Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry CBER Breakout Subgroup | Meeting Summary

October 6th, 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)
Erik Laughner	CBER	Robert Kowalski (Co-Lead)	PhRMA (Novartis)
Darlene Martin	CBER	Heidi Marchand	BIO (Gilead and Kite)
Carol Rehkopf	CBER	Lucy Vereshchagina	PhRMA

The PDUFA VII CBER Breakout subgroup discussion focused on the Patient Perspectives on Gene Therapy Products, CBER's Allergenics proposal, and additional discussion of industry proposals presented at the first meeting related to the Cell and Gene Therapy (CGT) Program and Advanced Biologics in CBER.

Patient Perspectives on Gene Therapy Products

Industry would like FDA to hold a Patient Focused Drug Development (PFDD) meeting to better understand patient perspectives on taking gene therapy products and the need for specific tools or methods to capture patient experience data unique to gene therapy treatments. FDA stated that additional discussion will be needed internally and with industry.

Continued Discussion on Other Industry Proposals

1. RMAT Program Proposals

FDA proposed to update the RMAT guidance and to conduct a review of the past RMAT requests to determine trends in denials versus granted designation and to provide a report to help sponsors considering RMAT designation. Industry stated it would like to see clarification around the use of Real World Evidence (RWE), including as it relates to RMAT and possibly post-approval safety monitoring and patient follow-up.

2. Individualized Therapy and Leveraging Prior Knowledge

Industry presented additional information on leveraging prior internal knowledge for development of CGT products. FDA described its approach to leveraging knowledge for individualized therapies. Industry also wants knowledge leveraged for products with larger patient population and the intent is not to limit to individualized therapy products. FDA and industry agreed additional discussion will continue.

3. CMC Development for CGT Products

Industry will present more specifics on the partial submission of CMC information in a real-time BLA review for CGT products at a future negotiation meeting.

Allergenics

CBER presented its proposal to incorporate certain allergenic extract products into PDUFA by modifying the general exclusion for allergenic extract products in the User Fee Statute. Inclusion of allergenic products would allow the benefits under PDUFA to be applied to newer allergenic extract products.

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