Preliminary Agenda  
(As of April 5, 2022)

Day 1 — Monday, May 16, 2022

9:00 a.m. ET  Welcoming Remarks  
*Kerry Jo Lee, M.D.*, Associate Director for Rare Diseases, Rare Diseases Team (RDT), Division of Rare Diseases and Medical Genetics (DRDMG), Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)  
*Philip John (P.J.) Brooks, Ph.D.*, Acting Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

9:20 a.m.  Session 1: Adequate and Well-Controlled Trials  
This session will provide an overview of rare disease clinical trial development with an emphasis on the importance and purpose of adequate and well-controlled trials. Topics include: an overview of rare disease clinical trials at CDER, confirmatory evidence in the setting of adequate and well-controlled clinical trials, and the role of translational research.  
**Moderator:** *Sheila Farrell, M.D.*, Medical Officer, DRDMG, ORPURM, OND, CDER, FDA  
**Panelists:**  
- *Janet Maynard, M.D., M.H.S.*, Director, ORPURM, OND, CDER, FDA  
- *Jennifer Rodriguez Pippins, M.D., M.P.H.*, Clinical Advisor, Office of New Drugs – Policy (ONDP), CDER, FDA  
- *Jeff Siegel, M.D.*, Director, Office of Drug Evaluation Sciences (ODES), OND, CDER, FDA

10:35 a.m.  Break

10:45 a.m.  Session 2: Case Studies — An Academic Perspective  
Through the experiences of academic researchers, this session will illustrate challenges related to demonstrating substantial evidence of effectiveness and their approach to the use of translational evidence.  
**Moderator:** TBD  
**Panelist:**  
- *Leslie B. Gordon, M.D., Ph.D.*, Professor of Pediatrics Research, Warren Alpert Medical School of Brown University; Professor, Department of Pediatrics, Hasbro Children's Hospital; Research Associate, Department of Anesthesia, Boston Children's Hospital and Harvard Medical School; Medical Director and Co-Founder, The Progeria Research Foundation

12:00 p.m.  Break
1:00 p.m.  **Session 3: Core Principles for Clinical Trials**
This session will provide information on principles integral to the fundamental design and analyses of rare disease clinical trials to maximize effective use of small populations.
**Moderator:** Katie Donohue, M.D., M.Sc., Director, DRDMG, ORPURM, OND, CDER, FDA
**Panelists:**
- Katie Donohue, M.D., M.Sc., Director, DRDMG, ORPURM, OND, CDER, FDA
- Yan Wang, Ph.D., Statistical Team Leader, Division of Biometrics IV, Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA
- Jie (Jack) Wang, Ph.D., Clinical Pharmacology Team Leader, Division of Translational and Precision Medicine, Office of Clinical Pharmacology (OCP), OTS, CDER, FDA

2:25 p.m.  **Break**

2:35 p.m.  **Session 4: Case Studies — Real World Experiences**
Through the experiences of academic researchers, this session will illustrate the challenges of designing and conducting rare disease clinical trials that are fit from a regulatory perspective. The panel will share lessons learned in the field.
**Moderator:** Tiina K. Urv, Ph.D., Program Director, ORDR, NCATS, NIH
**Panelists:**
- Matthias Kretzler, M.D., Principal Investigator, Nephrotic Syndrome Study Network (NEPTUNE), Rare Diseases Clinical Research Network (RDCRN); Professor, Internal Medicine-Nephrology and Computational Medicine & Bioinformatics, University of Michigan Medical School
- Brendan H.L. Lee, M.D., Ph.D., Principal Investigator, Brittle Bone Disorders Consortium (BBDC), RDCRN; Professor and Chair, Molecular and Human Genetics, Baylor College of Medicine
- Andrea L. Gropman, M.D., Principal Investigator, Urea Cycle Disorders Consortium (UCDC), RDCRN; Professor and Division Chief, Neurodevelopmental Pediatrics and Neurogenetics, Children’s National Hospital

4:00 p.m.  **Adjournment**
Day 2 — Tuesday, May 17, 2022

9:00 a.m. ET  **Session 5: The Nuts and Bolts of Investigational New Drug (IND) Applications and Additional Considerations**
This session will walk through the IND process and how to prepare for each step.  
**Moderator:** Cynthia Welsh, M.D., Medical Officer, Rare Diseases Team (RDT), Division of Rare Diseases and Medical Genetics (DRDMG), Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)  
**Panelists:**
- Mari Suzuki, M.D., Medical Officer, DRDMG, ORPURM, OND, CDER, FDA  
- Margaret Kober, R.Ph., M.P.A., Chief, Project Management Staff, Division of Regulatory Operations for Urology, Obstetrics, and Gynecology, Office of Regulatory Operations (ORO), CDER, FDA  
- Shamir Tuchman, M.D., M.P.H., Medical Officer, Division of Pediatrics and Maternal Health (DPMH), ORPURM, OND, CDER, FDA  
- Arianne Motter, Ph.D., Senior Toxicologist, Office of Infectious Diseases (OID), OND, CDER, FDA

10:45 a.m.  **Break**

11:00 a.m.  **Session 6: Additional Pathways to Interact with the FDA**
This session will share when to engage with the FDA, including a closer look at Critical Path Innovation Meetings (CPIMs) and Patient-Focused Drug Development (PFDD).  
**Speakers:**
- Chekesha Clingman-Henry, Ph.D., M.B.A., Commander, U.S. Public Health Service; Associate Director for Strategic Partnerships, Office of Translational Sciences (OTS), CDER, FDA  
- Robyn Bent, R.N., M.S., Captain, U.S. Public Health Service; Director, CDER PFDD Program, Office of the Center Director (OCD), CDER, FDA

11:45 a.m.  **Closing Remarks**
Kerry Jo Lee, M.D., Associate Director for Rare Diseases, RDT, DRDMG, ORPURM, OND, CDER, FDA  
Philip John (P.J.) Brooks, Ph.D., Acting Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

12:00 p.m.  **Adjournment**