6th Nordic Conference on Pediatric and Orphan Medicines
3-4 June 2025
Helsinki, Finland

Pediatric and Orphan Clinical Trials in Practice

Venue and Lecture Room: The University of Helsinki, Swedish School of Social Science (Svenska social- och kommunalhögskolan), Festsal, Snellmaninkatu 12, 00170 Helsinki, Finland.

Day 1: Regulatory Aspects and New Approaches to Clinical Trials

12:50 Welcome and practicalities, Mia Bengtström, Åbo Akademi University, University of Helsinki

Session 1: Legislative, Regulatory and Ethical updates

Chair: Kaisa Sunela, Head of Section, Clinical Assessor, Finnish Medicines Agency - Fimea

13:00 EU Legislation on Rare Diseases and Cross-Border Access – EU Parliament, MP, Stine Bosse, Denmark

13:25 *Regulatory update – Mode of Action and stepwise PIP - TBC, EMA PDCO*

13:50 MedEthics EU- Co-chair MedEthicsEU, Special advisor CCMO, Monique Al, Netherlands

14:15 *HTA Regulation 2025 implementation - pediatric & orphan medicines, TBC*

14.40-15:20 Coffee, Exhibition & Networking

Session 2: New Approaches to Clinical Trial Conduction

Chair: Outi Konttinen, General Secretary, National Committee on Medical Research Ethics - Tukija

15.20 *Joint EFPIA-EFGCP Cross Border initiative - recommendation – TBC*

15.45 *Cross-Border Clinical Trials - practical and operational aspects for easy access to participate, TBC*

16.10 Decentralized Clinical Trials (DCT) – EMA recommendation, Monique Al, Netherlands

16:35 New Trial Designs – Platform Trials – MD Mimi Kærsgaard, Clinical Trials Unit, Rigshospitalet, Denmark

17:00 Panel discussion about DCT, Platform trials and Cross-Border Trial practicalities of trial conduction

- Academic speaker, Company, Regulatory Agency, Ethics Committee

17:30 End of the Day 1, Conclusions Day 1, guidance to City Hall, Chair

17:40 - *Walking 15 min. to Old City Hall*

Networking Event

18:00 – 19:00 Networking Event – Reception Hosted by the City of Helsinki

Venue and Address: Old Court House, Aleksanterinkatu 20, 00100 Helsinki

Day 2: New Practical Tools, Models and Solutions to Support Trial Conduction

Session 3: Enhancing Pediatric and Orphan Clinical Trials in Practice

Chair: *TBC*

9:00 Welcome to Day 2, Chair

9:05 End of Pan-European c4c initiative – Experiences – Heidrun Hildebrandt, Alliance Manager Pediatric Development, Bayer, Germany

9:30 Site Quality Criteria for Pediatric Trial sites - Enpr-EMA – Pernille Skovby, National Coordinator DanPedMed, Denmark

9:50 *Pediatric CTU – new operational and clinical trial models – Academic speaker, TBC*

10:15 Advocating for Patients and Families – Sebastian Honoré, Rare Disease Dad & Advocate, Denmark

10:35 Protocol Digitalization (of the inclusion/exclusion criteria), Samu Kurki, Bayer

11:00-12:00 Lunch, Exhibition and Networking

Session 4: Finding the Right Patients, Dosing and Formulation

Chair: *TBC*

12:00 *Company topic - TBC*

12:25 Phase Appropriate Dosage Form Design for Paediatric Clinical Development Programs, Terry Ernest, Director, Manufacturing Science and Technology, Almac Pharma Services

12:45 Compounding System Solution for Paediatric Dosing and Treatments – Niklas Sandler, Founder and Chief Technology Officer, CurifyLabs, Finland

13:10 *Biobank / RWD / RWE /AI use to support clinical trials – TBC*

13.30 - 14.10 Coffee, Exhibition & Networking

Session 5: Innovative Solutions for Clinical Trials on Orphan Medicines

Chair: TBC

14:10 *Academic Orphan disease current topic – Academic speaker, TBC*

14:35 The establishment of a Norwegian Public-Private Partnership for Rare Diseases – MD Per Kristian Knudsen, Senior Consultant, Paediatric Clinical Research Unit, Oslo University Hospital, Norway

14:50 *Company topic - TBC*

15:15 Conference conclusions, next steps - thank you and goodbye!

15:30 End of the conference.