Pediatric Developmental Safety Assessment and New Approach Methodologies

A Hybrid 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
Great Room, White Oak Campus (with Remote Option)

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. Dionna Green, Director, Office of Pediatric Therapeutics
Office of the Commissioner, 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, 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 In Vitro Systems to Complement Pediatric Developmental Efficacy and Safety Assessment** 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, Associate Director of Computational Toxicology, ScitoVation
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
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, Associate Professor, Washington State University
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
3:45 PM – 4:00 PM	**Wrap Up** Dr. Gilbert Burckart, Office of Clinical Pharmacology, CDER FDA