Final Agenda

Day 1: Session 1 and 2 – Rare diseases

- Success stories and lessons learned...using old drugs in novel new ways.
- Stakeholder perspectives and viewpoints.
- Opportunities for repurposing drugs in rare diseases and rare cancers.
- Using real-world data, what process should be used to determine whether the signal should be confirmed in a randomized clinical trial.

Day 1: session 1
November 16, 2021 – (9:00 am to 11:00 am Eastern Time)

Topic: Rare Diseases

Moderators: Matt Might (UAB) + Shira Strongin (FDA)

Panelists:
1. Andy Crouse (UAB)
2. Ethan Perlstein (Perlara)
3. Eva Morava-Kozicz (Mayo)
4. Lisa Schill (RASopathies Network)
5. Omid Karkouti (Rarebase)
6. Clare Thibodeaux (Cures Within Reach)
7. Sandra Sermone (ADNP Kids Research Foundation)

Discussion Topics: TBA

<table>
<thead>
<tr>
<th>Presenter</th>
<th>Affiliation</th>
<th>Presentation title</th>
</tr>
</thead>
<tbody>
<tr>
<td>Matthew Might</td>
<td>UAB</td>
<td>Repurposing Case Study</td>
</tr>
<tr>
<td>Shira Strongin</td>
<td>FDA</td>
<td>Disease of Interest Summary</td>
</tr>
<tr>
<td>Sandra Sermone</td>
<td>ADNP Kids Research Foundation</td>
<td>AI tool predicted ketamine may help ADNP-driven autism</td>
</tr>
<tr>
<td>Ethan Perlstein</td>
<td>Perlara PBC</td>
<td>Repurposed drug to successfully treat PMM2-CDG with epalrestat – Industry perspective</td>
</tr>
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<td>Eva Morava -Kozicz</td>
<td>Mayo</td>
<td>Repurposed drug to successfully treat PMM2-CDG with epalrestat – Clinician perspective</td>
</tr>
</tbody>
</table>
Native text for Day 1: session 2
November 16, 2021 – (3:00 pm to 5:00 pm Eastern Time)

**Topic:** Rare Oncology

**Moderators:** Leslie Doros (FDA) and Sonia Singh (FDA)

**Panelists:**

1. Bill Tap (MSKCC)
2. Denise Reinke (U Michigan)
3. Kris Ann Schultz (Children’s MN)
4. Pan Pantziarka (Anticancer Fund)
5. Breelyn Wilky (U Colorado)

**Discussion Topics:** TBA

<table>
<thead>
<tr>
<th>Presenter</th>
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</tr>
</thead>
<tbody>
<tr>
<td>Leslie Doros</td>
<td>FDA</td>
<td>Introduction and background for sarcomas</td>
</tr>
<tr>
<td>Bill Tap</td>
<td>MSKCC</td>
<td>The use of repurposed drugs in sarcoma and current examples</td>
</tr>
<tr>
<td>Denise Reinke</td>
<td>U Michigan</td>
<td>Patient perspective on drug repurposing for sarcoma</td>
</tr>
<tr>
<td>Kris Ann Schultz</td>
<td>Children’s MN</td>
<td>Registries for rare tumors and CRFs</td>
</tr>
<tr>
<td>Pan Pantziarka</td>
<td>Anticancer Fund</td>
<td>Propranolol in Angiosarcoma - the story so far</td>
</tr>
</tbody>
</table>
Day 2: Session 3 and 4 – Special Populations and Regulatory Challenges

- Use of real-world data when clinical trials are not feasible due to loss of equipoise.
- Real-world data sources other than medical records, registries, and observational studies.
- Programs initiated by regulatory authorities to advance drug repurposing.
- What other opportunities such as financial incentives, patent law, and legislative action should be considered to help advance drug repurposing and potential unintended consequences.

Day 2: session 3
November 17, 2021 – (9:00 am to 11:00 am Eastern Time)

Topic: Special Populations

Moderators: Matt Laughon (UNC) + Kate Borkowski (FDA)

Panelists:
1. Anup Challa (Vanderbilt)
2. Rachel Greenberg (Duke)
3. Mili Duggal (FDA)
4. Matt Robinson (Hopkins)
5. Khyzer Aziz (Hopkins)
6. Perdita Taylor-Zapata (NICHD)
7. Kanwaljit Singh (INC)
8. Prabha Viswanathan (FDA)
9. Genny Taylor (UNC)
10. David Kimberlin (UAB)
11. Jason Lang (Duke)
12. Barbara Goodman (Cures Within Reach)

