FDA-University of Maryland CERSI Public Workshop:

ADEPT 10: Addressing Challenges in Neonatal Product Development – Leveraging Rare Disease Frameworks

Thursday, February 5th, 2026: 1:30 p.m. to 5:00 p.m. EST
Friday, February 6th, 2026: 8:30 a.m. to 4:00 p.m. EST

DAY 1: Thursday, February 5th, 2026

Welcome and Introductory Remarks

1:30 p.m. to 1:40 p.m.	An Massaro, MD *Office of Pediatric Therapeutics (OPT)* *Office of the Chief Medical Officer (OCMO)* *Office of the Commissioner (OC)* *US Food and Drug Administration (FDA)*
1:40 p.m. to 1:50 p.m.	Mallika Mundkur, MD, MPH *OCMO/OC/FDA*

Session 1: Setting the Scene – Neonatal and Rare Disease Drug Development

1:50 p.m. to 2:10 pm	***Neonates and Rare Diseases – Why Early Diagnosis and Approved Treatments are Imperative*** Melissa Lestini, MD *OPT/OCMO/OC, FDA*
2:10 p.m. to 2:30 pm	***Recognizing Common Challenges Across Investigations in Neonates and Rare Diseases Populations*** Marshall Summar, MD *Uncommon Cures, Inc.*
2:30 p.m. to 2:45 p.m.	**BREAK**

Session 2: Ethical Considerations in Neonatal and Rare Disease Clinical Trials

2:45 p.m. to 3:00 p.m.	***Introductory Remarks for Panel Discussions and Moderator*** Dalia Feltman, MD, MA *OPT/OCMO/OC, FDA*
3:00 p.m. to 4:00 p.m.	***Panel A: Weighing benefits and risks when evidence is limited***

Panelists:
Alison Bateman-House PhD, MPH, MA
NYU Grossman School of Medicine

Robert Nelson, MD, PhD
Johnson and Johnson

Allyson Berent, DVM, DACVIM
The Foundation for Angelman Syndrome Therapeutics (FAST)

Elliott Mark Weiss, MD, MSME
Seattle Children's Hospital

Matthew A. Rysavy, MD, PhD
McGovern Medical School at UTHealth Houston

Ashley O'Neil, CRNP
It's a NICU World

4:00 p.m. to 5:00 p.m. ***Panel B: Study design considerations***

Panelists:
Alison Bateman-House PhD, MPH, MA
NYU Grossman School of Medicine

Robert Nelson, MD, PhD
Johnson and Johnson

Lindsey Wahlstrom, MPH, CPH
Rona's FUN LAB

Elliott Mark Weiss, MD, MSME
Seattle Children's Hospital

Matthew A. Rysavy, MD, PhD
McGovern Medical School at UTHealth Houston

Jennifer Degl, MS
NICU Parent Network

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DAY 2: Friday, February 6th, 2026

Session 3: Innovative Strategies for Neonatal and Rare Disease Clinical Trials

8:30 a.m. to 8:35 a.m.	***Welcome*** An Massaro, MD *OPT/OCMO/OC, FDA*
8:35 a.m. to 8:55 a.m.	***Innovative study designs to address small populations*** Rebecca Chiu, PhD *Office of Biostatistics (OB), Center for Drug Evaluation and Research (CDER), FDA*
8:55 a.m. to 9:55 a.m.	***Incorporating novel approaches to facilitate neonatal/rare disease clinical trials*** ***Artificial Intelligence (AI)/ Digital Health Technologies (DHTs) in the NICU- Academic Perspective*** Ryan Michael McAdams, MD *University of Wisconsin School of Medicine and Public Health* ***AI/DHTs in Neonatology/Rare Disease- Regulatory Perspective*** Hussein Ezzeldin, PhD *Office of Medical Policy, CDER, FDA* ***Leveraging Real World Data (RWD) in Neonatology/Rare Diseases*** Kanwaljit Singh, MD, MPH, MBA *International Neonatal Consortium (INC), Critical Path Institute (C-Path)* ***Platform Trials/Master Protocols in Pediatrics*** Danny Benjamin, MD, PhD, MPH *Pediatric Trials Network (PTN), Duke Clinical Research Institute (DCRI)*
9:55 a.m. to 10:15 a.m.	***Selecting Patient-focused Outcomes for Neonatal Conditions and Rare Diseases*** Michelle Campbell, PhD *Office of Neuroscience, CDER, FDA*
10:15 a.m. to 10:30 a.m.	**BREAK**

10:30 a.m. to 12:00 p.m. ***Panel Discussion: Leveraging Networks/Consortia/Centers of Excellence to Optimize Harmonization and Operational Feasibility of Neonatal/Rare Disease Clinical Trials***

Moderator:
Shannon Hamrick, MD
OPT/OCMO/OC, FDA

Panelists:
Rachel G. Greenberg, MD, MB, MHS
PTN, DCRI

Augusto Santos Schmidt MD, PhD
Neonatal Research Network (NRN), Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), National Institutes of Health (NIH)

Klaus Romero, MD, MS, FCP
C-Path

Debra Regier, MD, PhD
RareCap
Levine Children's Hospital, Advocate Health

Ralph Bax, MD, MA
Paediatric Medicines Office, Scientific Evidence Generation Department, Human Division, European Medicines Agency (EMA)

Sarah Zaidi, MD
OPT/OCMO/OC, FDA

12:00 p.m. to 1:00 p.m. **LUNCH BREAK**

Session 4: Regulatory Landscape and Future Directions for Neonatal and Rare Disease Drug Development

1:00 p.m. to 2:00 p.m. ***Panel Discussion: Regulatory Perspective: How can Expedited Programs, Incentive Pathways, and other Regulatory Initiatives be Leveraged for Neonatal and Rare Disease Drug Development?***

Moderator:
Cynthia Rothblum-Oviatt, PhD
Rare Diseases Team (RDT), Division of Rare Diseases and Medical Genetics (DRDMG), CDER, FDA

Panelists:
Amy C. Rick, JD
Rare Disease Innovation Hub, FDA

Erika N. Torjusen, MD, MHS
Office of Orphan Products Development (OOPD), OCMO/OC/FDA

Janet Maynard, MD, MHS
Office of Rare Diseases, Pediatrics, Urology and Reproductive Medicine (ORPURM), CDER, FDA

Najat Bouchkouj, MD
Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER), FDA

Ralph Bax, MD, MA
Paediatric Medicines Office, Scientific Evidence Generation Department, Human Division, EMA

2:00 p.m. to 3:30 p.m. ***Panel Discussion: Stakeholder perspective: Lessons Learned, Gaps and Future Directions***

Moderator:
Gerri Baer, MD
Division of Hepatology and Nutrition (DHN), CDER, FDA

Panelists:
Yuliya Yasinskaya, MD
DRDMG, CDER, FDA

Annapurna Poduri, MD, MPH
Harvard Medical School

Susan McCune, MD
Scendea

Antonello Pileggi, MD, PhD, MSCTI
NICHD, NIH

Ronald J. Bartek
Friedreich's Ataxia Research Alliance (FARA)

Betsy Pilon
Hope for HIE

3:30 p.m. to 3:45 p.m. ***Summary and Closing Remarks***
Lynne Yao, MD
Division of Pediatrics and Maternal Health (DPMH), CDER, FDA