Draft Agenda for FDA Rare Disease Day 2020: Monday, February 24, 2020 (Great Room, FDA)

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: 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: 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: 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: 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: 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