

Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

November 18th, 2020 | 1:00pm-4:00pm

Virtual Format (Zoom)

PURPOSE

To continue discussion about FDA and Industry pre-market review process enhancement proposals.

PARTICIPANTS

FDA

Chris Joneckis	CBER
Ted Liazos	CDER
Alex May	CDER
Lubna Merchant	CDER
Mike Pacanowski	CDER
J. Paul Phillips	CDER
Carolina Reese	CDER
Khushboo Sharma	CDER
Jim Smith	CDER
Peter Stein	CDER
Mary Thanh Hai	CDER

Industry

E. Cartier Esham	BIO
Brad Glasscock	BIO (BioMarin)
Kelly Goldberg	PhRMA
Mathias Hukkelhoven	PhRMA (BMS)
Heidi Marchand	BIO (Gilead and Kite)
Mark Taisey	PhRMA (Amgen)

At the eighth meeting of the PDUFA VII pre-market subgroup, FDA and Industry continued discussions about FDA and Industry proposals to enhance the review process.

FDA/Sponsor Interactions (Meeting Management)

FDA and Industry continued discussions about proposals for enhanced interactions between the Agency and Sponsors for certain types of product development programs. FDA provided detailed feedback on Industry’s proposal to establish and communicate best practices related to PDUFA meeting management. Both sides addressed clarifying questions as FDA discussed the feasibility of addressing aspects of the proposal with ongoing and planned internal process improvement and modernization initiatives. Next, Industry provided feedback on FDA’s counterproposal presented at a previous negotiation session to formalize CBER’s INTERACT program and establish a similar program in CDER, focusing on program scope and related commitments. Industry also provided feedback on FDA’s counterproposal presented at a previous negotiation session for a novel type of formal meeting, focusing on process timelines.

Bioinformatics Review Expertise

FDA and Industry continued discussions about a proposal to enhance CBER and CDER's expertise in various aspects of bioinformatics to support the Agency's ability to provide detailed and consistently timed feedback to Industry earlier in the development cycle. Industry referenced an outstanding question about the appropriateness of requested resources.

Use-Related Risk Analysis (URRA) and Human Factor (HF) Protocol Review

FDA and Industry continued discussions about a proposal to enhance the review of HF protocols and URRAs submitted by Sponsors, especially during combination product development programs. Industry asked clarifying questions about FDA's proposal for resources potentially needed to establish a PDUFA goal for URRA review timelines in addition to a proposal to adjust HF protocol review timelines in response to increasing submission complexity and process challenges.

NME Milestones and PMRs/PMCs

FDA and Industry continued discussions about a proposal to decrease the incidence of late-stage negotiations on PMRs/PMCs during the marketing application review process. Both sides discussed and asked clarifying questions about specific challenges associated with different types of postmarketing studies, including those required by Sec. 505(o)(3) and the Pediatric Research Equity Act (PREA).

Medical Product Information

FDA and Industry continued discussions about a proposal to enhance the accessibility of FDA-approved medical product information for patients and healthcare providers. Both sides discussed the potential value and feasibility of identifying a subset of labeling sections and subsections to enhance with input from public stakeholders.

Real World Evidence (RWE)

FDA and Industry continued discussions about a proposal for a RWE pilot program to increase the use of Real World Data (RWD) during the review of applications and in regulatory decision-making. FDA asked clarifying questions about the proposed pilot program's scope, objectives, and processes, and Industry agreed to provide more detailed responses in writing.

Advancing Development of Efficacy Endpoints for Rare Disease

FDA and Industry continued discussions about a pilot program that would provide additional interaction between the Agency and Sponsors to facilitate the development of rare disease novel endpoints. Both sides discussed the possibility of including a limited number of common disease programs with innovative endpoints that have applicability to rare diseases.

There were no other substantive proposals, significant controversies, or differences of opinion discussed at this meeting.