

2019 Annual INC Neonatal Scientific Workshop

May 1-3, 2019

Hyatt Regency Bethesda, Maryland

Meeting Objectives:

- Share regulatory perspectives on developing safe and effective therapies for neonates.
- Provide foundational knowledge on innovative, regulated medicines development and factors influencing Go/No-Go decisions throughout the development pipeline.
- Discuss endpoints for clinical trials to evaluate pain medications.
- Address neonatal opioid issues through optimizing trial design for Neonatal Opioid Withdrawal Syndrome (NOWS) and Neonatal Abstinence Syndrome (NAS).
- Apply digital technologies to developing endpoints for neonatal trials.
- Review the progress and plans of INC workstreams, including 1) validating the neonatal adverse event scale; 2) developing neonatal terminology; 3) standardizing safety reporting; 4) constructing a communications toolkit for use in NICUs; 5) establishing blood pressure values and methodologies.

Day 1, May 1, 2019


| Morning Sessions for Individual Workgroups | |
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| 8:00 am – 12:00 pm | INC Hemodynamic Adaptation Workgroup Meeting <i>Heike Rabe & Janis Dionne, Co-chairs</i> Room – Old Georgetown |
| 8:00 am – 12:00 pm | INC Communications Workgroup Meeting <i>Christina Bucci-Rechtweg & Jennifer Degl, Co-chairs</i> Room – Congressional |
| 12:00 – 1:00 pm | Lunch |
| Full Workshop Commences – Room Regency I & II | |

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| 1:00 – 1:05 pm | Welcome <i>Joseph Scheeren</i> , President and CEO, Critical Path Institute | Slide presentations  |
| 1:05 – 1:15 pm | INC at Four Years: Neonatal Growth and Predicted Long-Term Outcomes <i>Susan McCune</i> , Director, Office of Pediatric Therapeutics, FDA | |
| 1:15 pm – 2:30 pm | Educating the Neonatology Community on the Drug Development Process <i>Ron Portman</i> (Novartis, INC Co-Director) Session I: Setting the Scene <i>Gerri Baer</i> (OC/FDA), Chair <i>Robert Ward</i> (U-Utah) <i>Susan McCune</i> (OC/FDA) <i>Laura Fabbri</i> (Chiesi) | |
| 2:30 – 3:00 pm | Coffee Break | |
| 3:00 – 4:25 pm | Session II: Harnessing the Science <i>Christina Bucci-Rechtweg</i> (Novartis), Chair <i>Norman Barton</i> (Takeda) <i>Chris Milne</i> (Tufts CSDD) <i>Thomas Miller</i> (Bayer) |  |
| 4:25 – 5:30 pm | Session III: Addressing Neonatal Needs within the Current Environment <i>Ed Connor</i> (I-ACT for Children), Chair Panel Discussants to include: <i>Susan McCune</i> (OC/FDA) <i>Agnes Klein</i> (Health Canada) <i>Aprile Pilon</i> (Trove Therapeutics) <i>Mary Short</i> (Lilly) <i>Mark Turner</i> (U- Liverpool) <i>Jennifer Degl</i> (Speaking for Moms and Babies, Inc) <i>Anne Zajicek</i> (NIH/OD) <i>Kelle Moley</i> (March of Dimes) <i>James Baumberger</i> (AAP) | |
| Networking Dinner | | |
| 6:00 – 9:00 pm | DINNER at Bacchus of Lebanon for all Workshop Participants 7945 Norfolk Ave., Bethesda | |

