



Symposium agenda & concept (V.10) – Day 1 (September 16, 2024)

Content	Speakers / Panelists
<b>12:00 - 13:00 (CET) Check-in &amp; Welcome Beverages</b>	
<b>13:00 – 13:15 (CET)</b> <b>Welcome</b>	<ul style="list-style-type: none"> <li>• Emer Cooke (EMA) &amp; Raimund Bruhin (Swissmedic)</li> <li>• Claus Bolte (Swissmedic) &amp; Kerry Jo Lee (FDA)</li> </ul>
<b>13:15 – 14:30 (CET)</b> <b>Keynote 1</b> Clinical Focus (High-level Overviews) <ul style="list-style-type: none"> <li>• More opportunities than challenges today!</li> <li>• Clinical &amp; translational research</li> <li>• Pharma R&amp;D (clinical development)</li> <li>• Pragmatic evidence generation in routine care</li> </ul>	<ul style="list-style-type: none"> <li>• Maria Ester Bernardo, Pediatrician (I) (<a href="#">Link</a>)</li> <li>• Tiina K. Urv, NIH (US) (<a href="#">Link</a>)</li> <li>• Thomas F. Miller, Industry (US), InsideScientific (<a href="#">Link</a>)</li> <li>• Lars Hemkens (CH), DKF USB, Pragmatic Evidence Lab (<a href="#">Link</a>)</li> </ul>
<b>14:30 – 15:10 (CET)</b> <b>Panel 1</b> Evidence Generation <ul style="list-style-type: none"> <li>• Academic clinicians and investigators</li> <li>• (Ultra-)Orphan Drug Designation?</li> </ul>	<ul style="list-style-type: none"> <li>• Keynote 1 speakers plus</li> <li>• Daniel Michaeli (D) Nat. Center for Tumor Diseases (<a href="#">Link</a>)</li> </ul>
<b>15:10 – 15:40 (CET) Brain &amp; Bio Break</b>	
<b>15:40 – 16:55 (CET)</b> <b>Keynote 2</b> Digital Health Technologies (DHT) <ul style="list-style-type: none"> <li>• Showcasing exciting tech developments</li> <li>• How rare diseases became common</li> <li>• (What) can we learn from Oncology?</li> <li>• Linking regulators and tech</li> </ul>	<ul style="list-style-type: none"> <li>• Jennifer Goldsack (US), Digital Medicine Society (<a href="#">Link</a>)</li> <li>• Dan O'Connor (UK), ABPI (<a href="#">Link</a>)</li> <li>• Blythe Adamson (US) (<a href="#">Link</a>)</li> <li>• Klaus Romero (US), Critical Path (<a href="#">Link</a>)</li> </ul>
<b>16:55 – 17:35 (CET)</b> <b>Panel 2</b> Integrating DHT <ul style="list-style-type: none"> <li>• How can DHT help (recruitment, data)?</li> <li>• Regulatory Science evolving</li> <li>• Low- and middle-income countries</li> </ul>	<ul style="list-style-type: none"> <li>• Keynote 2 speakers plus</li> <li>• Elisabeth Kunkoski, FDA (<a href="#">Link</a>)</li> <li>• Steffen Thirstrup, CMO EMA (<a href="#">Link</a>)</li> </ul>
<b>17:35 – 17:50 (CET) Brain &amp; Bio Break</b>	
<b>17:50 – 18:35 (CET)</b> <b>Lakeside chat with caregivers</b> <ul style="list-style-type: none"> <li>• How to better appreciate and integrate their journeys and experience?</li> </ul>	<ul style="list-style-type: none"> <li>• Bob Stevens (UK), MPS Society (<a href="#">Link</a>)</li> <li>• Christoph Poincilit (CH), NPD Suisse (<a href="#">Link</a>)</li> <li>• Klaus Rose (CH), Pediatrics, Sturge-Weber syndrome (<a href="#">Link</a>)</li> <li>• Julia Vitarello (US), Mila's Miracle Foundation (<a href="#">Link</a>)</li> </ul>
<b>18:35 – 18:45 (CET)</b> <b>Wrap-up</b>	<ul style="list-style-type: none"> <li>• Claus Bolte (Swissmedic) &amp; Kerry Jo Lee (FDA)</li> </ul>
<b>Adjourn @18:45 (CET)</b> (ICMRA Steering Committee & Faculty Dinner @20:00 (CET))	