## **WORKSHOP**

## From Childhood to Adulthood:

## Unraveling the Relevance of Developmental Pathways to Pulmonary Vascular Disease for Therapeutic Development

Thursday, March 27, 2025

7:30 am - 4:00 pm

| 7:30am     | Sign-in and Continental Breakfast  |
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| 7:55       | Welcome and Introduction   |
| 8:00am     | Setting the Stage: Interdisciplinary Care of the Infant and Young Child with PH Associated with Developmental Lung Disorders (a subtype of Group 3 PH) |
| Session 1: | Normal, Abnormal, and Repaired Cardiopulmonary Development   |
| 8:30       | Key factors in the regulation of Cardiac Morphogenesis   |
| 9:00       | Lung development, repair, regeneration, and potential for therapeutic development  |
| 9:30       | Defining the Typical Course of Persistent Pulmonary Hypertension of the Newborn: What Characterizes an Atypical Course?                                |
| 9:50       | Break  |
| Session 2: | Features and Modulation of the Lung Microenvironment Across the Lifespan   |

| 10:00      | Familial Experience with a Rare Lung Disease Diagnosis, Syndrome Recognition, and Formation of Disease-Targeted Foundation (Title to be determined)           |
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| 10:20      | Introduction: TBX4Life Lecture  |
| 10:25      | The TBX4Life Lecture  |
|            | "Is Adult PAH a Pediatric Disease?" Transcriptional Programs That<br>Orchestrate Fetal Lung Development Reactivate in Adult PAH<br>Patient-Derived Lung Cells |
| 11:00      | Lung Microenvironment's Modulation of Gene Transcription Across the Lifespan  |
| 11:30      | Lunch on your own   |
| Session 3: | Therapeutic Approaches to Target Pulmonary Vascular Disease,<br>Lung Protection and Regeneration:   |
|            | What is Possible and Where Are We Headed?   |
| 1:00       | Beyond "Possible": <i>In Utero</i> Gene Therapy for Developmental and Other Diseases  |
| 1:30       | Mesenchymal Cell therapy trial to heal the lungs of preterm infants   |
| 2:00       | Gene Therapy Targeting the Lung Microvascular Endothelium as a<br>Treatment for Pulmonary Vascular Diseases   |
| 2:30       | Break   |
| 2:40       | Generation of Induced Pluripotent Stem Cells to Develop Individualized Drug Therapeutic Approaches  |
| 3:10       | Known Drugs Doing New Things: Repurpose of Existing Drug<br>Therapies to Treat Pulmonary Vascular Diseases  |
| 3:40       | Rare Inform the Common: Preserving Vascular Development for the Prevention and Treatment of Neonatal PH   |
| 4:15       | Adjourn   |