FDA-Industry PDUFA VI Reauthorization Meeting – Regulatory Decision Tools Subgroup
November 4, 2015, 12:30 pm - 2:30 pm
FDA White Oak Campus, Silver Spring, MD
Building 51, Room 1215

Purpose

To discuss in detail FDA and Industry proposals for enhancements to FDA's initiative on the new drug Benefit-Risk Framework

Participants

FDA		Industry	
Sara Eggers	CDER	Beatrice Biebuyck	BIO (Alexion)
Patrick Frey	CDER	Cartier Esham	BIO
Laura Lee Johnson	CDER	Jeffrey Francer	PhRMA
Chris Joneckis	CBER	Sandra Milligan	PhRMA (Merck)
Lisa LaVange	CDER	Paula Rinaldi	PhRMA (Novartis)
Diane Maloney	CBER	Michelle Rohrer	BIO (Roche Genentech)
Theresa Mullin	CDER	Mark Taisey	PhRMA (Amgen)
Mary Parks	CDER		
Mike Pacanowski	CDER		
Graham Thompson	CDER		
Pujita Vaidya	CDER		

Discussion on Benefit-Risk Assessment in New Drug Review

Implementation of FDA's Benefit-Risk Framework

In this meeting, FDA and Industry focused their discussion on potential enhancements to FDA's structured benefit-risk assessment framework initiative. FDA provided an update on the Agency's progress in delivering on the commitments established in PDUFA V Section X focused on Benefit-Risk (B-R) Framework. Updates included: publishing a Draft Implementation Plan (February 2013); revising clinical review and management templates to incorporate the B-R Framework; initiating (CBER – 2013; CDER – 2015) the first phase of implementation of the revised templates in the review of NME NDAs and original BLAs, supported by staff training and coaching; and initiating a project (start of FY 2016), supported be a qualified third party, to evaluate the B-R Framework implementation in the new drug review process.

FDA and Industry then each discussed in more detail their respective proposals related to structured benefit-risk assessment in PDUFA VI. Industry indicated that FDA's progress with the B-R Framework so far in PDUFA V was consistent with the direction that Industry had hoped to see with respect to implementation of structured benefit-risk assessment, and that sponsors would further like to see structured benefit-risk assessment facilitate discussions between a drug sponsor and FDA throughout the drug development lifecycle, particularly early

in drug development when having a clearer understanding of FDA's thinking on potential risks and benefits would help inform a sponsor's internal decision making. FDA commented on the importance of benefit-risk assessment throughout the new drug lifecycle and indicated that the Agency considers the drug lifecycle to span from development in premarketing phases through its use after approved and post-market. FDA and Industry each broadly discussed their perspectives on how the application of the B-R Framework could be advanced through PDUFA VI, such as through updated implementation plans, guidance documents, and integration into FDA Manuals of Policy and Procedures (MAPPs) or Standard Operating Policies and Procedures (SOPPs). FDA and Industry also broadly discussed their perspectives on the best way to evaluate the implementation of the B-R Framework as it continues through PDUFA VI.

Exploring more formal decision-analytic approaches

FDA discussed its proposal to explore the appropriateness of incorporating additional decision-analytic approaches that could inform FDA's regulatory decision-making through the B-R Framework. As part of this proposal, the agency proposed convening at least one workshop to review potential approaches, such as the use of trial endpoints that capture ranked benefit and harm outcomes at the patient-level, and their applicability within FDA's drug regulatory context. Industry indicated that the value in this proposed area of exploration would need to be weighed against other biostatistics-related proposals under discussion.

Plan for Future Meetings

Industry and FDA agreed to plan for a follow up discussion on innovative clinical trials at the next meeting.