Meeting Agenda

EVERYLIFE FOUNDATION FOR RARE DISEASES | Scientific Workshop

Therapy Development for Small Populations: Evidence, Implications, & Policy in Characterizing Ultra-Rare

May 21, 2024 | 9:00 am – 4:00 pm
National Press Club, Washington, DC

9:00 am - 9:15 am: Opening Remarks

Welcome and Goals for the Day: **Jamie Sullivan,** MPH – EveryLife Foundation for Rare Diseases; **Tim Franson**, MD, Principal – Faegre Drinker Consulting

9:15 am – 9:45 am: Perspectives Across the Ecosystem

- Patient Advocacy Group Perspective – **Sharon King**, President – Taylor's Tale
- Industry perspective – **Adora Ndu**, PharmD, JD, Chief Regulatory Officer – BridgeBio
- Regulatory perspective – **Janet Maynard**, MD, MHS, Director – Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicines, FDA

9:45 am – 10:45 am: Panel: Considerations for Characterizing Small Populations

Moderator: Ethan Perlstein, PhD, CEO – Perlara PBC & Maggie's Pearl

- **Tim Franson**, MD, Principal – Faegre Drinker Consulting
- **Aaron Goldenberg**, PhD, MPH, Professor and Vice Chair – Department of Bioethics, Director – Center for Community Health and Genomics, Case Western Reserve University School of Medicine
- **Sharon King**, President – Taylor's Tale
- **Janet Maynard**, MD, MHS, Director – Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicines, FDA
- **Paul Melmeyer**, MPP, Vice President – Muscular Dystrophy Association
- **Adora Ndu**, PharmD, JD, Chief Regulatory Officer – BridgeBio
- **Sal Rais**, MD, MBA, Biotech Analyst – T. Rowe Price

10:45 am – 11:00 am: Break

11:00 am – 12:00 pm: When Data Isn't the Problem: Challenges in Very Small Population Therapy Development

Moderator: Esther Krofah, MPP, Executive Vice President, Milken Institute Health, which includes leadership of FasterCures

- Case Study: Barth Syndrome – **Emily Milligan**, MPH, Executive Director – Barth Syndrome Foundation & **Reenie McCarthy**, JD, Chief Executive Officer – Stealth Biotherapeutics
- Case Study: Neimann-Pick Disease – **Justin Hopkin**, MD, Chairman Emeritus - National Niemann-Pick Disease Foundation
- Common Threads: Logistical and Regulatory Complexities in Very Small Population Therapy Development – **A.J. Allen**, MD, PhD, Chief Medical Officer – I-Act for Children

12:00 pm – 12:45 pm: Lunch

12:45 pm – 2:00 pm: Getting to Approval: Rare Disease Approval Successes and the Lessons That Can Inform a Characterization of Ultra Rare

Moderator: Kishore Hari, Senior Program Manager, Chan Zuckerberg Initiative

- Case Study: Mucopolysaccharidosis Type VII (MPS VII) – **Matthew Ellinwood**, DVM, PhD, Chief Scientific Officer - National MPS Society
- Case Study: SOD1-mediated Amyotrophic Lateral Sclerosis (ALS) – **Teresa Fecteau**, PhD, Director - U.S. Medical for Rare Disease – Biogen
- Case Study: Activated PI3K Delta Syndrome – **Kevin Thorneloe**, PhD, Sr. Medical Director – Pharming & **Lynn Albizo**, JD, Chief Public Policy Officer – Immune Deficiency Foundation
- What can be Learned from Experiences with Larger Rare Diseases – **Frank Sasinowski**, MS, MPH, JD, Director – Hyman, Phelps, & McNamara P.C.; Vice Chair of the Board – EveryLife Foundation for Rare Diseases

2:00 pm – 2:30 pm Room Reflections and Discussion

2:30 pm – 2:45 pm: Break

2:45 pm – 3:45 pm: Leaning into Solutions: Implications for Innovation, Regulatory Practices, and Policy

Moderator: Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement

- **Janet Woodcock**, MD, Former Principal Deputy Commissioner – FDA
- **Peter Marks**, MD, PhD, Director – Center for Biologics Evaluation and Research (CBER), FDA
- **Joni Rutter**, PhD, Director – National Center for Advancing Translational Sciences (NCATS), NIH

3:45 pm – 4:00 pm: Summary Discussion: Characterizing Ultra Rare

Moderator: Tim Franson, MD, Principal – Faegre Drinker Consulting