Patient-Focused Drug Development Consultation Meeting
November 20, 2013, 3:00 – 4:00 pm
FDA White Oak Campus, Silver Spring, MD
Building 51, Room 1300

Participants

FDA

James Bona	Office of Orphan Product Development (OOPD)
Sara Eggers	Center for Drug Evaluation and Research (CDER)
Ray Ford	CDER
Andrea Furia-Helms	Office of Health and Constituent Affairs (OHCA)
Soujanya Giambone	CDER
Georgiann Ienzi	CDER
Theresa Mullin	CDER
Jordana O'Grady	CDER
Anne Pariser	OOPD
Salina Prasad	OHCA
Sayaka Simmons	OHCA
Ashley Slagle	CDER
Andrea Tan	CDER
Graham Thompson	CDER
Pujita Vaidya	CDER
James Valentine	OHCA
John Whyte	CDER

Patient Stakeholders

Kathleen Arntsen	Lupus Foundation of Mid and Northern New York, Inc.
Ronald Bartek	Friedreich's Ataxia Research Alliance
Cynthia Bens	Alliance for Aging Research / Accelerate Cure / Treatments for Alzheimer's Disease
Lauren Chiarello	National Multiple Sclerosis Society
Lee Claassen	Interstitial Cystitis Association
Mary Cathy Collet	Individual patient stakeholder
Diane Dorman	National Organization for Rare Disorders
Richard Gelula	National Alopecia Areata Foundation
Campbell Hutton	Juvenile Diabetes Research Foundation
Allison Kassir	King & Spalding / Muscular Dystrophy Association
Janet Long	Health and Medicine Counsel of Washington / U.S. Hereditary Angioedema Association
Laurie Markle	Arthritis Foundation
Kimberly McCleary	FasterCures
Martha Nolan	Society for Women's Health Research

Lisa Schlager	FORCE (Facing Our Risk of Cancer)
Jennifer Sheridan	Parkinson's Action Network
Jennifer Spotila	Individual patient stakeholder
Erika Sward	American Lung Association
Lona Vincent	Michael J. Fox Foundation for Parkinson's Disease
Patrick Wildman	ALS Association

Discussion Summary

FDA began the meeting with a presentation on the summary meeting report for the first Patient-Focused Drug Development meeting on chronic fatigue syndrome / myalgic encephalomyelitis (CFS/ME). FDA provided a background on the CFS/ME meeting structure and topics. FDA reiterated that the report is intended to faithfully capture patients' input, in their own words, in a way that is accessible for FDA experts. FDA outlined the structure of the meeting report, detailing how the report integrated the input received during the in-person meeting, the comments received during the meeting webcast, and the comments submitted to the public docket. FDA relayed the feedback received from patients and patient representatives, who have said the report accurately, fairly, and thoroughly reflects the input from the meeting. FDA noted that development of a draft guidance on drug development for CFS/ME is planned, and should be available for public input in Spring 2014.

FDA also provided an update on the most recent Patient-Focused Drug Development meeting on Narcolepsy. FDA stated that the level of participation in the meeting was a record high, despite being the first Patient-Focused meeting on a rare disease. FDA stressed that the tremendous interest and engagement from the patient and advocacy community was crucial in making the meeting such a success.

FDA then asked for patient stakeholder input on: a) how to best balance the perspectives of attendees in the room and on the web, given the limited amount of time for each meeting; and b) ways to prepare panelists and participants for the meetings, to help foster rich and focused dialogue.

Ashley Slagle gave the final presentation on FDA's Study Endpoints and Labeling Development (SEALD) staff and its role in supporting the development and qualification of clinical outcome assessments for drug evaluation. Dr. Slagle provided an overview of the types of outcome assessments, their purpose, and how they can demonstrate treatment benefit. She reiterated the importance of using well-defined and reliable measurements in clinical trials. She discussed the important role of patient-focused outcome measurements in clinical trials, and the challenges and advantages of such measurements. Dr. Slagle concluded with a roadmap that may guide drug developers and others in the development and validation of clinical outcome measures.