Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Pre-market subgroup | Meeting Summary

January 6, 2021 | 1:00pm-4:00pm

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
Alex May	CDER	Brad Glasscock	BIO (BioMarin)
Lubna Merchant	CDER	Kelly Goldberg	PhRMA
Mike Pacanowski	CDER	Mathias Hukkelhoven	PhRMA (BMS)
Rey Perrin	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 twelfth 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.

FDA/Sponsor Interactions (Meeting Management)

FDA and Industry briefly discussed the Agency's resource request 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. Industry noted preliminary general alignment with FDA's resource request.

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 requested resources for the review of URRA. Industry asked whether FDA's requested resources for the review of HF protocols were necessary to continue meeting the 60-day timeline established during PDUFA VI and proposed a reduced resource package for the Agency's consideration. FDA referenced FY 2020 performance metrics indicating that preliminary information indicates the Agency did not meet the 90% performance goal for 60-day HF protocol review timelines and noted that in the current year

(FY 2021) indicators show the Agency is also not on track to meeting the 90% performance goal. FDA believes that difficulty meeting the current timeline will likely increase throughout PDUFA VII, especially without additional resources.

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. Industry agreed with the value of potentially establishing a standard process to facilitate endpoint development, and FDA agreed to consider and propose a further reduction of requested resources and a corollary reduction of program scope and outputs, including the number of public workshops and the number of endpoint development programs accepted per year.

Bioinformatics Review Expertise
FDA and Industry briefly discussed the Agency's resource request for 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 noted preliminary general alignment with FDA's resource request.

Real World Evidence (RWE)
FDA and Industry continued discussions about a proposal 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. FDA provided additional details about the methodology used to estimate requested resources and agreed to provide further clarification about resource allocation between CDER and CBER to Industry. Industry asked clarifying questions about the number of proposals that would be accepted into the program per biannual cycle and per year, and FDA agreed to consider how to address possible instances where the number of received proposals deemed eligible for the program exceeds the limit for a given cycle.

NME Milestones and Postmarketing Requirements (PMRs)
FDA and Industry continued discussions about a proposal to establish formal timelines for the communication of intended PMRs to Sponsors and improve timely notification of postmarketing requirements during the marketing application review process. Industry asked clarifying questions about FDA's resource request, including the allocation of resources between CDER and CBER. The Agency noted that the need to establish a Clinical Data Scientist (CDS) program in CBER to enable earlier communication of PMRs versus the expansion of an existing CDS program in CDER. FDA also explained that while the Agency currently evaluates potential safety concerns and issues postmarketing requirements to Sponsors per statutory requirements, committing to consistent earlier evaluation and communication of PMRs would require additional resources to ensure review team capacity during periods of higher workload.

Both sides discussed potential draft commitments related to the proposal and addressed clarifying questions. Industry noted general alignment with FDA's adjusted proposed PMR communication timelines. FDA also noted that Industry's concerns about late-cycle determination of ARIA sufficiency could potentially be addressed by the proposed commitment to revise all MaPPs, SOPPs,

and relevant public documents related to postmarketing studies. FDA agreed to consider Industry's feedback about draft commitment language and discuss updates at a subsequent negotiation session.

Innovative Review Approaches: Split Real-Time Application Review (STAR)
FDA and Industry continued discussions about the proposed STAR pilot program that would allow the split submission and review of certain sections of eligible efficacy supplements for product types in all therapeutic areas. Industry asked whether STAR would replace or expand Oncology's Real-Time Oncology Review (RTOR) pilot program. FDA clarified that STAR was a PDUFA pilot program with unique criteria and goals; however, it was intended to introduce certain aspects and benefits of the RTOR to products in all therapeutic areas. The Agency reminded Industry that RTOR is not a PDUFA program, does not have performance goals, and indicated that RTOR would not be impacted by the STAR proposal. Industry and FDA discussed questions about proposed criteria for participation in STAR. FDA agreed to consider Industry's feedback about potential draft commitment language and discuss updates at a subsequent negotiation session.

In addition, FDA and Industry briefly discussed the Agency's resource request for the STAR proposal. Industry noted preliminary general alignment with FDA's resource request.

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