FDA-Industry PDUFA V Reauthorization Meeting
January 31, 2011, 9:30am – 10:30am and 1:30pm – 4:00pm
FDA White Oak Campus, Silver Spring, MD
Building 31, Room 2442

Purpose

To review the current status and progress of Sub-Group discussions.

Participants

FDA		Industry	
Wade Ackerman	OCC	Annetta Beauregard	EMD Serono
Jane Axelrad	CDER	Paul Eisenberg	Amgen
Ed Cox	CDER	Andrew Emmett	BIO
Patrick Frey	CDER	Jeffrey Francer	PhRMA
Debbie Henderson	CDER	Sascha Haverfield	PhRMA
John Jenkins	CDER	Kay Holcombe	Genzyme
Chris Joneckis	CBER	Florence Houn	Celgene
Brian Kehoe	OL	Paul Huckle	GlaxoSmithKline
Theresa Mullin	CDER	Rob Kowalski	Novartis
Donal Parks	CDER	Hilary Malone	Pfizer
Bob Yetter	CBER	Sara Radcliffe	BIO
		Jay Siegel	Johnson & Johnson
HHS		Mark Taisey	Eisai
		Helen Thackray	GlycoMimetics
Roger McClung	ASL	David Wheadon	PhRMA

The FDA-Industry Steering Group reviewed the status of ongoing discussions of the proposed enhancements for PDUFA V in light of the overall reauthorization timeline. FDA stated that sufficient time would be required for the department and administration clearance process before briefing the congressional committees and conducting the public review of the recommendations as required by statute.

FDA noted that Sub-Groups are approaching agreement on proposals for a pilot program for enhanced review transparency and communication involving a late-cycle meeting, REMS, and Sentinel. FDA also noted that discussions continue on the regulatory science proposals (patient-reported outcomes, biomarkers and pharmacogenomics, quality-by-design, and non-inferiority and adaptive trial designs). The agency stated that these emerging scientific areas are increasingly incorporated into sponsors' drug development programs. When the application is submitted, the agency must review this work as part of the human drug review process that is partially funded by PDUFA. FDA stated that this review work has increased throughout PDUFA IV and now exceeds the agency's resources to address these new areas.

Industry reiterated that the review model has been a primary concern, and now that discussions of that proposal have progressed, FDA and Industry have begun discussing the remaining proposals. Industry also agreed that the benefit-risk, meta-analysis, rare disease, REMS, and Sentinel proposals have advanced; but the ability to fund the remaining regulatory science proposals through user fees remained unresolved notwithstanding their potential value. While discussions continue on the proposal to enhance communication with emerging sponsors, FDA and Industry agreed that the ability to reach agreement on this proposal remains uncertain. Industry stated that this proposal to obtain regulatory advice is very

important to smaller companies. FDA noted that it had already developed a counter-proposal with estimated resource requirements, and shared that with industry. Industry and FDA agreed to continue discussing this proposal.

FDA also stated that agreement on a final package of proposed recommendations hinged on a satisfactory conclusion to the Financial Sub-Group discussions of potential changes to the inflation and workload adjusters.

In addition, Industry stated its concern that FDA's small business waiver proposal (reference October 12 minutes) would create too much administrative complexity. FDA stated that this proposal would simply make more explicit that all affiliates, including former affiliates, should be considered when determining whether an application is the first one filed by a small business. In a recent challenge to the agency's interpretation (*Winston Laboratories vs. Kathleen Sebelius and Margaret Hamburg*), a court signaled that only present affiliates could be considered in the analysis. Under such an interpretation, more small business waivers might be granted than were intended under PDUFA because a small company could reincorporate as a new entity, or sever its relationship with another company, before submitting an application, potentially resulting in an application being considered its first even though a former affiliate already filed an application with FDA. FDA stated that explicitly closing this potential loophole is a fairness issue for fee-paying sponsors because a higher number of small business waivers granted results in higher application fees for fee-paying sponsors. Industry restated its belief that firms not currently affiliated with other entities that had received a waiver was the appropriate position to maintain, and that current law on small business affiliation provides sufficient flexibility to address FDA's concerns about small business eligibility through corporate transactions.