**Science of Small Trials in the Age of Biological Plausibility**

Thursday, September 5, 2019
8:30 a.m. until 4:45 p.m.
Willard InterContinental Washington
1401 Pennsylvania Ave NW, Washington, DC  20004

**AGENDA**

8:30am-8:45am  Welcome & Meeting Overview  Julia Jenkins, Executive Director, EveryLife Foundation

8:45am-9:45am  Regulator Perspectives: A Conversation with FDA Leadership  
**Moderator:** Mark Dant, Board Chairman, EveryLife Foundation  
Janet Woodcock, MD, Director, CDER, FDA  
Peter Marks, MD, Director, CBER, FDA  
Janet Maynard, MD, MHS, Director, Office of Orphan Products and Development, FDA

9:45am-3:30pm  Case Study Sessions  
**Moderator:** James Valentine, JD, MHS, Regulatory Advisor to EveryLife Foundation

9:45am – 11:00am  Case Study Session No. 1: Lack of Natural History  
- Aspa Therapeutics (BridgeBio): Building a retrospective natural history for Canavan Disease, Kathleen Kirby, Head of Development Operations (10-12 min)  
- Audentes Therapeutics: Leveraging Retrospective & Prospective Natural History for X-linked Myotubular Myopathy, Sal Rico, SVP, Clinical Development (10-12 min)  
- Covance: Impact of “Virtual Natural Histories” in Clinical Development for aTTR, Dr. Leone Atkinson, Executive Director, Rare Disease and Pediatrics Team  
- FDA Perspective: Dragos Roman, MD, Associate Director, Division of Gastroenterology and Inborn Error Products, CDER, FDA (10-12 min)  
- Discussion (5-10 min)

11:00am-11:15am  Coffee Break

11:15am – 12:30pm  Case Study Session No. 2: Dearth of FDA-Validated Established Endpoints  
- Genentech (Roche): Early efforts to validate a novel endpoint for Huntington’s disease, Megan Zoschg-Canniere, Global Regulatory Franchise Head, Neurodegeneration & Rare (10-12 min)  
- Casimir: Contextualizing Barth Syndrome Clinical Data with Video Interviews, Mindy Leffler, President (10-12 min)  
- Friedreich’s Ataxia Research Alliance (FARA): Development of multiple measure Friedreich Ataxia Rating Scale (FARS), Ron Bartek, President (10-12 min)  
- FDA Perspective: Wilson Bryan, MD, Director, Office of Tissues and Advanced Therapies, CBER, FDA (10-12 min)  
- Discussion (5-10 min)

12:30 – 1:20pm  Lunch Buffet
1:20pm – 2:35pm  Case Study Session No. 3: Statistical Challenges Related to Small Sample Size
• Palvella Therapeutics: Use of a randomized withdrawal study design in pachyonychia congenita, Wes Kaupinen, CEO (10-12 min)
• Alnylam Pharmaceuticals: Cross-study comparison design for Hereditary Transthyretin Amyloidosis, Andrew Slugg, SVP Regulatory Affairs (10-12 min)
• Parent Project Muscular Dystrophy: Duchenne platform trial, Abby Bronson, Senior Vice President, Research Strategy (10-12 min)
• FDA Perspective: Dr. Peter Stein, Director, Office of New Drugs, CDER and Acting Director, Rare Diseases Program, CDER, FDA (10-12 min)
• Discussion (5-10 min)

2:35-2:50pm  Coffee Break

2:50pm-4:05pm  Case Study Session No. 4: Level of Evidence
• Ultragenyx Pharmaceutical: Multi-Domain Responder Index, Emil Kakkis, CEO (15 min)
• Hyman, Phelps & McNamara, P.C.: Quantum of Effectiveness Evidence Necessary for Orphan Drugs, Frank Sasinowski (15 min)
• Greenwich Biosciences: Leveraging expanded access in safety evaluation, Kathryn Nichol, Senior Director, Medical Affairs (15 minutes)
• FDA Perspective: Dr. Peter Stein, Director, Office of New Drugs, CDER and Acting Director, Rare Diseases Program, CDER, FDA (15 min)
• Discussion (5-10 min)

4:05pm – 4:45pm  Closing Remarks & Path Forward
Steve Silvestri, Director of Public Policy, EveryLife Foundation
Lisa Carlton, Co-Chair, Community Congress Regulatory Working Group

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