Pediatric Developmental Safety Assessment and New Approach Methodologies

A Virtual Workshop sponsored by the U.S. Food and Drug Administration and the Triangle Center of Excellence in Regulatory Science and Innovation

Friday, December 5, 2025
8:00 AM – 4:00 PM EST

Introduction

8:00 AM – 8:15 AM	**Welcome and Introduction** Dr. Gilbert Burckart, Associate Director for Pediatrics Office of Clinical Pharmacology, CDER, US FDA
8:15 AM – 8:35 AM	**Pediatric Developmental Safety, Extrapolating Drug Safety, and the Road Ahead for Pediatric Safety Assessments** Dr. Lynne Yao, Director of the Division of Pediatric and Maternal Health, Office of New Drugs, CDER, US FDA

Topic 1: Identifying Secondary Targets for Pediatric Developmental Safety

Moderator: Dr. Rebecca Racz, Office of Clinical Pharmacology, CDER, US FDA

8:35 AM – 8:55 AM	**Pediatric Developmental Genotype-Tissue Expression Program, National Human Genome Research Institute, NIH** Dr. Kristin Ardlie, Director, GTEx Laboratory Data Analysis and Coordination Center, Broad Institute, Cambridge, MA
8:55 AM – 9:15 AM	**FDA Experience with Secondary Targets for New Drugs** Dr. Rebecca Racz, Senior Pharmacokineticist, Office of Clinical Pharmacology, CDER, US FDA
9:15 AM – 9:35 AM	**Making Secondary Targets a Routine Part of Drug Development** Dr. Jean-Pierre Valentin, Head of Investigative Toxicology, UCB Biopharma; DruSafe Chair, IQ Consortium

9:35 AM – 9:55 AM	**Developing a Panel of Secondary Targets for Pediatric Developmental Safety** Dr. Nicholas Tatonetti, Vice Chair, Department of Computational Biomedicine, Cedars-Sinai Medical Center, Los Angeles, CA
9:55 AM – 10:15 AM	**BREAK**

Topic 2: Preclinical Tools for Pediatric Developmental Safety Assessment

Moderator: Dr. Jeffrey Fisher, most recently at ScitoVation LLC, Research Triangle Park NC

10:15 AM – 10:35 AM	**Industry Experience with Juvenile Animal Studies** Dr. Susan Laffan, Senior Director, Translational Safety and Risk Sciences, Amgen
10:35 AM – 10:55 AM	**Microphysiological Systems to Predict Pediatric Developmental Efficacy and Safety Outcomes** Dr. James Hickman, Chief Scientist, Hesperos, Inc.
10:55 AM – 11:15 AM	**In vitro and In Silico Tools for Safety Assessment for the Postnatal Period** Dr. Marjory Moreau, Principal Scientist and Lead for Next Generation Risk Assessment, ESQlabs
11:15 AM – 12:00 PM	**Topics 1 and 2 Panel Discussion and Questions** Dr. Gilbert Burckart, Office of Clinical Pharmacology, CDER FDA; Dr. Paul Watkins, UNC Chapel Hill & Triangle CERSI; Dr. Ikram Elayan, Pharm/Tox Supervisor, DPT-N, OND, CDER FDA
12:00 PM – 12:45 PM	**LUNCH BREAK**

Topic 3: Experience with Developing Tools for Specific Toxicology Screening

Moderator: Dr. Paul Watkins, Distinguished Professor, Eshelman School of Pharmacy, UNC-Chapel Hill and Co-Director, Triangle CERSI

12:45 PM – 1:05 PM	**Building a QSP Tool for Pediatric Safety Analysis: DILIsym** Dr. Paul Watkins, UNC Chapel Hill; Triangle CERSI
1:05 PM – 1:25 PM	**Adapting a secondary pharmacology-based tool to flag-up off-target interactions of particular concern in pediatric patients**

Dr. Will Redfern, Vice President, Quantitative Systems Toxicology and Safety, Certara

Topic 4: Using Prior Experience + Modeling & Simulation for Pediatric Developmental Safety

Moderators: Dr. Aaron Pawlyk, Chief, Obstetric and Pediatric Pharmacology and Therapeutics Branch, National Institute of Child Health and Human Development and Dr. Gilbert Burckart

1:25 PM – 1:45 PM	**Resources Available for Analysis of Prior Information for Pediatric Developmental Safety** Dr. Lang Li, Professor and Chair, Maternal and Pediatric Precision in Therapeutics (MPRINT) Data and Model Knowledge and Research Coordination Center, Dept. of Biomedical Informatics, College of Pharmacy, Ohio State University
1:45 PM – 2:05 PM	**Use of Modeling and Simulation in Safety Assessments** Dr. Rajanikanth Madabushi, Associate Director for Guidance and Policy, Office of Clinical Pharmacology, and CDER Quantitative Medicine Center of Excellence, US FDA
2:05 PM – 2:25 PM	**Multiomics-informed Modeling of Drug Disposition and Safety in Pediatric Patients** Dr. Bhagwat Prasad, Division Director, Translational and Clinical Pharmacology and Professor, Pediatrics, Cincinnati Children's Hospital Medical Center
2:25 PM – 2:45 PM	**How can we answer the Pediatric Developmental Safety Questions in ICH E11A Using These Techniques?** Dr. Gilbert Burckart, Office of Clinical Pharmacology, CDER FDA
2:45 PM – 3:45 PM	**Topics 3 and 4 Panel Discussion and Questions** Dr. Gilbert Burckart, Office of Clinical Pharmacology, CDER FDA; Dr. Paul Watkins, UNC Chapel Hill; Triangle CERSI; Dr. Mona Khurana, Pediatric Team Lead in Office of Rare Diseases, Pediatrics, Urologic an Reproductive Medicine, OND CDER FDA
3:45 PM – 4:00 PM	**Wrap Up** Dr. Gilbert Burckart, Office of Clinical Pharmacology, CDER FDA