

**Stakeholder Meeting on PDUFA VI Reauthorization**  
**October 29, 2015, 10:00 AM – 11:35 AM**  
**FDA White Oak Campus, Silver Spring, MD**

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**Purpose**

To continue discussions of the current status of the human drug and biologic review programs in the context of PDUFA reauthorization. Topics for discussion were based on stakeholder perspectives shared at the July 15, 2015 public meeting and docket submissions.

**Participants**

FDA

April Alexandrow	CDER
Steve Berman	CDER
Amanda Edmonds	OC
Sara Eggers	CDER
John Jenkins	CDER
Chris Joneckis	CBER
Chris Leptak	CDER
Theresa Mullin	CDER
Mary Parks	CDER
Grail Sipes	CDER
Graham Thompson	CDER
Terry Toigo	CDER

Registered Stakeholders

Jeff Allen	Friends of Cancer Research
Kathleen Arntsen	Lupus & Allied Diseases Association
Ryne Carney	Alliance for Aging Research
Brian Fiske	Epilepsy Foundation
Mark Fleury	American Cancer Society Cancer Action Network
Sandra Frear	Lupus & Allied Diseases Association
Kara Gainer	Cure SMA
Eric Gascho	National Health Council
Rob Goldsmith	Cancer Support Community
Lisa Goldstein	American College of Cardiology
Amanda Grimm	Cystic Fibrosis Foundation
Lori Hoffman	Sarcoma Foundation of America
Anna Hyde	Arthritis Foundation
Maureen Japha	FasterCures
Bennie Johnson	JDRF
Kevin Kaiser	American Heart Association
Annie Kennedy	Parent Project Muscular Dystrophy

Ian Kremer	LEAD Coalition (Leaders Engaged on Alzheimer's Disease)
Stephanie Krenrich	Cystic Fibrosis Foundation
Christin L. Engelhardt	National Coalition for Cancer Survivorship
Nick Manetto	FaegreBD Consulting
Amanda Pezalla	American Academy of Dermatology Association
Thair Phillips	RetireSafe
Tracy Rupp	National Center for Health Research
Sarah Sorscher	Public Citizen
Kristin Stephenson	Muscular Dystrophy Association (MDA)
Joseph Stewart	National Alopecia Areata Foundation
Saira Sultan	PANCAN
Timothy Swope	Bipartisan Policy Center
Ernest Voyard	The Leukemia & Lymphoma Society
Patrick Wildman	ALS Association
John Wylam	National MS Society

**Meeting Start Time:** 10:00 AM

The meeting on October 29 focused on providing stakeholders and overview and update on three of the topics of interest to the stakeholders and identified in the previous meeting in September. These topics included an overview of the use of biomarkers in human drug development, an update on the implementation of FDA's benefit risk assessment framework for new drugs, and the FDA's patient-focused drug development program.

**Biomarker Utility and Acceptance in Drug Development and Clinical Trials: an FDA Regulatory Perspective**

FDA provided a brief summary of its regulatory science activities related to biomarkers, described approaches to biomarker use in drug development programs and clinical trials, and outlined opportunities for FDA engagement. After providing a definition of biomarkers based on the FDA/NIH 2015 consensus working group, FDA provided examples of biomarkers and their utility in drug development and clinical experience. FDA explained that useful biomarkers must be fit for purpose and that successful biomarker development requires well-founded ideas, good data, adequate resources, and opportunities for mitigating challenges and collaboration. FDA outlined the modalities for regulatory acceptance of biomarkers through community consensus pathways, drug-specific pathways, and qualification. Finally, FDA described opportunities for stakeholder-FDA engagement including Critical Path Innovation Meetings and Letters of Support.

**FDA's Benefit-Risk Framework**

FDA provided a brief regulatory and historical context on the development of a structured benefit-risk (B-R) framework for new drug review. FDA described the two-fold goals of integrating structured B-R framework into new drug review: to better communicate the reasoning behind regulatory decisions, and to ensure that the "big picture" is kept in mind

throughout a complex and detailed review process. FDA described the framework and its usage in detail and concluded by providing an update on its on-going implementation and evaluation work.

### **FDA's Patient-Focused Drug Development**

FDA described its efforts on enhancing and encouraging patient-focused drug development (PFDD) as outlined in the PDUFA V commitments. Specifically, FDA detailed its progress towards developing a more systematic approach to gather and use patient perspectives on their conditions and available treatments, including progress in conducting the set of disease-specific PFDD meetings during PDUFA V. Each PFDD meeting captures patient input that is published in a Voice of the Patient report, which also serves to support FDA consideration of the clinical context of disease as part of its benefit-risk assessment during new drug review. Future development of PFDD activities at FDA are likely to include efforts to complete FDA commitments for the PDUFA V period including conduct of a total of 24 PFDD meetings in different disease areas. FDA concluded by discussing potential next steps to advance the science of patient input and to develop further guidance on pragmatic and methodologically sound strategies for patient communities, researchers, and drug developers to bridge from the conduct of a PFDD-type meeting to the development of tools to collect the most meaningful measures of patient experience in clinical trials.

### **Question and Answer and Open Discussion**

Following a reminder from FDA about the availability of the docket to collect feedback and proposals from stakeholders, the open discussion session began with a series of clarifying questions and statements on biomarker development and the integration of patient perspective into benefit-risk assessments throughout the medical product development and review lifecycle. Feedback was provided on the difficulty of applying unified standards to heterogeneous populations and varying patient, clinician, and caregiver experiences. Stakeholders identified a dearth of currently-available tools for patient-reported outcome (PROs) specific to diseases of interest and a desire for more and better PRO instruments in the drug development process. Finally, stakeholders communicated that from their perspective, the use of user-fee funding for post-market safety activities including Sentinel would be acceptable, given the importance of this activity and the unlikelihood of increased appropriated funds to support this work.

### **Plan for Next Meeting**

The Stakeholder Meeting on PDUFA VI Reauthorization scheduled for November 16 will focus on post-market safety activities.

**Meeting End Time: 11:35 AM**