Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Premarket Subgroup

March 5, 2026 | 10:30 am–12:30 pm

FDA White Oak Campus, Silver Spring, MD

MEETING PURPOSE

To discuss the Rare Disease, Incorporate Regulatory Science into Regulatory Decision-Making, and Facilitate First Cycle Reviews 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	Steve Berman	BIO
Irene Chan	CDER	Rob Berlin	BIO (Vertex)
Emily Ewing	CDER	Kelly Goldberg	PhRMA
Sonday Kelly	CDER	Kristy Lupejkis	PhRMA
Andrew Kish	CDER	Alison Maloney	PhRMA (Bayer)
Phillip Kurs	CBER	Adora Ndu	BIO (Bridge Bio)
Mark Levenson	CDER	Katrin Rupalla	PhRMA (J&J)
Janet Maynard	CDER	Drew Sansone	BIO (Alkermes)
Jennifer Mercier	CDER	Derek Scholes	BIO
Paul Phillips	CDER	Lucy Vereshchagina	PhRMA
Katie Rivers	CBER		
John Scott	CBER		

MEETING SUMMARY

Industry proposed revisions to FDA's Rare Disease Endpoint Advancement (RDEA) program draft commitment letter language. FDA and Industry each presented counterproposals for the Incorporate Regulatory Science into Regulatory Decision-Making action package checklist subproposal. FDA and Industry discussed the scope of the third-party assessment.

Approach to Draft Commitment Letter Language

Industry proposed revisions to the RDEA draft commitment letter language. FDA and Industry discussed the revisions. FDA agreed to respond to the draft commitment letter language in a future meeting.

Industry confirmed it agreed with FDA's revisions to the Facilitate First Cycle Reviews pivotal protocols draft commitment letter language, after FDA made a minor revision in response to discussion at the March 3rd meeting.

Approach to Incorporate Regulatory Science into Regulatory Decision-Making Proposal

Industry presented an overview of the entire Incorporate Regulatory Science into Regulatory Decision-Making proposal, focusing on the problem statement, proposed solutions to the problem, and the proposal's negotiation history. Industry noted in its problem statement that as pilots and programs for which PDUFA resources have been provided conclude, FDA and Industry must work to ensure that learnings from these experiences are implemented consistently across all products, disease areas, and review divisions.[1] Industry also stated that FDA must also seek additional transparency to facilitate shared learning that will strengthen sponsor submissions and encourage consistency and predictability in FDA review.

Industry proposed translating all regulatory science pilots into standard FDA regulatory review practice to consistently implement innovative approaches, an action package checklist, narrative summary, and aggregate regular performance reporting analyzing how and where various innovative tools were proposed and reviewed.[2] Industry removed the narrative summary and aggregate performance reporting subproposals due to FDA concerns about workload. Industry modified its action package checklist proposal, noting that its counterproposal presented at the March 3rd meeting suggested that sponsors would have the option to pre-populate the checklist in the application and FDA would not be required to provide feedback but would include this checklist in the action package. Industry reiterated that it found value in the proposal because it could inform a broader variety of sponsors, patient groups and stakeholders regarding how regulatory science tools were being used.

FDA responded to Industry's overview, stating that the while it agreed with regulatory innovation and getting safe and effective drugs to market sooner, it did not believe the action package checklist would support these goals. FDA noted that the PDUFA pilots and programs had

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[1] Industry also noted that the current action packages do not consistently discuss the assessment of all regulatory science tools included in the submission, and that while publishing best practice examples is useful, it does not allow an assessment of the frequency of use, regulatory impact, and context in which regulatory science tools are used.

[2] See the January 27th meeting summary for details on the action package checklist, narrative summary, and aggregate regular performance reporting subproposals. See the November 20th, and December 4th summaries for details on Industry's subproposal on translating all regulatory science pilots into standard FDA regulatory review practice.

not concluded, particularly those that were initiated under PDUFA VII such as the Rare Disease Endpoint Advancement (RDEA) Pilot Program. FDA stated that while it was open to additional transparency efforts, some regulatory science tools[3] submitted are not fit-for-purpose or of poor quality, making them challenging to evaluate in the regulatory review process. FDA also stated that submission of a checklist cannot be required of all sponsors and those who do decide to complete the checklist may not be consistent in type or quality of regulatory science tool. In addition, poor quality submissions may result in a complete response and important lessons from these programs would not be available publicly. As such, the information gathered from the checklist may be biased and lead to a wrong conclusion. While FDA agreed there is value in shared learnings to advance appropriate regulatory science tools to expedite drug development, FDA did not agree that sponsor submission of a pre-populated checklist would promote shared learning. Industry responded that they still see the value in this proposal, which is higher than any potential biases and would help with uptake of the regulatory science tools.

FDA presented a counterproposal to hold a public meeting focused on discussing best practices for communicating the use of regulatory science approaches in drug development and regulatory decision making. FDA stated it would use the input on communication methods and practices from the public meeting as appropriate to enhance communication and transparency regarding the use of regulatory science tools in the drug development regulatory decision-making process.

Industry asked clarifying questions about how FDA's counterproposal addressed the problems Industry identified. FDA was also open to including research questions regarding best practices for the use of regulatory science tools in regulatory decision-making as part of a third-party assessment. Industry agreed to review FDA's proposal and provide a detailed response in a future meeting.

Approach to Facilitate First Cycle Reviews Proposal

Industry presented a counterproposal to the redacted action packages subproposal. Industry proposed posting redacted action packages for priority efficacy supplements within 6 months after the approval date with an accompanying resource request. FDA did not accept Industry's counterproposal. After further discussion, FDA and Industry agreed that both parties had reached an impasse for the subproposal and agreed to remove it from negotiations.

FDA and Industry discussed the proposed scope for the third-party assessment. FDA and Industry agreed that the scope of the third-party assessment would include New Molecular Entity (NME) New Drug Applications (NDAs) and original Biologics License Applications (BLAs). Industry also proposed that the timing of FDA labeling comments on efficacy supplements be included in the assessment. FDA and Industry both agreed to propose draft commitment letter language

[3] Regulatory science tools include novel endpoint development, Real-World Evidence (RWE), Drug Development Tools (DDT), including the use of biomarkers, Model-Informed Drug Development (MIDD), Complex Innovative Design (CID), Digital Health Technologies (DHTs), and Selective Safety Data Collection (SSDC).

regarding the scope of labeling comments in the assessment. FDA confirmed it would draft commitment letter language for the third-party assessment for discussion at a future meeting.

Next Steps

The goals for the next meeting on March 12th will be to discuss the Incorporate Regulatory Science into Regulatory Decision-Making and Facilitate First Cycle Reviews proposals.