CANCELLED: 2020 Pediatric Academic Societies Meeting – International Stakeholder Collaboration to Develop Neonatal Therapeutics: How to Play Well in the Global Sandbox

https://2020.pas-meeting.org/

Type: Scientific Program Proposal 2nd

Session Role: Panel Discussion

Session Title: **International Stakeholder Collaboration to Develop Neonatal Therapeutics: How to Play Well in the Global Sandbox**

Description: Although the field of neonatology has evolved significantly since patients were routinely administered chloramphenicol and 100% oxygen, major knowledge gaps and unmet therapeutic needs persist. The majority of drugs used to treat critically ill neonates are used off-label, with incomplete information on dosing, safety, and/or efficacy. Despite efforts to design and perform high-quality neonatology clinical trials, optimal treatments for the unique conditions related to preterm birth remain elusive. To begin addressing these unmet needs, the Food and Drug Administration (FDA) and the Critical Path Institute (C-Path) launched the International Neonatal Consortium (INC) in May of 2015. The Consortium convenes academic experts, regulators, nurses, industry sponsors, and patient/parent advocates from across the globe to "forge a predictable regulatory path for evaluating the safety and effectiveness of therapies for neonates."

Introduction: Susan McCune – "How Did a Group of Academic Researchers, Families, Nurses, Regulators, and Industry Representatives Team Up to Facilitate Neonatal Research?" – ***10 mins***

Talk 1: Janet Soul (Seizure Workgroup; Boston Children's): "Developing a Master Protocol for Trials of Neonatal Seizure Therapies" – *15 mins.*

Talk 2: Thomas Salaets (Clinical Pharmacology Workgroup; KU Leuven): "Developing and Validating a Neonatal Adverse Event Severity Scale" – *15 mins.*

Talk 3: Robin Steinhorn (BPD Workgroup; Children's National): "Chronic Pulmonary Insufficiency of Prematurity: Developing Optimal Trial Endpoints" – *15 mins.*

Talk 4: Lois Smith (ROP Workgroup; Boston Children's): "Development of a Retinopathy of Prematurity Activity Scale and Clinical Outcome Measures for Use in Clinical Trials" – *15 mins.*

Panelists representing academia, industry, advocacy, and regulatory organizations) to address questions and discuss next steps (50 minutes including introducing panelists):

- Gerri Baer- FDA – initial presentation 10 minutes: "Is there a path forward for neonatal therapeutics?"
- Tom Miller – Bayer
- Ron Portman – Novartis
- Ralph Bax – European Medicines Agency
- Mark Turner – Connect 4 Children (C4C)
- Jennifer Degl – INC Communications Workgroup – Parent