Applying Regulatory Science to Neonates: Third Annual Scientific Workshop

March 27 – 29, 2017

Bethesda North Marriott Hotel and Conference Center
5701 Marinelli Road, North Bethesda,
Maryland 20852 USA

Sponsors: Critical Path Institute and U.S. Food and Drug Administration

Agenda – Day 1, March 27, 2017

8:00 am – 4:00 pm (Brookside B)	**INC Severity Scale for Neonatal Adverse Events Workgroup Meeting**
8:00 am – 4:30 pm (Oakley)	**INC Retinopathy of Prematurity Workgroup Meeting**
9:00 am – 2:00 pm (Linden Oak)	**INC Seizures Workgroup Meeting**
6:00 – 9:00 pm (Salon C)	**Workgroup Dinner at the Bethesda North Marriott Hotel**

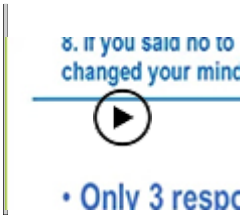
Agenda – Day 2, March 28, 2017

Meeting Objectives:

- Share regulatory perspectives on developing safe and effective therapies for neonates.
- Review the progress of INC workgroups.
- Discuss challenges in conducting registration trials to prevent pre-term birth.

- Break out into smaller groups to tackle specific regulatory science issues, including developing 1) a severity scale for neonatal adverse events; 2) long-term outcome measures; 3) a neonatal common protocol template; 4) communications to promote a research culture; and 5) multiple enrollment in clinical trials.
- Receive updates on other partnership efforts related to INC: pediatric trial networks and nonclinical models of neonatal therapeutics.

8:00 – 8:15 am	**Welcome** – *Jonathan Davis* (Tufts University, INC Co-Director)**A New Era for Developing Neonatal Therapeutics** – *Susan McCune* (Director, Office of Pediatric Therapeutics, FDA)	
8:15 – 9:00 am	**Panel for Workgroup Updates** – *Ronald Portman* (Novartis, INC Co-Director) • Retinopathy of Prematurity (*Melissa Liew, Boubou Hallberg*) • Hemodynamic Adaptation (*Heike Rabe, Janis Dionne*) • Data (*Thomas Diacovo, Michael Padula*) • Seizures (*Janet Soul, Ronit Pressler*) • Bronchopulmonary Dysplasia (*Robin Steinhorn, Wolfgang Göpel*) • Clinical Pharmacology (*Karel Allegaert, Robert Ward, Jeffery Barrett*) • Communications (*Christina Bucci-Rechtweg, Jennifer Degl*)	
9:00 – 10:00 am	**Developing Endpoints for Use in Regulated Neonatal Trials** – *Gerri Baer* (FDA) and *Ralph Bax* (EMA), co-chairs *Gerri Baer* (FDA) *Junko Sato* (PMDA) *Agnes Klein* (Health Canada) R*alph Bax* (EMA)	
10:00 – 10:30 am	**Coffee Break**	

10:30 am – 12:30 pm	**Plenary Session on Challenges in Conducting Registration Trials to Prevent Pre-Term Birth** – *Mark Turner* (University of Liverpool, INC Co-Director), chair *Olof Rugarn* (Ferring Pharmaceuticals) *Yosuke Komatsu* (GSK) *Louise Kenny* (University College Cork) *Errol Norwitz* (Tufts) *Mehali Patel* (BLISS) *Deborah Discenza* (PPA) *Ralph Bax* (EMA) *Nicole Thiele* (EFCNI) *Barbara Wesley* (FDA)	
12:30 – 1:30 pm	**Lunch**	
1:30 – 3:00 pm	**Concurrent Breakouts I** **Multiple Enrollment in Clinical Trials** – *Jonathan Davis* and *Gerri Baer*, co-chairs (Brookside) *Agnes Klein* (Health Canada) *Skip Nelson* (OC/FDA) *Ralph Bax* (EMA) *Linda Storari* (Chiesi) *Simin Baygani* (Lilly) *Anne Zajicek* (NICHD/NIH) *Karel Allegaert* (University of Leuven)	
	Communication Strategies to Promote a Research Culture – *Christina Bucci-Rechtweg* and *Jennifer Degl*, co- chairs (Seneca Boardroom) *Nicole Thiele* (EFCNI)	
	Long-term Outcomes – *Neil Marlow* and *Mark Turner*, co-chairs (*Cabin John Boardroom*)	
3:00 – 3:30 pm	**Coffee Break**	

3:30 – 5:00 pm	**Concurrent Breakouts II** **Developing a Neonatal Common Protocol Template** – *Ronald Portman* and *Anne Cropp* (Echelon Pharma Solutions), co-chairs (Seneca Boardroom) *Anne Cropp* (Echelon Pharma Solutions) *Dionna Green* (FDA) *Michelle Culp* (NCATS/NIH) *Janet Soul* (Harvard)	
	Applying Generic Severity Grading Criteria to Persistent Pulmonary Insufficiency of Prematurity – *Karel Allegaert* and *Thomas Salaets*, co-chairs (Brookside) *Wolfgang Göpel* (University of Lübeck) *Bob Ward* (University of Utah) *Ron Ariagno* (Stanford) *Jon Davis* (Tufts University, INC Co-Director) *Merran Thomson* (Chiesi, Hillingdale Hospital NHS Trust)	
	Regulatory Challenges in Conducting Trials to Prevent Pre-Term Birth – *Mark Turner*, chair (Cabin John Boardroom) *Olof Rugarn* (Ferring Pharmaceuticals) *Yosuke Komatsu* (GSK) *Louise Kenny* (University College Cork) *Deborah Discenza* (PPA) *Ralph Bax* (EMA) *Barbara Wesley* (FDA) *Tonse Raju* (NICHD/NIH)	
5:00 – 5:30 pm	**Report out on Breakouts** – *Ronald Portman*, chair 5-minute summary from each breakout group	
5:30 – 5:45 pm	**Role of INC in Pediatric Trial Networks** **I-ACT for Children** – *Edward Connor* (PTC, I-ACT) **IMI2 Pediatric Trial Network** – *William Treem* (Janssen)	

5:45 – 6:00 pm	**Nonclinical Models of Neonatal Therapeutics** *Susan McCune* (FDA; DART committee of ILSI HESI)	
6:00 pm	**Closing Remarks** – *Mark Turner* (INC Co-Director)	
6:30 pm	**Networking Dinner at Bethesda North Marriott Hotel**	

Agenda – Day 3, March 29, 2017

8:00 am – 12:00 pm (Brookside A)	**INC Bronchopulmonary Dysplasia Workgroup Meeting**
8:30 am – 4:30 pm (Brookside B)	**INC Data Workgroup Meeting**
9:00 am – 4:30 pm (Seneca Boardroom)	**INC Hemodynamic Adaptation Workgroup Meeting**