Discussion Topics: TBA

<table>
<thead>
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</tr>
</thead>
<tbody>
<tr>
<td>Kate Borkowski</td>
<td>FDA</td>
<td>Ranking diseases in pregnancy for drug repurposing</td>
</tr>
<tr>
<td>David Kimberlin</td>
<td>UAB</td>
<td>Congenital CMV treatment options and existing guidelines/ Standing up a trial for valganciclovir, and FDA &amp; BPCA</td>
</tr>
<tr>
<td>Jason Lang</td>
<td>Duke</td>
<td>Efficacy of montelukast in NICU for bronchopulmonary dysplasia</td>
</tr>
<tr>
<td>Mili Duggal</td>
<td>FDA</td>
<td>The need to develop survey tools</td>
</tr>
<tr>
<td>Rachel Greenberg</td>
<td>Duke</td>
<td>Accomplishments and challenges in pediatric drug development: The Pediatric Trials Network experience</td>
</tr>
<tr>
<td>Genny Taylor</td>
<td>UNC</td>
<td>Global Rank Score for use in real-world data</td>
</tr>
</tbody>
</table>
**Day 2: session 4**  
**November 17, 2021 – (3:00 pm to 5:00 pm Eastern Time)**

**Topic:** Regulatory

**Moderators:** David Simon (Harvard) + Marco Schito (C-Path)

**Panelists:**

1. Jonathan Darrow (Harvard)
2. John Liddicoat (Cambridge)
3. Sundeep Agrawal (FDA)
4. Daniel O’Connor (MHRA)
5. Lydie Meheus (Anticancer Fund)
6. Heather Stone (FDA)
7. Perdita Taylor-Zapata (NICHD)
8. Nitin Bagul (TGA)
9. César Hernandez Garcia (AEMPS Spain)
10. Momir Radulović (Slovenian Medicines Agency)
11. Agnes Klein (Health Canada)

**Discussion Topic:** TBA

<table>
<thead>
<tr>
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</tr>
</thead>
<tbody>
<tr>
<td>Marco Schito</td>
<td>C-Path</td>
<td>Introduction and Workgroup Goals</td>
</tr>
<tr>
<td>Jonathan Darrow</td>
<td>Harvard</td>
<td>Issues regarding drug repurposing</td>
</tr>
<tr>
<td>John Liddicoat</td>
<td>Cambridge</td>
<td>Closing the repositioning gap with a resource hidden in plain site</td>
</tr>
<tr>
<td>Sundeep Agrawal</td>
<td>FDA</td>
<td>Project Renewal</td>
</tr>
<tr>
<td>Daniel O’Connor</td>
<td>MHRA</td>
<td>ILAP initiative</td>
</tr>
<tr>
<td>Lydie Meheus</td>
<td>Anticancer fund</td>
<td>Role of non-profits in advancing drug repurposing</td>
</tr>
</tbody>
</table>
Lessons learned from the COVID-19 VISUR disease registry.
Challenges in automating data extraction from electronic health records from US and non-US sites.
Opportunities and challenges in creating knowledgebases using automated data extraction from electronic health record data to identify how existing drugs can be optimized in different health care settings.
Despite recent advances, what barriers exist that prevent optimizing data sharing and what are the prospects for innovation.

**Day 3: session 5**

November 18, 2021 – (9:00 am to 12:00 pm Eastern Time)

**Topic: Electronic Health Records**

**Panel 1**

**Moderators:** Smith Heavner (C-Path) + Aysun Tekin (Mayo Clinic)

**Panelists:**
1. Rahul Kashyap (VIRUS Registry)
2. Matt Robinson (JHU)
3. Paul Nagy (JHU)
4. Matt Roe (Verana Health)
5. Laura Merson (Oxford)

**Discussion Topic:** “Lessons Learned in Registry Development”

- Selection of variables, data automation, site recruitment and retention, interpandemic period, challenges and regulatory barriers

**Panel 2**

**Moderator:** Laura Merson (Oxford) + Smith Heavner (C-Path)

**Panelist:**
1. Will Stevens (RECOVERY Trial, University of Oxford)
2. Ann-Marie Mallon (NHS Digital)
3. Kalynn Kennon (IDDO)
4. Miguel Pedrera Jimenez (Hospital 12 de Octubre, Madrid, Spain)

**Discussion Topic:** “Perspectives from Across the Pond”
- Mapping EHR data, data integration, use of EHR RWD for clinical trials and observational research, limitations of EHR for research questions

<table>
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<tbody>
<tr>
<td>Laura Merson</td>
<td>Oxford (UK)</td>
<td>International Perspective Barriers to Automating ISARIC/RECOVER</td>
</tr>
<tr>
<td>Miguel Pedrera Jimenez</td>
<td>Hospital Universitario, Madrid, Spain</td>
<td>Mapping EHR data to inform clinical characterization</td>
</tr>
<tr>
<td>Will Stevens</td>
<td>RECOVERY</td>
<td>Combining RWD and case report forms to deliver the RECOVERY trial</td>
</tr>
<tr>
<td>Paul Nagy, Matt Robinson</td>
<td>JHU</td>
<td>The Edge Tool</td>
</tr>
<tr>
<td>Rahul Kashyap, Smith Heavner</td>
<td>VIRUS</td>
<td>Automating the VIRUS COVID-19 Registry</td>
</tr>
</tbody>
</table>