



Final Agenda

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, Division of Rare Diseases Research Innovation (DRDRI), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)
- 9:15 a.m. **Session 1: Approach to Demonstrating Substantial Evidence of Effectiveness for Rare Disease Drug Products**
- The session will provide an overview of the approach to clinical drug development, with an emphasis on designing programs capable of demonstrating substantial evidence of effectiveness. Topics include: the importance of adequate and well-controlled trials in rare disease drug development, the use of confirmatory evidence to substantiate results from a single trial, and the role of translational research in rare disease drug development.
- 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 Drug Policy, OND, CDER, FDA
 - *Jeff Siegel, M.D.*, Director, Office of Drug Evaluation Sciences (ODES), OND, CDER, FDA
- Q&A will follow the presentations.*
- 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:** *Elizabeth A. Ottinger, Ph.D.*, Deputy Director of Programs and Head of Project Management, Therapeutic Development Branch, Division of Preclinical Innovation (DPI), NCATS, NIH
- 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
 - *Raphaella T. Goldbach-Mansky, M.D., M.H.S.*, Senior Investigator and Chief, Translational Autoinflammatory Diseases Section (TADS), Laboratory of Clinical Immunology and Microbiology (LCIM), National Institute of Allergy and Infectious Diseases (NIAID), NIH
 - *Bita Shakoory, M.D.*, Study Coordinator, TADS, LCIM, NIAID, NIH
- Q&A will follow the presentations.*



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:

- *Jie (Jack) Wang, Ph.D.*, Clinical Pharmacology Team Leader, Division of Translational and Precision Medicine, Office of Clinical Pharmacology (OCP), Office of Translational Sciences (OTS), CDER, FDA
- *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), OTS, CDER, FDA

Q&A will follow the presentations.

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 for purpose from a regulatory perspective. The panel will share lessons learned in the field.

Moderator: *Tiina K. Urv, Ph.D.*, Program Director, DRDRI, NCATS, NIH

Panelists:

- *Andrea L. Gropman, M.D.*, Principal Investigator, Urea Cycle Disorders Consortium (UCDC), Rare Diseases Clinical Research Network (RDCRN); Professor and Division Chief, Neurodevelopmental Pediatrics and Neurogenetics, Children's National Hospital
- *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
- *Matthias Kretzler, M.D.*, Principal Investigator, Nephrotic Syndrome Study Network (NEPTUNE), RDCRN; Professor, Internal Medicine-Nephrology and Computational Medicine & Bioinformatics, University of Michigan Medical School

Q&A will follow the presentations.

4:00 p.m. **Adjournment**



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Day 2 — Tuesday, May 17, 2022

- 9:00 a.m. ET **Welcome**
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)
- 9:05 a.m. **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, including special considerations for pediatric studies and basics of preclinical packages.
Moderator: *Cynthia Welsh, M.D.*, Medical Officer, RDT, DRDMG, ORPURM, OND, CDER, 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 L. Motter, Ph.D., DABT*, Senior Toxicologist, Division of Pharmacology and Toxicology for Infectious Diseases (DPTID), Office of Infectious Diseases (OID), OND, CDER, FDA*Q&A will follow the presentations.*
- 10:50 a.m. **Break**
- 11:00 a.m. **Session 6: Additional Pathways to Interact with FDA CDER**
 This session will share when to engage with FDA CDER, 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*Q&A will follow the presentations.*
- 11:50 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, Division of Rare Diseases Research Innovation (DRDRI), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)
- 12:00 p.m. **Adjournment**