

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



Pediatric and Orphan Clinical Trials in Practice

Venue: The University of Helsinki, Helsinki, Finland.

Preliminary Topics, Times EET

Day 1: Regulatory Aspects and New Approaches to Clinical Trials (12.30 – 17.50)

Session 1: Legislative, Regulatory and Ethical updates

- EU Legislation - Perspectives on rare diseases and cross border access
- Regulatory update – stepwise PIP – Mode of Action
- Med Ethics EU - introduction
- HTA Regulation 2025 – pediatrics & orphans

Session 2: New Approaches to Clinical Trial Conduction

- Decentralized Clinical Trials – introduction
- Cross-Border Access to Clinical Trials
- New Trial Designs – Platform Trials etc.
- Panel – discussion (presented topics of Session 2)

17:50 End of Day 1 - 18.00 Networking Event at the Old City Hall hosted by the City of Helsinki

Day 2: New Practical Tools, Models and Solutions to Support Clinical Trials (08:30 - 16:00)

Session 3: Enhancing Pediatric and Orphan Clinical Trials in Practice

- End of Pan-European c4c network initiative – experiences
- Trial Site Quality Criteria for Pediatric Clinical Trials - Enpr-EMA
- Pediatric Clinical Trial Units – new operational models
- Patient and Families – ways for active involvement

Session 4: Finding the Right Patients, Targets and Dose

- Complex Drug Dosing
- Protocol Digitalization
- Utilizing Registries, Biobanks, Real World Data and AI

Session 5: Innovative Solutions for Clinical Trials on Orphan Medicines

- Current topics on Orphan diseases – Academic perspectives
- Orphan Diseases Projects
- Company examples of successful drug development for Unmet Medical Needs

16:00 End of the conference.