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
1401 Pennsylvania Ave NW, Washington, DC 20004

8:30a Breakfast & Registration

9:00a Welcome & Overview of the Foundation by Julia Jenkins, Executive Director, EveryLife (10 min)

9:10a Community Congress Update & Overview of the Day (10 min)

9:20a Perspectives on Progress at the FDA (80 min)
  - FDA CDER Re-Organization and Impact on Rare Diseases
    o Janet Woodcock, MD, Director, CDER (confirmed)
  - FDA Oncology Center of Excellence Successes
    o Richard Pazdur, MD, Director, CDER Oncology Center of Excellence (invited)
  - Gene Therapy for Rare Diseases
    o Wilson Bryan, MD, Associate Director, Division of Clinical Evaluation and Pharmacology/Toxicology, CBER (confirmed)
  - Case Study of Success with Breakthrough Process for Cystic Fibrosis
    o Annetta Beauregard/Scott McGooohan, Vertex (confirmed)

10:40a Coffee Break

11:00a Continued Challenges at the FDA: Patient & Academic Perspectives (60 min)
  - Challenges with Inconsistencies Across FDA Review Divisions
    o Isabelle Lousada, CEO, President, Amyloidosis Research Consortium (confirmed)
  - Duchenne Muscular Dystrophy Case Study
    o Annie Kennedy, Parent Project Muscular Dystrophy (PPMD) (confirmed)
  - Regulatory Challenges for Batten’s Disease Treatments
    o Ronald Crystal, MD, Weill Cornell Medicine (confirmed)
  - The Challenges of Managing Heterogeneity in Rare Diseases (confirmed)
    o Emil Kakkis, Board Member, EveryLife Foundation

12:00p LUNCH
1:00p  Continued Challenges at the FDA: Industry Examples (80 Mins)
   • PKU Case Study: Use of Surrogate Endpoints
     o Brad Glasscock, PharmD, BioMarin (confirmed)
   • ALS Case Study: Clinical Trial Designs for Small Patient Populations
     o Ralph Kern, MD, Brainstorm (confirmed)
   • X-Linked Hypophosphatemia Case Study
     o Ultragenyx Representative (confirmed)
   • MPS-1 Case Study: Clinical Trial Designs for FDA & EMA Approvals
     o Mathias Schmidt, PhD, PD, ArmaGen (confirmed)
   • Fibrocell on Epidermolysis bulosa

2:20p  Coffee Break (30 mins)

3:00p  Presentation & Panel Discussion on Potential Model Center of Excellence for Rare Diseases
   • Moderator Presentation (15 min)
     o Frank Sasinowski, JD, Vice Chair of the Board, EveryLife Foundation
   • Panel Discussion (60 min)
     o Rich Moscicki, MD, Executive Vice President, PhRMA (confirmed)
     o Paul Melmeyer, Director of Federal Policy, NORD (confirmed)
     o Lucas Kempf, MD, Acting Associate Director Rare Diseases Program, FDA (confirmed)
     o Celia Witten, MD, PhD, Deputy Director, FDA CBER (confirmed)
     o Alan Beggs, PhD, Director, The Manton Center for Orphan Disease Research (confirmed)

4:15p  Final Discussion & Thoughts on Next Steps

4:30p  Adjourn