Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry CBER Breakout Subgroup | Meeting Summary

October 27th, 2020 | 10:00am-12:00pm

Virtual Format (Zoom)

PURPOSE

To discuss FDA and industry CBER specific enhancement proposals.

PARTICIPANTS

FDA		Industry	
Rachael Anatol	CBER	E. Cartier Esham	BIO
Angela Granum	CBER	Brad Glasscock (Lead)	BIO (BioMarin)
Chris Joneckis (FDA Lead)	CBER	Mathias Hukkelhoven	PhRMA (BMS)
Bharat Khanna	CDER	Robert Kowalski (Co-Lead)	PhRMA (Novartis)
Erik Laughner	CBER	Heidi Marchand	BIO (Gilead and Kite)
Carol Rehkopf	CBER	Lucy Vereshchagina	PhRMA

The PDUFA VII CBER Breakout subgroup discussion focused on three draft proposals from Industry which included draft timelines and deliverables from FDA.

Patient Perspectives on Gene Therapies

FDA and Industry discussed a proposal for a patient focused drug development meeting to better understand patient and caregiver's perspectives on gene therapy products.

Clarifying Expedited Pathway Evidentiary Standards

FDA and Industry discussed a proposal on clarifying expedited pathway evidentiary standards through workshops and potential guidance. There was also discussion on the use of real-world evidence and challenges in drug development for smaller patient populations.

Leveraging Internal Prior Knowledge for Review of Gene Therapies

FDA and Industry discussed a proposal for a workshop to discuss how to leverage sponsors' own internal prior knowledge to create more efficient and effective approaches to the development and review of next generation gene therapy products.

FDA and Industry agreed to continue discussion of these specific proposals at future negotiation sessions.

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