current PFDD form should make it possible for FDA reviewers to identify if an application contains PED and its placement within the application, and if FDA reviews the submitted PED, the reviewers should be able to document if they used it in regulatory decision-making. Industry also stated that they recognized an opportunity for Industry to help FDA clearly understand when PED is included that could possibly enable the Agency's articulation of how that information was used in the action package and asked if their understanding was correct. Industry also stated that it was unclear how documenting how PED was used in regulatory decision making would delay the action packages, expressing that PED is very important to Industry, especially its inclusion in labelling.

Industry also indicated that there is PED being utilized in Europe but not in the US,, and any additional detail from FDA would be useful. Additionally, Industry heard that patients would like more clarity on how their PED is being utilized.

In response to Industry's first question, FDA responded that it was considering more positive examples of case studies, stating that the Agency was open to discussing what types of case studies would be beneficial to Industry. FDA clarified that PED is currently used in the review process and that the checklist exists, reiterating that the Agency's main challenge is that it is difficult to identify what and where PED is in sponsor submissions. FDA stated it acknowledged that Industry expressed that there were limitations with the checklist in its proposal. FDA presented the position that it is open to documenting the use of PED in regulatory decisions and labeling but expressed concern that there would be added time at the end of the review cycle to outline limitations of PED evidence as opposed to outlining the data that supports the Agency's approval. FDA and Industry agreed to continue discussing Industry's proposal at a subsequent negotiation.

FDA Proposed Change to Streamlining Review of Certain Efficacy Supplements Proposal

FDA proposed removing its Streamlining Review of Certain Efficacy Supplements proposal from the negotiating table, citing concerns with meeting both sides' goal of concluding negotiations in March and the number of proposals within the premarket subgroup. FDA also stated that the Agency currently has the authority to streamline review of efficacy supplements that meet certain criteria under Section 3031 of the Cures Act and that Agency does not need a user fee commitment to do so. Industry agreed to consider FDA's proposal and discuss their position at a subsequent meeting.

Industry Counterproposal for Facilitating First Cycle Reviews Subproposal

Industry proposed prioritizing certain Investigational New Drug (IND) protocols to obtain tracking metrics that ensure Industry receives more timely feedback on critical protocols, stating that these tracking metrics compared to baseline provide an opportunity to course correct sooner than a third-party assessment. Industry agreed to share the counterproposal in writing with FDA for discussion at a subsequent meeting.

Next Steps

The goals for the next meeting on December 16th will be to discuss Industry's response to FDA's Rare Disease proposal and continue discussing Industry's Enhancing Transparency and Consistency Related to PED proposal, Industry's Improve FDA-Sponsor Interactions proposal, as well as FDA's Meetings Management proposal.