Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

January 27th, 2021 | 2:00pm-4:00pm

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 fifteenth 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 and commitments.

NME Milestones and Postmarketing Requirements (PMRs)

FDA and Industry discussed aspects of draft commitment language for establishing formal timelines for the communication of anticipated PMRs to Sponsors and for establishing a process for the post-approval review of existing PMRs. FDA clarified that the pre-approval communication timelines discussed for standard and priority NME NDAs and original BLAs, if implemented, would be formal commitments under PDUFA VII rather than suggested best practices. Both sides discussed aspects of the process for reviewing existing PMRs post-approval, including the feasibility of proposed timelines for providing decisions to Sponsors following a request for PMR release and information to be included in the decisional letter from FDA.

Advancing Development of Efficacy Endpoints for Rare Disease

FDA and Industry briefly discussed the Agency's resource request and aspects of draft commitment language 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. Industry noted general alignment with FDA's reduced resource request which would correspondingly reduce the program's outputs. FDA clarified that any public workshops conducted under the program would most likely be collaborative efforts between CDER and CBER.

Use-Related Risk Analysis (URRA) and Human Factor (HF) Protocol Review
FDA and Industry discussed aspects of draft commitment language for enhancing the review of HF protocols and URRAs submitted by Sponsors, especially during combination product development programs. The Agency clarified that HF protocols are evaluated without an accompanying URRA and referenced currently available guidance for industry. Industry discussed the potential need for further guidance to be developed during PDUFA VII.

Innovative Review Approaches: Split Real-Time Application Review (STAR)
FDA and Industry briefly discussed revisions to 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.

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