

AGENDA - WORKSHOP

THURSDAY, MARCH 27, 2025

From Childhood to Adulthood: Unraveling the Relevance of Developmental Pathways to Pulmonary Vascular Disease for Therapeutic Development

7:30am *Registration | Continental Breakfast | Exhibits Open*

7:55 **Welcome and Introduction**
Drs. Abman, Austin, and Fineman

8:00 **Setting the Stage: Interdisciplinary Care of the Infant and Young Child with PH Associated with Developmental Lung Disorders (a subtype of Group 3 PH)**
Nidhy P. Varghese, MD

SESSION 1: Normal, Abnormal, and Repaired Cardiopulmonary Development

Moderators: Roberta L. Keller and Eric Austin

8:30 **FOX1 Variants in Lung Development, Repair, and Adult PAH**
Jose Gomez-Arroyo, MD, PhD

9:00 **Defining the Typical Course of Persistent Pulmonary Hypertension of the Newborn: What Characterizes an Atypical Course?**
Stephanie M. Tsoi, MD

9:30 *Break | Exhibits*

SESSION 2: Features and Modulation of the Lung Microenvironment Across the Lifespan

Moderators: Jeffrey Whitsett, MD and Cristina Alvira, MD

9:50 **Familial Experience with a Rare Lung Disease Diagnosis, Syndrome Recognition, and Formation of Disease-Targeted Foundation**
Anton Morkin, Founder and CEO, TBX4Life

10:20 **Introduction: TBX4Life Lecture**
Eric D. Austin, MD, MSc

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- 10:25 **The TBX4Life Lecture**
“Is Adult PAH a Pediatric Disease?” Transcriptional Programs That Orchestrate Fetal Lung Development Reactivate in Adult PAH Patient-Derived Lung Cells
Soni S. Pullamsetti, PhD – TBX4 Life
- 11:00 **Lung Microenvironment’s Modulation of Gene Transcription Across the Lifespan**
Lance S. Prince, MD
- 11:30 *Lunch on your own*

SESSION 3: Therapeutic Approaches to Target Pulmonary Vascular Disease, Lung Protection and Regeneration: What is Possible and Where Are We Headed?

Moderators: Steven H. Abman and Bernard Thébaud

- 1:00 **Beyond “Possible”: In Utero Gene Therapy for Developmental and Other Diseases**
Tippi MacKenzie, MD
- 1:30 **Mesenchymal Cell therapy trial to heal the lungs of preterm infants**
Bernard Thébaud, MD
- 2:00 **Gene Therapy and Drug Delivery Precisely to the Lung Microvascular Endothelium as a Treatment for Pulmonary Vascular Diseases**
Vladimir Kalinichenko, MD, PhD
- 2:30 *Break | Exhibits*
- 2:40 **Generation of Induced Pluripotent Stem Cells to Develop Individualized Drug Therapeutic Approaches**
Darrell N. Kotton, MD
- 3:10 **Known Drugs Doing New Things: Repurpose of Existing Drug Therapies to Treat Pulmonary Vascular Diseases**
Edda Spiekerkoetter, MD
- 3:40 **Lung development, repair, regeneration, and potential for therapeutic development**
Edward E. Morrisey, PhD
- 4:10 **Rare Inform the Common: Preserving Vascular Development for the Prevention and Treatment of Neonatal PH**
Steven H. Abman, MD
- 4:40-5:00 **Group Discussion/Adjourn**