Drug Development Considerations for the Treatment of

Neonatal Enterovirus Infection and Congenital Cytomegalovirus Infection

Virtual Public Workshop

May 7-8, 2024

Day 1 – General Principles of Pediatric and Neonatal Drug Development – Neonatal Enterovirus Infection

Time	Topic	Speaker(s) and Affiliation
9-9:10	Introductory Remarks	Yodit Belew, MD FDA
Session 1. General Principles of Pediatric and Neonatal Drug Development		
9:10-9:25	Ethical Considerations for Pediatric Clinical Trials	Prabha Viswanathan, MD FDA
9:25-9:40	Clinical and Regulatory Considerations for Neonatal Antiviral Drug Development	An Massaro, MD FDA
9:40-9:55	Clinical Pharmacology Considerations for Dose Selection in Pediatric Patients	Kunyi Wu, PharmD FDA
9:55-10:05	Life of a NICU Parent: Decision-making in Clinical Trial Enrollment	Betsy Pilon Hope for HIE
10:05-10:20	Facilitating Neonatal and Pediatric Drug Development: Leveraging Pediatric Trial Networks and Global Collaboration	Yeruk Mulugeta, PharmD FDA
10:20-10:35	Real-world Data and Real-world Evidence in Drug Development	John Concato, MD, MPH FDA
10:35 –11:00	Clarifying Questions and Answers	
11:00 –11:20	Break	
Session 2. Enterovirus Epidemiology and Disease Background		
11:20-11:25	Enterovirus Session Introduction	Natalie Pica, MD, PhD FDA
11:25-11:35	Picornaviruses and Neonatal Sepsis	Amy Rosenfeld, PhD FDA
11:35-11:45	National Surveillance Data on Neonatal Enterovirus Infections in the United States	Miranda Delahoy, PhD CDC
11:45-12:05	Neonatal Enterovirus Infections: Challenges and Opportunities	Mark Abzug, MD University of Colorado
12:05-12:20	Clarifying Questions and Answers	
12:20-1:00	Lunch	
Session 3: Enterovirus Trial Design Challenges		
1:00-1:10	Reconvene and Introduce the Panel	Natalie Pica, MD, PhD FDA

1:10-2:00	**Panel Discussion on Drug Development Considerations for Products to Treat Neonatal Enterovirus Infection** 1) Please discuss the key challenges in antiviral drug development for the treatment of enterovirus infection in infants and neonates • Comment on what additional nonclinical or basic science work may be needed to help drive therapeutic development for treatment of enterovirus infection in infants and neonates. 2) Please discuss potential strategies that could be considered to improve collaboration between industry, academia, and parents/caregivers to facilitate antiviral therapeutic development for the treatment of enterovirus infection in infants and neonates	Moderators: Natalie Pica, MD, PhD; FDA Yodit Belew, MD; FDA
2:00-2:15	Break	
2:15-3:30	**Panel Discussion on Clinical Trial Designs to Evaluate Treatment of Neonatal Enterovirus Infection** 3) Discuss the ideal study populations for enrollment into a clinical trial • Age group (e.g., neonates only; infants and neonates) • Infection severity (mild symptomatic infection or severe infection/disease) 4) Considering the ideal population, please discuss the appropriate trial endpoints (e.g., mortality, time to hospital discharge, etc.) 5) Please discuss the most appropriate comparator treatment group. • Please comment on the potential role of real-world data and real-world evidence	Moderators: Natalie Pica, MD, PhD; FDA Yodit Belew, MD; FDA
3:30	Wrap-up and adjourn	

