Agenda

Annual Meeting Day 1 | Oct 19

Day 1 Cell and Gene Therapies: Implications for Neonates

- Focus on the rapidly evolving field of cell and gene therapy in neonatal population
- Discuss the current landscape of gene therapy clinical trials in neonates
- Perspectives from Industry, Academic, Regulator, Nurse and Parent partners of INC
- Subsequent discussion on the diagnostic platforms for whole genome sequencing, and ethical considerations related to diagnosis of genetic conditions and participation in gene therapy trials

10:00AM-12:15PM ET
Cell and Gene Therapies (C&GTx): Implications for Neonates

10:00 – 10:05AM
WELCOME & INTRODUCTORY COMMENTS DAY 1
- Kanwaljit Singh, INC EXECUTIVE DIRECTOR, C-Path

10:05 – 10:25AM
PLENARY
- Jeffrey Siegel, Office Director, Office of Drug Evaluation Sciences, Food and Drug Administration (FDA)

10:25 – 10:35 AM
INAUGURAL FRIEND OF INC AWARD
- Susan McCune, Vice President Pediatrics and Rare Diseases, PPD

CHAIRS AND MODERATORS
- Mark Turner, Professor of Neonatology, University of Liverpool
- Tom Miller, Vice President Pediatric Development, Bayer

10:35 – 11:30AM
C&GTx: LANDSCAPE OF SPONSORED TRIALS
- Jill Maron, Pediatrician-in-Chief, Women & Infants Hospital of Rhode Island
- Bernard Thebaud, Neonatologist, Ottawa Hospital and Children’s Hospital of Eastern Ontario
- Nicole LaMarca, Global Medical Director, Data Exploration and Generation Lead-SME, Novartis
11:30AM – 12:15PM DELIVERY OF CLINICAL TRIALS PANEL DISCUSSION AND Q&A
MODERATOR, KANWALJIT SINGH
- ELIZABETH HART, BRANCH CHIEF, GENERAL MEDICINE 1, CBER, FDA
- CATHERINE WALSH VOCKLEY, GENETIC COUNSELOR, DIVISION OF GENETIC AND GENOMIC MEDICINE, UPMC
- LAURENT SERVAIS, PROFESSOR OF PEDIATRIC NEUROMUSCULAR DISEASES, UNIVERSITY OF OXFORD
- DEBORAH GILL, PROFESSOR OF GENE MEDICINE, OXFORD UNIVERSITY
- RICHARD SCOTT, CHIEF MEDICAL OFFICER, GENOMICS ENGLAND
- ERIN WARD, CO-FOUNDER & PRESIDENT, MTM-CNMS FAMILY CONNECTION

12:15PM – 1:00PM BREAK

1:00-2:00PM ET
INC Workgroup Updates

WELCOME BACK AND INC OVERVIEW VIDEO – KANWALJIT SINGH
COMMUNICATIONS: MARY SHORT, RETIRED INDUSTRY/NEONATAL NURSE
SAFETY/ADVERSE EVENTS: THOMAS SALAETS, PEDIATRIC CARDIOLOGY FELLOW, UZ LEUVEN
DATA TERMINOLOGY: MIKE PADULA, MEDICAL DIRECTOR OF INFORMATICS DIVISION OF NEONATOLOGY, CHILDREN'S HOSPITAL OF PHILADELPHIA
HEMODYNAMIC ADAPTATION: HEIKE RABE, PROFESSOR PERINATAL MEDICINE, UNIVERSITY OF SUSSEX
PAIN MEASUREMENTS: REBECCA SLATER, PROFESSOR PEDIATRIC NEUROSCIENCE, UNIVERSITY OF OXFORD

ANNUAL MEETING RESUMES IN 4 HOURS
6:00-8:00 PM ET
Genetic Testing Technologies: Diagnostics and Ethics

6:00 – 6:05PM
WELCOME BACK
CHAIRS & MODERATORS
• Kanwaljit Singh, INC Executive Director, C-Path
• Jon Davis, Neonatologist & INC CO-Director, Tufts Medical Center
• Jerry Vockley, Chief of Genetic and Genomic Medicine, University of Pittsburgh

