FDA-Industry PDUFA VI Reauthorization Meeting – Regulatory Decision Tools Subgroup
October 7, 2015, 12:30am-2:30pm
FDA White Oak Campus, Silver Spring, MD
Building 51, Room 1211

Purpose

To provide FDA and Industry perspectives on Regulatory Decision Tools enhancements (Benefit-Risk and Patient-Focused Drug Development) for PDUFA VI, and to discuss schedule of meetings moving forward.

Participants

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

Discussion of Industry Regulatory Decision Tools Enhancement Proposals

Industry representatives discussed three areas for proposed enhancements in PDUFA VI with respect to drug development toold. The three areas discussed were: 1) Benefit-Risk, 2) Patient Focused Drug Development, and 3) Innovative Trial Designs. Industry recognized that the three areas have overlapping themes.

1. **Proposal for enhancing benefit-risk assessment in regulatory decision making.** Industry proposed that PDUFA VI build on the PDUFA V performance goals for further implementation of a structured benefit-risk framework to inform regulatory decision-making.

2. **Proposal for patient-focused drug development.** Industry proposed building on PDUFA V efforts by holding a public stakeholder workshop that would lead to FDA guidance to address a wide range of challenging methodological and process issues related to advancing the science of patient input.

   FDA agreed that there is a lot of challenging work that needs to be done and stated that there is some overlap between FDA's PFDD and B-R proposals and those discussed by industry.

3. **Proposal for innovative trial designs.** It was agreed to postpone discussion of this topic until the next meeting on October 14, 2015.

Discussion of FDA Regulatory Decision Tools Enhancement Proposals

FDA identified several areas for proposed enhancement of tools in PDUFA VI, including further work on patient-focused drug development, benefit-risk assessment and enhancement of statistical approaches and data standards. In the meeting the agency discussed its proposals related to first two areas.

1. **Proposal for advancing the science of patient input (Patient Focused Drug Development).** FDA identified a need to bridge learnings from PDUFA V patient-focused drug development-type meetings to the development of methodologically sound fit-for-purpose tools to systematically collect key information about patients' experience including the burden of disease, and benefit as well as potential burden of therapy. To address this FDA proposed to use public workshops to develop a series of guidances focusing on recommended approaches including collection of comprehensive patient-community input, impacts that are important to patients, and the measurement of those impacts. FDA noted that the capacity for increasing patient engagement and review work would require increased staffing.

2. **Proposal for benefit-risk assessment in human drug review.** FDA noted that with implementation of the qualitative benefit-risk framework gaining solid ground in the regulatory process, a next phase of possible enhancement could involve exploring more formal decision-analytic approaches to benefit-risk assessment that might add value if integrated into the benefit-risk framework. As part of this work FDA proposed convening a public workshop to review potential approaches and applicability within FDA's drug regulatory context.

Plan for Future Meetings

Industry and FDA agreed to discuss regulatory decision tools enhancement in greater detail in future meetings. Proposals related to innovative trial designs, analysis data standards, and improved subgroup analysis will be topics on the agenda on October 14.