



Symposium draft agenda & concept (V.7) – Day 1 (September 16, 2024)

Content	Speakers / Panelists
12:00 - 13:00 (CET) Check-in & Welcome Beverages	
13:00 - 13:15 (CET) Welcome	<ul style="list-style-type: none">• Agency & ICMRA Head (tbc)• Claus Bolte (Swissmedic) & Kerry Jo Lee (FDA)
13:15 - 14:30 (CET) Keynote 1 Clinical Focus <ul style="list-style-type: none">• More opportunities than challenges today!• How to utilize registries• Basic & translational research• Pharma R&D (clinical development)	Keynote 1 High-level Overviews <ul style="list-style-type: none">• Alessandro Aiuti, Immunologist (I), HSR Research (Link)• Bernhard Landwehrmeyer, Neurologist (D) (Link)• Tiina K. Urv, NIH (US) (Link)• Thomas F. Miller, Industry (US), InsideScientific (Link)
14:30 - 15:15 (CET) Panel 1 Evidence Generation <ul style="list-style-type: none">• Academic clinicians and investigators• Lessons learned, suggestions• (Ultra-)Orphan Drug Designation?	Panel 1 <ul style="list-style-type: none">• Keynote 1 speakers plus• Lars Hemkens (CH) Pragmatic Evidence Lab (Link)• Daniel Michaeli (D) Nat. Center for Tumor Diseases (Link)
15:15 - 15:30 (CET) Brain & Bio Break	
15:30 - 16:45 (CET) Keynote 2 Moving the needle? <ul style="list-style-type: none">• Showcasing exciting tech developments• How rare diseases became common• (What) can we learn from Oncology?• Linking regulators and tech	Keynote 2 Digital Health Technologies <ul style="list-style-type: none">• Jennifer Goldsack (US), Digital Medicine Society (Link)• Dan O'Connor (UK), ABPI (Link)• Blythe Adamson (US) (Link)• Klaus Romero (US), Critical Path (Link)
16:45 - 17:30 (CET) Panel 2 Integrating DHT <ul style="list-style-type: none">• How can DHT help (recruitment, data)?• Regulatory Science evolving• Low- and middle-income countries	Panel 2 <ul style="list-style-type: none">• Keynote 2 speakers plus• Elisabeth Konkoski, (US) FDA (Link)• EU and RoW Regulator
17:30 - 17:45 (CET) Brain & Bio Break	
17:45 - 18:30 (CET) Lakeside chat with caregivers <ul style="list-style-type: none">• How to better appreciate and integrate their journeys and experience?	<ul style="list-style-type: none">• Bob Stevens (UK), MPS Society (Link)• Christoph Poincilit (CH), NPD Suisse (Link)• Klaus Rose (CH), Pediatrics, Sturge-Weber syndrome (Link)• Julia Vitarello (US), Mila's Miracle Foundation (Link)
18:30 - 18:40 (CET) Wrap-up	<ul style="list-style-type: none">• Claus Bolte (Swissmedic) & Kerry Jo Lee (FDA)
Adjourn @18:45 (CET) (ICMRA Steering Committee & Faculty Dinner @20:00 (CET))	