CONCEPTUALIZING AN FDA RARE DISEASE CENTER OF EXCELLENCE

Hosted by the EveryLife Foundation for Rare Diseases
Thursday, September 13th, 2018
8:30am to 4:30pm
Willard Intercontinental Hotel

8:30 am **Breakfast & Registration**

9:00 am **Welcome & Overview of the Foundation**
Julia Jenkins, Executive Director, EveryLife Foundation for Rare Disease

9:10 am **Community Congress Update & Overview of the Day**
Christina Hartman, MPH, Senior Director of Policy & Advocacy, EveryLife

9:20 am **Perspectives on Progress at the Food and Drug Administration**

- FDA CDER Re-Organization and Impact on Rare Diseases
  Janet Woodcock, MD, Director, FDA, Center for Drug Evaluation and Research (CDER)
- FDA Oncology Center of Excellence Successes
  Amy McKee, MD, Deputy Director, FDA, CDER Oncology Center of Excellence
- Gene Therapy for Rare Diseases
  Wilson Bryan, MD, Associate Director, Division of Clinical Evaluation and Pharmacology/Toxicology, FDA, CBER
- Innovative Approaches for Rare Disease: Cystic Fibrosis Case Study
  Annetta Beauregard, MS, MBA, Vice President, Regulatory Policy & Operations, Vertex

10:35 am **Coffee Break**

10:50 am **Continued Challenges at the FDA: Patient & Academic Perspectives**

- Challenges with Inconsistencies Across FDA Review Divisions
  Isabelle Lousada, CEO, President, Amyloidosis Research Consortium
- Integrating PFDD Data within Regulatory Review: Progress & Existing Opportunities
  Annie Kennedy, Senior Vice President, Legislation & Policy Parent Project Muscular Dystrophy (PPMD)
- The Challenges of Managing Heterogeneity in Rare Diseases
  Emil Kakkis, MD, PhD, Board Member, EveryLife Foundation for Rare Diseases, Founder, CEO, Ultragenyx Pharmaceutical
- Regulatory Challenges for Batten's Disease Treatments
  Ronald Crystal, MD, Chair, Genetic Medicine, Weill Cornell Medicine
- IPEX Syndrome Gene Therapy Case Study
  Rosa Bacchetta, MD, Associate Professor, Pediatric Stem Cell Transplantation and Regenerative Medicine, Stanford School of Medicine

12:05 pm **Lunch**

1:00 pm **Continued Challenges at the FDA: Industry Examples**

- PKU Case Study: Use of Surrogate Endpoints
  Brad Glasscock, Group Vice President, Head of Global Regulatory Affairs at BioMarin Pharmaceutical Inc.
- ALS Case Study: Clinical Trial Designs for Small Patient Populations
  Ralph Kern, MD, MHSc, Chief Operating Officer and Chief Medical Officer Brainstorm Cell Therapeutics
- X-Linked Hypophosphatemia Case Study
  Alison Skrinar, PhD, Executive Director, Clinical Outcomes Research & Evaluation, Ultragenyx Pharmaceutical
- MPS-1 Case Study: Clinical Trial Designs for FDA & EMA Approvals
  Mathias Schmidt, PhD, Chief Executive Officer, ArmaGen
- Gene Therapies for Rare Skin and Connective Tissue Diseases
  Anna Malyala, PhD, Director, Product Development, Fibrocell
- Application of Current FDA Statute to Rare Disease Drug Development: A Fabry Case Study
  Dunni Odumosu, MS Associate Director, Global Regulatory Affairs, Amicus Therapeutics, Inc.

2:30 pm **Break**

2:45 pm **FDA Center of Excellence for Rare Diseases Potential Model**

- Frank Sasinowski, MS, MPH, JD Vice-Chair of the Board, EveryLife Foundation, Director, Hyman, Phelps, & McNamara, P.C.
- **Panel Discussion**
  - Rich Moscicki, MD, Executive Vice President, PhRMA
  - Paul Melmeyer, Director of Federal Policy, NORD
  - Lucas Kempf, MD, Associate Director Rare Diseases Program, FDA
  - Celia Witten, MD, PhD, Deputy Director, FDA CBER
  - Alan Beggs, PhD, Director, The Manton Center for Orphan Disease Research

4:00 pm **Final Discussion & Thoughts on Next Steps**

4:30 pm **Adjourn**

Hosted by

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