FDA Rare Disease Day 2020: Supporting the Future of Rare Disease Product Development

Public Meeting
February 24, 2020
FDA White Oak Campus
Building 31, the Great Room

Agenda

8:00-9:00am **Registration**

9:00-9:15am **Welcome**
Janet Maynard, MD, MHS, Director, Office of Orphan Products Development (OOPD), FDA

9:15-9:30am **Opening Remarks**
Amy Abernethy, MD, PhD, Principal Deputy Commissioner and Acting Chief Information Officer, FDA

Morning Session: Strategies to Optimize Registry and Natural History Data to Support Rare Disease Product Development

9:30-10:20am **Panel 1: Discussion with FDA Senior Staff**
Session Goal: Provide perspectives on regulatory considerations related to natural history and registry data

Moderator: Erika Torjusen, MD, MHS, Director of the Rare Pediatric Disease and Humanitarian Use Device Designation Programs and Pediatric Device Consortia Grants Program, OOPD, FDA

Panelists:

- Wilson Bryan, MD, Director, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), FDA
- Daniel Caños, PhD, MPH, Acting Director, Office of Clinical Evidence and Analysis, Office of Product Evaluation and Quality, Center for Devices and Radiological Health (CDRH), FDA
- Peter Stein, MD, Director, Office of New Drugs and Acting Director for Rare Disease Group, Office of New Drugs (OND), Center for Drugs Evaluation and Research (CDER), FDA

10:20-10:35am **Break**

10:35-11:30am **Panel 2: Natural History and Registry Data in Rare Diseases**
Session Goals:
-Outline the importance of collaboration to support successful registries and natural history studies
-Identify common challenges and strategies to address these challenges
-Consider the types of data that are being collected and intended use of the data

Moderator: Theresa Mullin, PhD, Associate Director for Strategic Initiatives, CDER

Panelists:

- Kathleen Donohue, MD, Clinical Team Leader, Division of Gastroenterology and Inborn Errors Products (DGIEP), OND, CDER, FDA
- Jen Farmer, MS, Chief Executive Officer, Friedreich's Ataxia Research Alliance
- Petra Kaufmann, MD, MSc, Vice President R&D, Translational Medicine, AveXis, a Novartis company
- Anne Pariser, MD, Director, Office of Rare Disease Research, National Center for Advancing Translational Sciences, National Institutes of Health
- Klaus Romero, MD, MS, Executive Director Clinical Pharmacology and Quantitative Medicine, Critical Path Institute

11:30am-12:30pm **Lunch**

Afternoon Session: Rare Disease Product Development: New Opportunities and Challenges

12:30-12:50pm **Introductory Remarks for the Afternoon**
Stephen M. Hahn, MD, FDA Commissioner

12:50-1:50pm **Panel 3: Discussion with FDA Center Directors**
Session Goal: Provide perspectives on new challenges and solutions for rare disease product development

Moderator: Janet Maynard, MD, MHS, Director, OOPD, FDA

Panelists:

- Peter Marks, MD, PhD, Director, CBER, FDA
- Jeffrey Shuren, MD, JD, Director, CDRH, FDA
- Janet Woodcock, MD, Director, CDER, FDA

1:50-2:00pm **Break**

2:00-3:00pm **Panel 4: Perspectives on Individualized Therapies**
Session Goal: Provide various perspectives on individualized therapies, with an emphasis on regulatory considerations

Moderator: Maarika Kimbrell, JD, MS, Deputy Director, OND Policy, CDER, FDA

Panelists:

- Ella Balasa, patient with cystic fibrosis and recipient of phage therapy, Virginia Commonwealth University
- Patroula Smpokou, MD, Clinical Team Leader, DGIEP, OND, CDER, FDA
- Julia Vitarello, Founder and CEO, Mila's Miracle Foundation
- Celia Witten, PhD, MD, Deputy Director, CBER, FDA
- Timothy Yu, MD, PhD, Attending Physician, Division of Genetics and Genomics, Assistant Professor in Pediatrics, Harvard Medical School

3:00-4:00pm **Panel 5: Ecosystem of Rare Disease Product Development**
Session Goals:
-Consider the importance of collaboration to support successful strategies in rare disease product development
-Discuss factors and considerations in the ecosystem of rare disease product development

Moderator: Susan McCune, MD, Director, Office of Pediatric Therapeutics, FDA

Panelists:

- Christopher P. Austin, MD, Director, National Center for Advancing Translational Sciences, National Institute of Health
- Martha Donoghue, MD, Clinical Lead, Gastrointestinal Cancer Team, Division of Oncology 3, Office of Oncologic Diseases, FDA
- Sheila Mikhail, JD, MBA, CEO, Co-Founder, AskBio
- Vasum Peiris, MD, MPH, Chief Medical Officer and Director – Pediatrics and Special Populations CDRH, FDA
- Rhiannon Perry, patient with sickle cell disease and lupus

4:00-4:30pm **Open Public Comment Period**

4:30-4:50pm **Closing Remarks**
Janet Maynard, MD, MHS, Director, OOPD, FDA