C-Path Hosts Its Scientific Breakthrough Summit 2023

On October 26 and 27, 2023 Critical Path Institute's Pediatrics Program hosted a Scientific Breakthrough Summit (SBS) in Arlington, VA. The objectives for the meeting were:

- Explore the unmet needs and potential solutions relevant to specific aspects of Type-1 Diabetes (T1D), Alpha-1 Antitrypsin Deficiency (AATD), Lysosomal Diseases (LDs), and neonatal drug development.??
- Through compelling case studies, demonstrate the critical role data sharing plays in enhancing drug development for vulnerable patient populations with conditions that require interventions early in life.?
- Review the current landscape of disease modifying therapies (including drugs, biologics, and regenerative therapies) and identify regulatory opportunities that could expedite the development of such groundbreaking products.

The agenda with links to the audio recordings and slides are available below.

Please contact pediatricsadmin@c-path.org with any questions about this event.

Day 1: Thursday, October 26

Welcome and Opening Remarks

Daniel Jorgensen (C-Path), Klaus Romero (C-Path), Kanwaljit Singh (C-Path)

Keynote: Impact of Global Public-Private Collaborations on Drug Development

Moderators: Klaus Romero (C-Path), Kanwaljit Singh (C-Path)

Understanding the Regulatory Landscape

Janet Maynard (FDA)

Exploring Gene and Cell-Based Therapies

Peter Marks (FDA)

Fireside chat: Pediatric Global Perspectives

Ralph Bax (EMA), Agnes Klein (Health Canada), Junko Sato (PMDA), Kristen VanGoor (Takeda)

Fireside chat: Impact and Future of Real-World Data and Real-World Evidence

Deb Discenza (Preemie World/Alliance for Black NICU Families), Wakako Eklund (Pediatrix Medical Group), Thomas Miller (Bayer), Perdita Taylor-Zapata (NICHD)

Session I: Disease Modification

Opening Remarks for Session I

Klaus Romero (C-Path)

Disease Modification in Alpha-1 Antitrypsin Deficiency

Identifying and quantifying sources of variability for disease modification of AATD

Jeffery Teckman (SLU)

Panel Moderators: Kanwaljit Singh (C-Path), Gina Smith (C-Path), Panelists: Frank Anania (FDA), Jon Hagstrom (Alpha-1 Foundation), Prateek Shukla (FDA), Jeffery Teckman (SLU)

Disease Modification in Lysosomal Diseases and Bronchopulmonary Dysplasia

Advancing Disease Modification in Lysosomal Diseases: Leveraging Biomarkers and Public-Private Collaborations at Critical Path Institute

Collin Hovinga (C-Path), Krista Casazza (C-Path)

Current landscape in biological therapies to modify disease course of BPD

Victoria Niklas (Oak Hill Bio)

Panel I-b **Moderators: Collin Hovinga (C-Path), Krista Casazza (C-Path) Panelists: Gerri Baer (FDA), Ebony Ford (Miracle Mamas), Christine Hon (FDA), Victoria Niklas (Oak Hill Bio)**

Lunch Panel: Persistent Issues in Pediatric Drug Development: Challenges and Opportunities

Moderator: Tim Franson (C-Path), Panelists: AJ Allen (I-ACT for Children), Jonathan Davis (Tufts Medical Center), Daniel Jorgensen (C-Path), Susan McCune (PPD), Gary Noel (GJN Consulting), Stephen Spielberg (C-Path)

Session II: Regulatory Perspectives

Panel II-a Continuity of Drug Development for Disease Across the Lifespan

Moderators: Joseph Hedrick (C-Path), Huong Huynh (C-Path) Panelists: Hide Nakamura (National Center for Child Health, Tokyo, JAPAN), Jeff Siegel (FDA), Celia Witten (FDA), Lauren Wood Heickman (FDA), Anita Zaidi (FDA)

Panel II-b Global Perspectives of Pediatric Drug Development

Moderators: Cécile Ollivier (C-Path), Kanwaljit Singh (C-Path) Panelists: Ralph Bax (EMA), Agnes Klein (Health Canada), Mona Khurana (FDA), Perdita Taylor-Zapata (NICHD), Sarah Zaidi (FDA)

Closing Remarks

Kanwaljit Singh (C-Path)

Day 2, Friday, October 27

Session III: Disease Prevention

Session III-a: Disease Prevention in T1D and Neonates

Opening Remarks for Sessions III

Jonathan Davis (Tufts)

Case study in T1D: Solutions to accelerate disease-modifying medications

Klaus Romero (C-Path), Jessica Dunne (Novo Nordisk)

INC current perspective: Conducting gene therapy clinical trials in neonatal and early pediatric age, including newborn screening

Jim Wilson (Penn)

Panel III-a: **Moderators: Joseph Hedrick and Klaus Romero (C-Path) Panelists: Justin Earp (FDA), Larissa Lapteva (FDA), Esther Latres (JDRF), Melissa Lestini (FDA), Justin Penzenstadler (FDA), Stefany Shaheen (T1D Patient Advocate), Jim Wilson (Penn)**

Session III-b: Disease Prevention in Lysosomal Diseases

Drug development in Cell and Gene Therapy – Industry Perspective:

Tom Miller (Bayer)

Panel III-b **Moderators: Kanwaljit Singh, Krista Casazza (C-Path) Panelists: Elizabeth Hart (FDA), Gavin Imperato (FDA), Tom Miller (Bayer), Galina Nesterova (Thermo Fisher), Laurie Turner (National Niemann-Pick Disease Foundation), Jim Wilson (Penn)**

Session IV: Clinical Trial Design Considerations

Panel IV-a Considerations for Clinical Trial Design Optimization in Alpha-1 Antitrypsin Deficiency and Lysosomal Diseases

Moderators: Gina Smith, Krista Casazza (C-Path) Panelists: Jon Hagstrom (Alpha-1 Foundation), Christine í Dali (Zevra), Gavin Imperato (FDA), Jonathan Jacoby (SOAR-NPC), Ed Marins (Takeda), Jeff Siegel (FDA), Yulia Yasinskaya (FDA)

Panel IV-b Considerations for Clinical Trial Design Optimization in Neonates and T1D

Moderators: Kanwaljit Singh (C-Path), Joe Hedrick (C-Path), Panelists: A.J. Allen (I-ACT for Children), Ralph Bax (EMA), John Concato (FDA), An Massaro (FDA), Victoria Niklas (Oak Hill

Bio), Betsy Pilon (Hope for HIE), John Sharretts (FDA)

Closing Remarks

Klaus Romero, Kanwaljit Singh, and Joseph Hedrick (C-Path)