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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
</table>
| 8:00 am – 12:00 pm | **INC Hemodynamic Adaptation Workgroup Meeting**  
|                | *Heike Rabe & Janis Dionne*, Co-chairs       |
|                | Room – Old Georgetown                        |
| 8:00 am – 12:00 pm | **INC Communications Workgroup Meeting**    
<p>|                | <em>Christina Bucci-Rechtweg &amp; Jennifer Degl</em>, Co-chairs|
|                | Room – Congressional                         |
| 12:00 – 1:00 pm | <strong>Lunch</strong>                                    |
|               | <strong>Full Workshop Commences – Room Regency I &amp; II</strong> |</p>
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
<tbody>
<tr>
<td>1:00 – 1:05 pm</td>
<td><strong>Welcome</strong> &lt;br&gt;Mother Scheeren, President and CEO, Critical Path Institute</td>
</tr>
<tr>
<td>1:05 – 1:15 pm</td>
<td><strong>INC at Four Years: Neonatal Growth and Predicted Long-Term Outcomes</strong> &lt;br&gt;<em>Susan McCune</em>, Director, Office of Pediatric Therapeutics, FDA</td>
</tr>
<tr>
<td>1:15 pm – 2:30 pm</td>
<td><strong>Educating the Neonatology Community on the Drug Development Process</strong> &lt;br&gt;<em>Ron Portman</em>, (Novartis, INC Co-Director) &lt;br&gt;<strong>Session I: Setting the Scene</strong> &lt;br&gt;<em>Gerri Baer</em> (OC/FDA), Chair &lt;br&gt;<em>Robert Ward</em> (U-Utah) &lt;br&gt;<em>Susan McCune</em> (OC/FDA) &lt;br&gt;<em>Laura Fabbri</em> (Chiesi)</td>
</tr>
<tr>
<td>2:30 – 3:00 pm</td>
<td><strong>Coffee Break</strong></td>
</tr>
<tr>
<td>3:00 – 4:25 pm</td>
<td><strong>Session II: Harnessing the Science</strong> &lt;br&gt;<em>Christina Bucci-Rechtweg</em> (Novartis), Chair &lt;br&gt;<em>Norman Barton</em> (Takeda) &lt;br&gt;<em>Chris Milne</em> (Tufts CSDD) &lt;br&gt;<em>Thomas Miller</em> (Bayer)</td>
</tr>
<tr>
<td>4:25 – 5:30 pm</td>
<td><strong>Session III: Addressing Neonatal Needs within the Current Environment</strong> &lt;br&gt;<em>Ed Connor</em> (I-ACT for Children), Chair &lt;br&gt;<strong>Panel Discussants to include:</strong> &lt;br&gt;<em>Susan McCune</em> (OC/FDA) &lt;br&gt;<em>Agnes Klein</em> (Health Canada) &lt;br&gt;<em>Aprile Pilon</em> (Trove Therapeutics) &lt;br&gt;<em>Mary Short</em> (Lilly) &lt;br&gt;<em>Mark Turner</em> (U- Liverpool) &lt;br&gt;<em>Jennifer Degl</em> (Speaking for Moms and Babies, Inc) &lt;br&gt;<em>Anne Zajicek</em> (NIH/OD) &lt;br&gt;<em>Kelle Moley</em> (March of Dimes) &lt;br&gt;<em>James Baumberger</em> (AAP)</td>
</tr>
<tr>
<td>6:00 – 9:00 pm</td>
<td><strong>Networking Dinner</strong> &lt;br&gt;DINNER at Bacchus of Lebanon for all Workshop Participants &lt;br&gt;7945 Norfolk Ave., Bethesda</td>
</tr>
</tbody>
</table>

**Day 2, May 2, 2019**

**Regency I & II**
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
</table>
| 8:00 – 8:15 am | **Challenges of Developing Analgesics and Treatments for NOWS in the Setting of the Opioid Crisis**  
*Sharon Hertz, Director of Anesthesia, Analgesia, and Addiction Products (CDER/FDA)* |
| 8:15 – 10:00 am | **Endpoints for Evaluating Pain Treatments for Neonates**  
*John Van den Anker* (Children’s National Medical Center and University of Basel Children’s Hospital)  
and *Lily Mulugeta* (CDER/FDA), Co-chairs  
*Lisa Wiltrout* (CDER/FDA)  
*Lynne Yao* (CDER/FDA)  
*Ralph Bax* (EMA), by Webex  
*Rebecca Slater* (U-Oxford)  
*Gary Walco* (U-Washington)  
*Edress Darsey* (Pfizer)  
*Dick Tibboel* (Erasmus MC-Sophia Children’s Hospital) |
| 10:00 – 10:30 am | **Coffee Break** |
| 10:30 am – 12:30 pm | **Optimizing Clinical Trials for Neonatal Opioid Withdrawal Syndrome (NOWS) and Neonatal Abstinence Syndrome (NAS)**  
*Gerri Baer* (OC/FDA) and *Jon Davis* (Tufts U and INC Co-Director), Co-chairs  
*Hendree Jones* (U-North Carolina)  
*Gioia Guerrieri* (CDER/FDA)  
*Ju Lee Oei* (U – New South Wales)  
*Thierry Lacaze* (U-Calgary, Alberta)  
*Merran Thomson* (Chiesi consultant)  
*Norma Terrin* (Tufts U)  
*Walter Kraft* (Thomas Jefferson U)  
*Victoria Kuck* (Parent representative) |
<p>| 12:30 – 1:30 pm | <strong>Lunch</strong> |</p>
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
</table>
| 1:30 – 3:00 pm| **Applying Digital Technologies to Developing Endpoints for Neonatal Trials**  
**Mark Turner** (U-Liverpool and INC Co-Director) and **Prakesh Shah** (Mt. Sinai and U-Toronto), Co-chairs  
**Joseph Frassica** (Philips)  
**Leonard Sacks** (CDER/FDA)  
**Doug Silverstein** (CDRH/FDA)  
**Chris McKinlay** (U-Auckland)  
**Wes Onland** (Emma Children’s Hospital AMC, Amsterdam)  
**Elin Haf Davies** (Aparito)  
**Slide presentations Joseph Frassica presentation** |
| 3:00 – 3:30 pm| **Coffee Break**                                                        |
| 3:30 – 4:45 pm| **INC Workstream Updates**  
**Ronald Portman** (Novartis, INC Co-Director), Chair  
**Hemodynamic Adaptation** (**Stephen Bremner** – Brighton Sussex Medical School, **Simin Baygani** – Eli Lilly and Company)  
**Data: Terminology** (**Michael Padula**, Children’s Hospital of Pennsylvania)  
**Adverse Event Severity Scale** (**Karel Allegaert** – University of Leuven)  
**Standardizing Safety Reporting** (**Gerri Baer** – FDA, **Jon Davis** – Tufts U)  
**Communications** (**Christina Bucci-Rechtweg** – Novartis, **Jennifer Degl** – Speaking for Moms and Babies) |
| 4:45 – 5:00 pm| **Closing Remarks**                                                     
**Jon Davis** (Tufts U and INC Co-Director) |
| 5:00 pm      | **Full Workshop Adjourns**                                             |

**Day 3, May 3, 2019 – for Individual Workstreams**
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Co-Chairs</th>
</tr>
</thead>
</table>
| 8:00 am – 12:00 pm | **Safety**  
*Jon Davis, Karel Allegaert, Robert Ward, and Gerri Baer, Co-Chairs*  
Room – Old Georgetown  
- 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**  
*Mike Padula & Mark Turner, Co-chairs*  
Room – Congressional |