Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry CBER Breakout Subgroup | Meeting Summary

October 20th, 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)
Darlene Martin	CBER	Lucy Vereshchagina	PhRMA
Carol Rehkopf	CBER		

The PDUFA VII CBER Breakout subgroup discussion focused on the Cell and Gene Therapy (CGT) Program in CBER.

CBER Cell and Gene Therapy Program Resource Allocation

CBER provided additional information about the positions needed for the Cell and Gene Therapy Program (CGTP) including positions for support, indirect, and direct review roles. CBER stated that guidance development is part of indirect costs included in the resource request. CBER periodically publishes a list of guidance to indicate what is anticipated for publication in a given year. CBER will discuss the allocation and the sequencing of on-boarding positions requested under this proposal. CBER provided data on the current positions and discussed the approach for balancing resources for example to achieve an optimal ratio of supervisor to staff. Recruiting, hiring, and training of new resources was also discussed and is a priority for CBER. FDA proposed that hiring be phased in with a goal to hire new resources and on-board as quickly as feasible. It takes time for a new reviewer to be trained and experienced to a level of proficiency after on-boarding. CBER will provide estimates for the training needs of existing and new staff.

Additional FDA estimates, including the type and number of forecasted submissions were provided for FY2023. CBER explained that the forecast is conservative and that the FY2020 data used in the forecasting model would be updated. CBER will also refresh the modeling data.

Several clarifying questions from industry were discussed, including staff training, guidance development, and the role of the Oncology Center of Excellence (OCE) medical officers in cell and gene therapy reviews. Challenges with applying the pilot program, Real Time Oncology Review (RTOR), to products reviewed in the CGTP were discussed. CGTP products are widely diverse, highly complex, challenging to manufacture, and FDA's experience with these products is limited. Additional discussions would be needed to explore how review could potentially benefit from this pilot.

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