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

10am-12pm 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 – 10:20AM  PLENARY TALK
JEFFREY SIEGEL, OFFICE DIRECTOR, OFFICE OF DRUG EVALUATION SCIENCES

CHAIRS AND MODERATORS
MARK TURNER, PROFESSOR OF NEONATOLOGY, UNIVERSITY OF LIVERPOOL,
TOM MILLER, VICE PRESIDENT PEDIATRIC DEVELOPMENT, BAYER

10:20 – 10:50AM  C&GTx: LANDSCAPE OF SPONSORED TRIALS
BERNARD THEBAUD, NEONATOLOGIST, OTTAWA HOSPITAL AND CHILDREN’S HOSPITAL OF EASTERN ONTARIO
ELIZABETH HART, BRANCH CHIEF, GENERAL MEDICINE 1, CBER, FDA
10:50AM – 12:00PM
Delivery of Trials
MODERATOR, KANWALJIT SINGH
NICOLE LAMARCA, GLOBAL MEDICAL DIRECTOR, DATA EXPLORATION AND GENERATION
LEAD-SME, NOVARTIS
CATHERINE WALSH VOCKLEY, GENETIC COUNSELOR, DIVISION OF GENETIC AND GENOMIC MEDICINE, UPMC
LAURENT SERVAIS, PROFESSOR OF PAEDIATRIC NEUROMUSCULAR DISEASES, UNIVERSITY OF OXFORD
DEBORAH GILL, PROFESSOR OF GENOME MEDICINE, OXFORD UNIVERSITY
RICHARD SCOTT, CHIEF MEDICAL OFFICER, GENOMICS ENGLAND

12:15-1:15PM ET
INC Workgroup Updates

SAFETY/ADVERSE EVENTS: THOMAS SALAETS, PEDIATRIC CARDIOLOGY FELLOW, UZ LEUVEN
COMMUNICATIONS: MARY SHORT, RETIRED INDUSTRY/NEONATAL NURSE
DATA TERMINOLOGY: MIKE PADULA, MEDICAL DIRECTOR OF INFORMATICS DIVISION OF NEONATOLOGY, CHILDREN’S HOSPITAL OF PHILADELPHIA
HEMODYNAMIC ADAPTATION: HEIKE RABE, PROFESSOR PEDIATRICS, ROYAL ALEXANDRIA CHILDREN’S HOSPITAL
RACIAL & HEALTH EQUITY: DEB DISCENZA, FOUNDER/CEO PREEMIEWORLD; JENNIFER DEGL, SPEAKING FOR MOMS AND BABIES INC; TAMORAH LEWIS, NEONATOLOGIST, CHILDREN’S MERCY KANSAS CITY

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
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
10am – 12pm 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 (CSO), 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 – 11:00AM  DATA COLLABORATION – C-PATH PERSPECTIVE
C-Path Data Collaboration Center/QuanT Med – Will Roddy/Amanda Borens,
Jagdeep Podichetty/Megan Cala

11:00 – 12:00PM  PANEL DISCUSSION/TESTIMONIALS:
Moderator: Kanwaljit Singh
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
Satoshi Kusuda, Neonatologist, Kyorin University
Michael Padula, Medical Director of Informatics Division of Neonatology,
Children’s Hospital of Philadelphia

12:00PM – 12:15PM  AWARDS AND CLOSING

12:15 PM ET
ANNUAL MEETING CONCLUDES
THANK YOU