

Patient-Focused Drug Development Consultation Meeting

April 19, 2013, 2:00 – 3:30 pm

FDA White Oak Campus, Silver Spring, MD

Building 51, Room 1300

Participants

FDA

Mark Ascione	Center for Drug Evaluation and Research (CDER)
Barbara Buch	Center for Biologics Evaluation and Research
Patrick Frey	CDER
Andrea Furia-Helms	Office of Health and Constituent Affairs (OHCA)
J. Lloyd Johnson	Office of Orphan Drug Products Development
Georgiann Ienzi	CDER
Sonia Kim	CDER
Theresa Mullin	CDER
Anne Pariser	CDER
Andrea Tan	CDER
Graham Thompson	CDER
Pujita Vaidya	CDER
James Valentine	OHCA

Patient Stakeholders

Terrell Baptiste	Health and Medicine Counsel of Washington/Crohn's and Colitis Foundation of America
Theresa Barnes	Coalition for Pulmonary Fibrosis
Cynthia Bens	Alliance for Aging Research/Accelerate Cure/Treatments for Alzheimer's Disease
Sarah Buchanan	Interstitial Cystitis Foundation/Nephcure Foundation
Dane Christiansen	Health and Medicine Counsel of Washington/Pulmonary Hypertension Association
Deanne Clare	The TMJ Association
Mary Cathy Collet	Individual patient stakeholder
Quardricos Driskell	ZERO - The End of Prostate Cancer
Peg Ford	Ovarian Cancer Alliance of San Diego
Eric Gascho	National Health Council
Richard Gelula	National Alopecia Areata Foundation
Carolyn Gondran	Individual patient stakeholder
Joshua Griffis	Pulmonary Hypertension Association
Campbell Hutton	Juvenile Diabetes Research Foundation
Scott Johnson	Veterans with ALS
Annie Kennedy	Muscular Dystrophy Association
Dolly Kervitsky	Pulmonary Fibrosis Foundation
Linda Keyes	Orphan Disease Network

Paul Konanz	Friedreich's Ataxia Parents' Group
Kimberly McCleary	CFIDS Association of America
Mishka Michon	Coalition for Pulmonary Fibrosis
Kristen Mizzi	Children's Cause for Cancer Advocacy
Jonathan Monkemeyer	Hereditary Disease Circle
Thomas Murphy	Individual patient stakeholder
Martha Nolan	Society for Women's Health Research
Jennifer Sheridan	Parkinson's Action Network
Andrew Sperling	National Alliance on Mental Illness
Jennifer Spotila	Individual patient stakeholder
Erika Sward	American Lung Association
Charles Swindell	Sturge-Weber Foundation
Ernest Voyard	Leukemia & Lymphoma Society
Ryan Witt	Society for Participatory Medicine

Discussion Summary

FDA began the meeting with several updates on Patient-Focused Drug Development. The list of Patient-Focused Drug Development public meetings for fiscal years 2013-2015 was published in the Federal Register on April 11, 2013. FDA explained how the disease areas reflect the published criteria, public input received, and perspectives of the FDA review divisions. FDA noted that a second public process would be utilized to determine the meetings for fiscal years 2014-2015. FDA also highlighted positive experiences in planning for the first Patient-Focused Drug Development meeting, on Myalgic Encephalomyelitis and Chronic Fatigue Syndrome (ME and CFS).

The group discussed experiences with online patient communities. FDA discussed online patient communities such as PatientsLikeMe that collect and aggregate patient information by disease. Several patients and patient advocates pointed out that reaching certain patient populations, such as older populations or patients who don't have chronic illnesses, can be challenging. A few patient stakeholders noted concerns about data validation and sample biases, while others commented on the value for patients in tracking and sharing data.

FDA provided a presentation on patient perspectives in drug development, including the new provisions for patient and patient advocate interactions under the Food and Drug Administration Safety and Innovation Act (FDASIA). FDA noted the different options for patient interaction with FDA, including the Patient Representative Program, the Rare Diseases Program, and the expert consultation process. The presentation also highlighted opportunities for patients to become engaged in the drug development process outside of FDA, such as through development of natural history studies.

The final presentation was provided by Kim McCleary, from the CFIDS (Chronic Fatigue and Immune Dysfunction Syndrome) Association of America. She provided an overview of the efforts of the CFIDS Association in engaging with patients and patient stakeholders in advance of the Patient-Focused Drug Development meeting on ME and CFS. The group conducted an open-ended survey that contained the published questions from FDA on symptoms that matter most to patients and patient perspectives on approaches to treatment, in addition to complementary questions on relevant topics. The CFIDS

Association also developed a six-part webinar series on topics such as drug development, FDA's role and mission, effective ways of providing testimony, and information on CFS advocacy and research.