

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

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| 10:00 – 10:05AM | WELCOME & INTRODUCTORY COMMENTS DAY 1 <ul style="list-style-type: none">• KANWALJIT SINGH, INC EXECUTIVE DIRECTOR, C-PATH |
| 10:05 – 10:25AM | PLENARY <ul style="list-style-type: none">• JEFFREY SIEGEL, OFFICE DIRECTOR, OFFICE OF DRUG EVALUATION SCIENCES, FOOD AND DRUG ADMINISTRATION (FDA) |
| 10:25 – 10:35 AM | INAUGURAL FRIEND OF INC AWARD <ul style="list-style-type: none">• SUSAN MCCUNE, VICE PRESIDENT PEDIATRICS AND RARE DISEASES, PPD |
| | CHAIRS AND MODERATORS <ul style="list-style-type: none">• 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 <ul style="list-style-type: none">• 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-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

- 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

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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

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**
- 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!**