Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

January 21st, 2021 | 4:30pm-6:00pm

Virtual Format (Zoom)

PURPOSE

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

PARTICIPANTS

FDA		Industry	
John Concato	CDER	E. Cartier Esham	BIO
Chris Joneckis	CBER	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)
Rey Perrin	CDER	Mark Taisey	PhRMA (Amgen)
J. Paul Phillips	CDER		
Khushboo Sharma	CDER		
Jim Smith	CDER		
Peter Stein	CDER		
Mary Thanh Hai	CDER		

At the fourteenth meeting of the PDUFA VII pre-market subgroup, FDA and Industry continued discussions about FDA and Industry proposals to enhance the review process. Both sides acknowledged the need to continue discussions about overall proposal resource requests at a subsequent negotiation meeting.

Advancing Development of Efficacy Endpoints for Rare Disease

FDA and Industry briefly discussed the Agency's resource request for a proposed pilot program that would provide additional interaction between FDA 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. FDA noted that commitment language for the pilot program could include information describing the process and criteria for endpoint selection. Industry will consider proposed resource requests presented by the Agency at previous negotiation meetings and provide feedback.

FDA/Sponsor Interactions (Meeting Management)

FDA and Industry discussed aspects of draft commitment language for establishing and communicating best practices related to PDUFA meeting management, establishing a novel formal meeting type, and formalizing CBER's INTERACT program while establishing a similar program in CDER. Both sides discussed potential language describing the scope and objectives for the

proposed novel formal meeting, and FDA clarified that preliminary comments provided by the Agency ahead of INTERACT meetings are expected to eliminate the need for separate formal meeting minutes. Both sides discussed the possibility of sharing metrics related to all PDUFA meeting types, including the new formal meeting type and INTERACT, at a public workshop during PDUFA VII. FDA discussed a counterproposal to the potential follow-up opportunity originally proposed by Industry, suggesting timelines for Sponsors to submit questions following the receipt of meeting minutes or a WRO and for the Agency to respond to questions received, and Industry noted preliminary alignment.

Real World Evidence (RWE)
FDA and Industry discussed aspects of draft commitment language for advancing RWE by establishing a pilot program to develop new methods and knowledge for Real World Data (RWD) to be used in regulatory decision-making, including the review of applications. Both sides discussed details related to the proposed program's scope, objectives, and outputs, including a potential public workshop.

Use-Related Risk Analysis (URRA) and Human Factor (HF) Protocol Review
FDA and Industry briefly discussed the Agency's resource request for a proposal to enhance the review of HF protocols and URRAs submitted by Sponsors, especially during combination product development programs. Industry noted preliminary alignment with FDA's counterproposal requesting additional resources to maintain the 60-day review timeline for HF protocols established during PDUFA VI.

Bioinformatics Review Expertise
FDA and Industry briefly discussed aspects of draft commitment language for enhancing 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. Both sides discussed details related to guidance documents potentially to be developed by FDA and the opportunities to harmonize standards and methodologies related to bioinformatics internationally.

Innovative Review Approaches: Split Real-Time Application Review (STAR)
FDA and Industry discussed aspects of draft commitment language for allowing the split submission and review of certain sections of eligible efficacy supplements for product types in all therapeutic areas. Both sides discussed potential criteria, components, and process details for application submissions to be reviewed under STAR.

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