Stakeholder Meeting on PDUFA VI Reauthorization  
January 15, 2016, 2:00 PM – 3:00 PM  
FDA White Oak Campus, Silver Spring, MD

**Purpose**
To continue discussions of the current status of the human drug and biologic review programs in the context of PDUFA reauthorization.

**Participants**

**FDA**
- Steve Berman  CDER
- Chris Joneckis  CBER
- Theresa Mullin  CDER
- Miranda Raggio  CDER
- Graham Thompson  CDER
- Terry Toigo  CDER

**Registered Stakeholders**
- James Baumberger  American Academy of Pediatrics
- Cynthia Bens  Alliance for Aging Research
- Lauren Bloch  Faegre BD Consulting
- Ryne Carney  Alliance for Aging Research
- Beatriz Duque Long  Epilepsy Foundation
- Mark Fleury  American Cancer Society Cancer Action Network
- Eric Gascho  National Health Council
- Steve Gibson  The ALS Association
- 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
- Annie Kennedy  Parent Project Muscular Dystrophy
- Madeleine Konig  American Heart Association
- Marina Kozak  Friends of Cancer Research
- Ian Kremer  LEAD Coalition
- Jeffrey Last  Alzheimer's Association
- Andrea Lowe  Society for Women's Health Research
- Paul Melmeyer  National Organization for Rare Disorders
- Sarah Mills  DPG - Cure SMA
- Elisabeth Nugent  National Organization for Rare Disorders
- Samantha Roberts  Friends of Cancer Research
- Tracy Rupp  National Center for Health Research
Michael Shea  
Friends of Cancer Research

Travis Smith  
ALS Association

Kristin Stephenson  
Muscular Dystrophy Association (MDA)

Timothy Swope  
Bipartisan Policy Center

Laura Thornhill  
Alzheimer's Association

Ernest Voyard  
The Leukemia & Lymphoma Society

John Wylam  
National MS Society

**Meeting Start Time:** 2:00 PM

The meeting on January 15, 2016 included two presentations by FDA, one an overview of the Breakthrough Therapy Designation Program, and the other an update on the Prescription Drug User Fee Act reauthorization.

**Overview of the Breakthrough Therapy Designation Program**

FDA presented an overview of FDA’s expedited drug development and review programs, focusing on the Breakthrough Therapy Program. Designation as a Breakthrough Therapy expedite both the development and the review of a drug or biologic. Section 506(a) of the Federal Food, Drug, and Cosmetic Act provides for designation of a drug as a breakthrough therapy if the drug: 1) is intended to treat a serious or life threatening disease or condition, and 2) has preliminary clinical evidence that indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. FDA, after noting that CBER follows similar processes as CDER, described CDER’s procedure for the review of Breakthrough Designation Requests and the procedures by which a breakthrough designation speeds drug development and review. FDA reviewed the data of the Breakthrough Program’s usage since 2012, listed examples of its successes in bringing needed drugs to market quickly, and discussed the resource burden incumbent on the program and the Centers.

**PDUFA Reauthorization Update**

FDA provided a brief status update as the reauthorization process as negotiation meetings with industry near completion. The areas identified as major areas for enhancement are: administrative enhancements to ensure the long-term stability of the program (hiring and financial), pre-market review, regulatory decision tools, and post-market safety. FDA provided brief descriptions of the enhancements that have been discussed in each of these areas and reviewed the plans for progressing towards statutory reauthorization.

**Plan for Next Meeting**

The Stakeholder Meeting on PDUFA VI Reauthorization is scheduled for February 19th at 1:30pm.

**Meeting End Time:** 3:00 PM