Day 2, May 2, 2019

Regency I & II

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| 8:00 – 8:15 am | <p>Challenges of Developing Analgesics and Treatments for NOWS in the Setting of the Opioid Crisis <i>Sharon Hertz</i>, Director of Anesthesia, Analgesia, and Addiction Products (CDER/FDA)</p> | <p>Slide presentations</p>  |
| 8:15 – 10:00 am | <p>Endpoints for Evaluating Pain Treatments for Neonates <i>John Van den Anker</i> (Children’s National Medical Center and University of Basel Children’s Hospital) and <i>Lily Mulugeta</i> (CDER/FDA), Co-chairs <i>Lisa Wiltrout</i> (CDER/FDA) <i>Lynne Yao</i> (CDER/FDA) <i>Ralph Bax</i> (EMA), by Webex <i>Rebecca Slater</i> (U-Oxford) <i>Gary Walco</i> (U-Washington) <i>Edress Darsey</i> (Pfizer) <i>Dick Tibboel</i> (Erasmus MC-Sophia Children’s Hospital)</p> | |
| 10:00 – 10:30 am | <p>Coffee Break</p> | |
| 10:30 am – 12:30 pm | <p>Optimizing Clinical Trials for Neonatal Opioid Withdrawal Syndrome (NOWS) and Neonatal Abstinence Syndrome (NAS) <i>Gerri Baer</i> (OC/FDA) and <i>Jon Davis</i> (Tufts U and INC Co-Director), Co-chairs <i>Hendree Jones</i> (U-North Carolina) <i>Gioia Guerrieri</i> (CDER/FDA) <i>Ju Lee Oei</i> (U – New South Wales) <i>Thierry Lacaze</i> (U-Calgary, Alberta) <i>Merran Thomson</i> (Chiesi consultant) <i>Norma Terrin</i> (Tufts U) <i>Walter Kraft</i> (Thomas Jefferson U) <i>Victoria Kuck</i> (Parent representative)</p> | <p>Slide presentations</p>  |
| 12:30 – 1:30 pm | <p>Lunch</p> | |

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| 1:30 – 3:00 pm | <p>Applying Digital Technologies to Developing Endpoints for Neonatal Trials <i>Mark Turner</i> (U-Liverpool and INC Co-Director) and <i>Prakesh Shah</i> (Mt. Sinai and U-Toronto), Co-chairs <i>Joseph Frassica</i> (Philips) <i>Leonard Sacks</i> (CDER/FDA) <i>Doug Silverstein</i> (CDRH/FDA) <i>Chris McKinlay</i> (U-Auckland) <i>Wes Onland</i> (Emma Children’s Hospital AMC, Amsterdam) <i>Elin Haf Davies</i> (Aparito)</p> | <p>Slide presentations Joseph Frassica presentation</p>  |
| 3:00 – 3:30 pm | <p>Coffee Break</p> | |
| 3:30 – 4:45 pm | <p>INC Workstream Updates <i>Ronald Portman</i> (Novartis, INC Co-Director), Chair Hemodynamic Adaptation (<i>Stephen Bremner</i> – Brighton Sussex Medical School, <i>Simin Baygani</i> – Eli Lilly and Company)</p> <p>Data: Terminology (<i>Michael Padula</i>, Children’s Hospital of Pennsylvania)</p> <p>Adverse Event Severity Scale (<i>Karel Allegaert</i> – University of Leuven)</p> <p>Standardizing Safety Reporting (<i>Gerri Baer</i> – FDA, <i>Jon Davis</i> – Tufts U)</p> <p>Communications (<i>Christina Bucci-Rechtweg</i> – Novartis, <i>Jennifer Degl</i> – Speaking for Moms and Babies)</p> | |
| 4:45 – 5:00 pm | <p>Closing Remarks <i>Jon Davis</i> (Tufts U and INC Co-Director)</p> | |
| 5:00 pm | <p>Full Workshop Adjourns</p> | |

Day 3, May 3, 2019 – for Individual Workstreams

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| 8:00 am – 12:00 pm | Safety <i>Jon Davis, Karel Allegaert, Robert Ward, and Gerri Baer, Co-Chairs</i> Room – Old Georgetown <ul style="list-style-type: none">• Standardizing Safety Reporting• Prospective Validation of the Neonatal Adverse Event Severity Scale• Retrospective Validation of the Neonatal Adverse Event Severity Scale |
| 8:00 am – 12:00 pm | Neonatal Terminology <i>Mike Padula & Mark Turner, Co-chairs</i> Room – Congressional |