

International Neonatal Consortium Scientific Annual Meeting Oct 19-20, 2021

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>x): 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 – 10:35AM

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>x: 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
- DEBORAH GILL, PROFESSOR OF GENE MEDICINE, OXFORD UNIVERSITY
 - 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
 - RICHARD SCOTT, CHIEF MEDICAL OFFICER, GENOMICS ENGLAND
 - ERIN WARD, CO-FOUNDER & PRESIDENT, MTM-CNM 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 TAKENOUCI, 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

PANELISTS/Q&A WITH ALL PANELISTS AND SPEAKERS

- MARIA ESCOLAR, PROFESSOR OF PEDIATRICS AND NEURODEVELOPMENTAL DISABILITIES, UNIVERSITY OF PITTSBURGH
- 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
- CINDY PROWS, GENETICS CLINICAL NURSE SPECIALIST, CINCINNATI CHILDREN'S

DAY 1 CONCLUDES

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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
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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 KEYNOTESPEECH

- 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

- WILLIAM RODDY, SR. DATA ENGINEER, C-PATH
 - PRESENTATION: PLUGGING IN DATA: HARMONIZATION AND INTEGRATION OF REAL-WORLD DATA
- JAGDEEP PODICHETTY & MEGAN CALA
 - PRESENTATION: A CASE STUDY IN RWD DATA INTEROPERABILITY AND ANALYTICS PRESENTATION

11:10 – 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!**