6:05 – 6:30PM
DIAGNOSTIC TECHNOLOGIES
• Stephen Kingsmore, President/CEO Rady’s Children’s Inst. for Genomic Medicine
• Ryan Taft, VP Scientific Research, Illumina

6:30 – 6:50PM
TRIAL RESULTS
• Toshiki Takenouchi, Assistant Professor of Pediatrics, Keio University

6:50 – 7:15PM
ETHICS
• Alison Bateman-House, Asst. Professor, NYU Langone Health
• John Lantos, Dir. Pediatric Bioethics, Children’s Mercy Kansas City

7:15 – 8:00PM
PANELLISTS/Q&A WITH ALL PANELISTS AND SPEAKERS
• Gerri Baer, Supervisory Medical Officer Neonatology, FDA OC
• Jeff Barrett, Sr. Vice President, C-Path
• Chip Chambers, Assistant Clinical Professor of Surgery, Vanderbilt University Medical Center, Founder & President DADA2 Foundation
• Maria Escolar, Professor of Pediatrics and Neurodevelopmental Disabilities, University of Pittsburgh
• Cindy Prows, Genetics Clinical Nurse Specialist, Cincinnati Children’s

DAY 1 CONCLUDES
Agenda

Workshop Day 2 | Oct 20

Real-World Data
• Focus on the utility of real-world data to generate real-world evidence as it pertains to neonatal research
• Accelerate our understanding of some of the most challenging conditions in the neonatal population by applying Critical Path Institute’s core competencies of data science and analysis to real-world data

10:00AM – 12:15PM ET
REAL-WORLD DATA

10:00 – 10:05AM  WELCOME & INTRODUCTORY COMMENTS DAY 2
• KANWALJIT SINGH, INC EXECUTIVE DIRECTOR, C-PATH

CHAIRS:
• KLAUS ROMERO, CHIEF SCIENCE OFFICER, C-PATH
• NEENA MODI, PROFESSOR NEONATAL MEDICINE, IMPERIAL COLLEGE LONDON

10:05 – 10:30AM  PARENT AND FDA KEYNOTE SPEECH
• DEB DISCENZA, FOUNDER/CEO PREEMIEWORLD
• JOHN CONCATO, ASSOCIATE DIRECTOR FOR REAL-WORLD EVIDENCE, FDA CDER

10:30 – 10:45AM  CONSIDERATIONS FOR RWD IN NEONATOLOGY
• RALPH BAX, HEAD OF PEDIATRIC MEDICINES, EUROPEAN MEDICINES AGENCY

10:45 – 11:10AM  FROM DATA TO SOLUTIONS – C-PATH PERSPECTIVE
• PLUGGING IN DATA: HARMONIZATION AND INTEGRATION OF REAL-WORLD DATA, WILL RODDY
• A CASE STUDY IN RWD DATA INTEROPERABILITY AND ANALYTICS PRESENTATION, JAGDEEP PODICHETTY & MEGAN CALA
11:00 – 12:00PM  
PANEL DISCUSSION AND Q&A  
MODERATOR: KANWALJIT SINGH  
- SATOSHI KUSUDA, NEONATOLOGIST, KYORIN UNIVERSITY  
- LAURA FABBRI, HEAD OF CLINICAL NEONATOLOGY, CHIESI PHARMACEUTICALS  
- ANDREW BREMER, CHIEF, PEDIATRIC GROWTH AND NUTRITION BRANCH, NICHD  
- COLLIN HOVINGA, SR. VP CLINICAL AND SCIENTIFIC AFFAIRS, I-ACT FOR CHILDREN  
- MICHAEL PADULA, MEDICAL DIRECTOR OF INFORMATICS DIVISION OF NEONATOLOGY, CHILDREN’S HOSPITAL OF PHILADELPHIA  
- SHEULI PORKESS, CHAIR OF THE POLICY AND COMMUNICATIONS GROUP AND VICE-PRESIDENT ELECT AT THE FACULTY OF PHARMACEUTICAL MEDICINE, DIRECTOR OF ACTAROS CONSULTANCY  

12:00 – 12:15PM  
DATA PIONEER AWARDS AND CLOSING  
- BAYER, CHIESI, NICHD, TUFTS MEDICAL CENTER

12:15 PM ET  
ANNUAL MEETING CONCLUDES  
THANK YOU!