Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

December 2nd, 2020 | 3:00pm-6:00pm

Virtual Format (Zoom)

PURPOSE

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

PARTICIPANTS

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

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

Innovative Review Approaches

FDA and Industry continued discussions about a proposal to enhance the efficiency of efficacy supplement review in order to expedite patient access to treatments that may demonstrate a substantial improvement over available therapies. FDA discussed a counterproposal to Industry's original proposal to expand the scope of FDA's Real-Time Oncology Review (RTOR) pilot program to additional product types and review disciplines. The Agency presented a proposed pilot program framework for splitting the submission of required sections of a marketing application and discussed application package parameters, review timelines, and an internal mid-program assessment. FDA also noted that the proposed pilot program would be adopted by both CDER and CBER.

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 revisions to HF protocol review timelines established during PDUFA VI, proposing

to extend the review timeline from 60 days to 75 days. FDA noted that due to the increasing complexity of HF protocol submissions, continuing to meet the 60-day timelines established during PDUFA VI would not be feasible, even with increased resources.

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 addressed clarifying questions about the Agency's counterproposals for establishing and communicating best practices related to PDUFA meeting management, establishing a novel type of formal meeting, and formalizing CBER's INTERACT program while establishing a similar program in CDER. FDA provided additional details about a proposal to conduct a combined public workshop during PDUFA VII focused on lessons learned and best practices to improve PDUFA formal meeting interactions.

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 and potentially a limited number of common disease programs with innovative endpoints that have applicability to rare diseases. Industry asked about the potential impact on the scope and output of the pilot program depending on the resources available. FDA offered to consider if the program could be scoped to reduce the resource need.

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. Industry noted that while FDA's counterproposal to identify a subset of labeling sections and subsections to enhance with input from public stakeholders might have potential value, such an initiative should occur outside of PDUFA. Both sides agreed to terminate discussion of this proposal in the context of PDUFA VII negotiations.

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 requested a detailed breakdown of resources, and FDA agreed to provide additional details about potential resource allocation.

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