Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

November 12th, 2020 | 11:30pm-1:00pm

Virtual Format (Zoom)

PURPOSE

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

PARTICIPANTS

FDA		Industry	
Bob Ball	CDER	E. Cartier Esham	BIO
John Concato	CDER	Brad Glasscock	BIO (BioMarin)
Chris Joneckis	CBER	Kelly Goldberg	PhRMA
Ted Liazos	CDER	Mathias Hukkelhoven	PhRMA (BMS)
Alex May	CDER	Heidi Marchand	BIO (Gilead and Kite)
Lubna Merchant	CDER	Mark Taisey	PhRMA (Amgen)
Mike Pacanowski	CDER		
J. Paul Phillips	CDER		
Marilyn Pitts	CDER		
Carolina Reese	CDER		
Khushboo Sharma	CDER		
Jim Smith	CDER		
Peter Stein	CDER		
Mary Thanh Hai	CDER		
Kathy Weil	CDER		

At the seventh meeting of the PDUFA VII pre-market subgroup, FDA and Industry continued discussions about FDA and Industry proposals to enhance the review process. After addressing each topic noted below, both sides agreed to further exploration of each proposal and preparation of responses to questions raised.

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. Industry provided feedback and suggested modifications in response to FDA's proposed program structure discussed at a previous negotiation session. Both sides discussed various aspects of the proposed pilot program, including the number of RWE proposals to accept annually, possible infrastructure for a centralized RWE Committee, and public stakeholder engagement opportunities.

FDA noted that the pilot program would include representation and support from both CDER and CBER.

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 potential benefits and feasibility of expanding the scope of the proposed pilot program to include endpoint development for common diseases with small patient populations in addition to rare diseases.

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. FDA discussed the challenges of using the capacity planning adjustor to provide up-front resource needs to accommodate URRA review timelines.

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 noted by Industry with currently available mechanisms for discussing the feasibility and potential release or reissue of PMRs.

FDA/Sponsor Interactions (Meeting Management)
FDA and Industry continued discussions about proposals for enhanced interactions between FDA and Sponsors for certain types of product development programs. FDA discussed thoughts on Industry's proposal to expand the scope of and expedite timelines within CBER's INTERACT program and establish a similar program in CDER. FDA noted the estimated resources required to enable such enhancements for both CBER and CDER and discussed clarifying questions with Industry. The Agency also provided feedback about Industry's detailed proposal for a novel formal meeting type discussed at a previous negotiation session and discussed potential resources required to implement the additional meeting mechanism.

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