Day 2- Congenital CMV (cCMV) Infection

Time	Topic	Speaker(s) and Affiliation
9 – 9:10	Introductory Remarks	Aimee Hodowanec, MD FDA
Session 1. Congenital CMV Infection Epidemiology and Clinical Overview		
9:10-9:25	Surveillance and Epidemiology of cCMV in the United States	Tatiana Lanzieri, MD CDC
9:25-9:40	CMV and the Maternal-Fetal Dyad: Whom to Screen, How to Screen, and When to Treat?	Mark Schleiss, MD University of Minnesota
9:40-10:00	New Horizons in Clinical Diagnosis and Treatment of Congenital CMV	Roberta DeBiasi, MD, MS Children's National Hospital
10:00-10:15	Living with Congenital CMV: Parent Perspectives	Megan Pesch, MD University of Michigan
10:15-10:25	Clarifying Questions and Answers	
10:25-10:40	Break	
Session 2. Congenital CMV Infection Drug Development Considerations		
10:40-10:55	Preclinical Models of Congenital CMV Infection	Emma Mohr, MD, PhD University of Wisconsin-Madison
10:55-11:10	cCMV and Hearing Loss: Study Design Considerations	Lindsay DeVries, AuD, PhD FDA
11:10-11:20	Alternative Routes of Drug Administration	Ryan Kau, MD FDA
11:20-11:35	Neurodevelopmental Outcomes of Children with cCMV: A Wide Spectrum	Megan Pesch, MD University of Michigan
11:35-11:50	cCMV Drug Development: Where do we go from here? Experience of the Pediatric Trials Network	Rachel Greenberg, MD, MB, MHS Duke University Medical Center
11:50-12:10	Clarifying Questions and Answers	
12:10-1:00	Lunch	
Session 3. Congenital CMV Infection: Trial Design Challenges		
1-1:10	Reconvene and Introduce the Panel	Aimee Hodowanec, MD FDA
1:10-2:00	**Panel Discussion on Drug Development Considerations for Products to Treat cCMV Infection** 1) Please discuss the key challenges in antiviral drug development for the treatment of cCMV infection	Moderators: Aimee Hodowanec, MD; FDA Prabha Viswanathan, MD; FDA

	• Comment on what additional nonclinical or basic science work may be needed to help drive therapeutic development for treatment of cCMV infection *2)* Please discuss potential strategies that could be considered to improve collaboration between industry, academia, and parents/caregivers to facilitate antiviral therapeutic development for the treatment of cCMV infection	
2:00-2:15	Break	
2:15-3:30	**Panel Discussion on Clinical Trial Designs to Evaluate Treatment of cCMV Infection** 3) Please discuss the ideal study population(s) for clinical trial enrollment (e.g., symptomatic, hearing loss only, asymptomatic) 4) Considering the different populations, please discuss the appropriate efficacy endpoints • Hearing loss (total ear vs worst ear vs best ear; at what time point?) • Neurodevelopmental outcomes 5) Please discuss the most appropriate comparator treatment group • Please comment on the potential role of real-world data and real-world evidence	Moderators: Aimee Hodowanec, MD; FDA Prabha Viswanathan, MD; FDA
3:30	Closing Remarks/Adjourn	Prabha Viswanathan, MD FDA

All Speakers and Panelists:

FDA: Yodit Belew, John Concato, Lindsay DeVries, Aimee Hodowanec, Ryan Kau, An Massaro, Lily (Yeruk) Mulugeta, Natalie Pica, Amy Rosenfeld, Prabha Viswanathan, Kunyi Wu

External (see full panelist Affiliations and Disclosures using the workshop webpage link below):

Mark Abzug, University of Colorado School of Medicine; **Tien Bo,** Takeda Pharmaceuticals; **David Byron,** AntiVirus Therapeutics; **Roberta DeBiasi,** Children's National Hospital and Research Institute, George Washington School of Medicine; **Miranda Delahoy,** Centers for Disease Control and Prevention (CDC); **Rachel Greenberg,** Duke University School of Medicine, Duke Clinical Research Institute; **Paul Griffiths,** University College London; **Jeffrey Hincks,** ViroDefense, Inc.; **David Kimberlin,** University of Alabama at Birmingham; **Tatiana Lanzieri,** CDC**; Kevin Messacar,** University of Colorado Children's Hospital Colorado; **Emma Mohr,** University of Wisconsin-Madison; **M. Steven Oberste,** CDC; **Megan Pesch,** University of Michigan/Michigan Medicine; **Betsy Pilon,** Hope for Hypoxic Ischemic Encephalopathy (HIE); **Mark Schleiss,** University of Minnesota Medical School; **Matthew Vogt,** University of North Carolina at Chapel Hill School of Medicine

Speaker slides and other workshop materials will be posted before/after workshop at:

Workshop Webpage Link (with registration): https://www.fda.gov/drugs/news-events-human-drugs/drug-development-considerations-treatment-congenital-cytomegalovirus-infection-and-neonatal

Public Zoom Link (day of meeting): Register in advance for this webinar using this Zoom link. After registering, you will receive a confirmation email containing information about joining the webinar.