Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry Negotiation Regulatory Decision Tools Subgroup | Meeting Summary

October 6th, 2020 | 9:00am-11:00am

Virtual Format

PURPOSE

To provide FDA and Industry perspectives on Regulatory Decision Tools enhancements for PDUFA VII (Patient-Focused Drug Development, and to have a follow up discussion on previously discussed topics (Model-Informed Drug Development).

PARTICIPANTS

FDA		Industry	
Robyn Bent	CDER	Rob Blanks	BIO (Ardelyx)
Richard Forshee	CBER	Kristin Dolinski	PhRMA
Rajanikanth Madabushi	CDER	Danielle Friend	BIO
Theresa Mullin	CDER	Carl Garner	PhRMA (Eli Lilly)
Dionne Price	CDER	Kelly Goldberg	PhRMA
Graham Thompson	CDER	Ann Kurowski	BIO (Alkermes)
Julia Tierney	CBER	Mark Taisey	PhRMA (Amgen)

The meeting discussion was focused on the issues of interest to industry and FDA.

FDA & Industry Perspectives on Patient-Focused Drug Development and Model-Informed Drug Development

Due to a schedule conflict, FDA and Industry agreed to move discussion of Complex Innovative Trial Designs to the following meeting, and to use this meeting to discuss Patient-Focused Drug Development (PFDD) and Model-Informed Drug Development (MIDD).

In this meeting FDA discussed the current PFDD program and the progress in meeting PDFUA VI commitments, as well as the lessons learned to date. FDA then discussed its proposals related to PFDD. FDA identified a need to build upon lessons learned from PDUFA VI to further enhance the quality of submitted patient experience data for regulatory decision-making. To address this, FDA proposed enhancements related to sustainability of the PFDD program, standard core sets of Clinical Outcome Assessments (COAs), and patient preference information (PPI) studies.
To increase the sustainability of the PFDD program, FDA proposed focused recruitment for FDA, public workshops to raise awareness and discuss patient-experience related methodological

challenges, building FDA's capacity to review patient experience data through internal training, and expand outreach to raise awareness of the importance of PFDD among industry and other stakeholders.

FDA also proposed expanding on current development of a public repository of standard core COAs for use by industry and other stakeholders, as well as PPI studies.

Industry asked questions related to the current operation of the PFDD program, the current resource and staffing allocations for PFDD, and for more information regarding FDA's PFDD proposals and how they might be implemented.

FDA and Industry also had a follow-up discussion on Model-Informed Drug Development. FDA provided additional details pertaining to the MIDD Pilot Program Selection Committee roster, selection process, internal review, multidisciplinary team engagement and sponsor interactions involved in the MIDD Paired Meeting Pilot. FDA also provided further details regarding its MIDD proposals, including on how the proposals would be implemented.

Plan for Future Meetings

The goals for the next meeting on October 13th will be to have a follow up discussion on potential enhancements related to Model-Informed Drug Development and Patient-Focused Drug Development, as well as to introduce and discuss in more detail potential enhancements related to Complex Innovative Trial Designs.

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