

Agenda - ERDERA Young Patients Training

Day 1 - Friday 2nd May, 2025

08:00 – 09:30 | Opening Session

- Welcome & introduction to the ERDERA training (15 mins)
- Welcome & introduction from the KIDS groups (30 mins)
- The role of advocacy and patient organizations (30 mins)
- Q&A and discussion (15 mins)

09:30 – 10:00 | Break

10:00 – 11:30 | Rare and Genetic Diseases Specificities

- Overview of rare and genetic diseases in pediatric patients (30 mins)
- Challenges in diagnosis, research and care (30 mins)
- Case studies and interactive discussion (30 mins)

11:30 – 12:00 | Break

12:00 – 13:30 | Patients' and Children's Rights

- Legal and ethical considerations in pediatric research (30 mins)
- The role of young patients in matters related to their health (30 mins)
- Group discussion and real-world examples (30 mins)

13:30 – 14:30 | Lunch Break

14:30 – 16:00 | Translational Research Challenges

- Innovative therapies; ethical and legal issues in pediatric research (45 mins)
- Unmet needs and prioritization in health and research decision-making (45 mins)
- Interactive session with case-based scenarios (30 mins)

16:00 – 16:30 | Break

16:30 – 18:00 | Activities

Day 2 - Saturday 3rd May, 2025

08:00 – 09:30 | Patient Engagement in Clinical Trials

- Involvement in CTs and innovative studies (PROMs, PREMs, etc.) (30 mins)
- Consent/assent, clinical study protocol, and lay summaries (30 mins)
- Q&A and participant reflections (30 mins)

09:30 – 10:00 | Break

10:00 – 11:30 | Orphan Medicinal Products & Pharmacovigilance

- Availability and access to orphan drugs in the EU (30 mins)
- Pharmacovigilance and off-label use in pediatric settings (30 mins)
- Group discussion and case analysis (30 mins)

11:30 – 12:00 | Break

12:00 – 13:30 | Closing Session

- Key takeaways from the training (40 mins)
- Participant feedback and evaluation (40 mins)
- Closing remarks (10 mins)

13:30 – 14:30 | Lunch Break



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