Patient-Focused Drug Development Consultation Meeting
December 11, 2012, 1:00 – 2:00 pm
FDA White Oak Campus, Silver Spring, MD
Building 51, Room 1300

Participants

FDA

Brooklynne Dunbar	Center for Drug Evaluation and Research (CDER)
Sara Eggers	CDER
Forest Ford	CDER
Patrick Frey	CDER
Andrea Furia-Helms	Office of Special Health Issues
Georgiann Ienzi	CDER
Brian Kehoe	Office of Legislation
Theresa Mullin	CDER
Gayatri Rao	Office of Orphan Product Development
Andrea Tan	CDER
Graham Thompson	CDER
James Valentine	Office of Special Health Issues
Robert Yetter	Center for Biologics Evaluation and Research

Patient Stakeholders

Jeff Allen	Friends of Cancer Research
Ronald Bartek	Friedreich's Ataxia Research Alliance
Cynthia Bens	Alliance for Aging Research/Accelerate Cure/Treatments for Alzheimer's Disease
Dane Christiansen	U.S. Hereditary Angioedema Association/Health and Medicine Counsel of Washington
Mary Cathy Collet	Individual patient stakeholder
Diane Dorman	National Organization for Rare Disorders
Edna Fiore	Patient Representative
Ryan Fischer	Parent Project Muscular Dystrophy
Richard Gelula	National Health Council
Steve Gibson	ALS Association
Joshua Griffis	Pulmonary Hypertension Association
Natalie Hamm	American Cancer Society, Cancer Action Network
Lori Hoffman	Sarcoma Foundation of America
Campbell Hutton	Juvenile Diabetes Research Foundation
Scott Johnson	Veterans with ALS
Vicki Kalabokes	National Alopecia Areata Foundation
Allison Kassir	King & Spalding LLP
Jeffrey Kaufman	Adenoid Cystic Carcinoma Research Foundation
Dolly Kervitsky	Pulmonary Fibrosis Foundation

Janet Long	U.S. Hereditary Angioedema Association/Health and Medicine Counsel of Washington
Marjana Marinac	Juvenile Diabetes Research Foundation
Aimee Martin	International Myeloma Foundation
Kimberly McClearly	CFIDS Association of America
Thomas Murphy	Individual patient stakeholder
Kathy Page	Restless Leg Syndrome Foundation
Teri Robert	Alliance for Headache Disorders Advocacy, American Headache and Migraine Association
Stephen Rose	Foundation Fighting Blindness
Brian Rosen	Leukemia & Lymphoma Society
Lisa Schlager	FORCE (Facing Our Risk of Cancer Empowered)
Gary Sherwood	National Alopecia Areata Foundation
Andrew Sperling	National Alliance on Mental Illness
Charles Swindell	Sturge-Weber Foundation
Patrick Wildman	ALS Association

Discussion Summary

FDA opened up the meeting with an update on the docket comment analysis and an overview of the proposed questions for gathering patients' perspective during the Patient-Focused Drug Development meetings. The questions focus on eliciting patient views on the impact of a condition on daily life and current treatment options. The group provided feedback on the structure and wording of the draft questions, and provided suggestions for readability and ease of understanding for patients.

Several patients and patient advocates mentioned that clarity of language was an important issue in gathering useful information and promoting patient understanding. Participants stated that phrases such as "clinical manifestations" are not as consumer-friendly, and that more general terminology would be preferable, such as "symptoms" or "effects." Another suggestion was to include examples for each question.

Participants commented on how to gather input from children, and suggested modifying the questions for this purpose. The group discussed including caregivers, family members, and advocates in the meetings, in addition to patients. Several participants noted that gathering patient input should include perspectives related to the entire lifecycle of the disease.

It was noted that for diseases with few or no approved treatment options, many patients turn to individualized or off-label therapies to mitigate their symptoms, and that FDA should seek to capture this information at the Patient-Focused Drug Development meetings. FDA invited feedback on how to properly phrase questions to capture these individual differences. Some participants stated that it is important to distinguish between treatments used to treat the disease itself and those used to treat symptoms. Another suggestion was providing examples of the different types of treatments, as patients might only associate the word "treatment" with something provided by a doctor or healthcare provider. FDA concluded by thanking the participants for their input and assistance in reframing the questions to better capture the targeted information on the patient perspective.