

OCTOBER 28TH • 29TH

TRAILS 2025



Imperial Riding School Autograph Collection
Ungargasse 60, 1030 Wien, Austria | Vienna

08.50 – 09.00 Opening – *F. Lagler*

Session 1 THE PATH TO DIAGNOSIS

Moderator: *M. Magner*

09.00 – 09.30 Early diagnosis in LSDs: strategies in clinical practice – *P. Oliva, A. Hermida*

09.30 – 09.50 Harnessing the power of national child health monitoring programs – *C. Lampe, F. Lagler*

09.50 – 10.10 Advancing diagnosis with AI
M. Scarpa (recorded speech)

10.10 – 11.00 On-line available tools to reduce diagnostic delays
M. Fruchart, F. AbdulBaqi Al Jasmi, L. Buch

11.00 – 11.20 **Coffee Break**

11.20 – 12.00 New born screening: Austrian and Taiwanese perspectives
Shuan-Pei Lin & Hsiang-Yu Lin, M. Zeyda

12.00 – 12.10 DISCUSSION - Q&A

Moderator: *M. Magner*

Discussants: *All Session Faculty*

Session 2 ESTABLISHED TREATMENTS AND THEIR CAVEATS

Moderator: *F. Lagler*

12.10 – 12.30 Alpha-Mannosidosis: from Velmanase Alfa long-term clinical trial data to real-world evidence
N. Guffon

12.30 – 12.45 Skeletal abnormalities in LSDs: addressing the unmet needs – *P. Kunkel*

12.45 – 13.00 DISCUSSION - Q&A

13.00 – 14.00 **Lunch**

14.00 – 15.00 Challenging scenarios in LSDs: sharing experiences

- Early treated – *Sarar Hamza Elhassan Mohamed*
- HSCT in MPS and AM – *M. Magner*
- Healthcare migration: implications in rare diseases – *S. Gasperini*

15.00 – 15.15 Synthetic control arms: a strategy for building evidence in rare disease – *A. Signori*

15.15 – 15.45 DISCUSSION - Q&A

Moderator: *F. Lagler*

Discussants: *All Session Faculty*

Session 3 THE MEANING OF MANAGEMENT

Moderator: *F. Lagler*

15.45 – 16.15 The PROMs value:

- In evaluation of therapeutic outcomes and quality of life in Lysosomal Storage Diseases
K. Stepien (from remote)
- In reimbursement decision – *C. Schwartz*

16.15 – 16.30 **Coffee Break**

16.30 – 17.30 Integrated patients' care:

- Behavioural and sleep disturbances – *J.B. Hennermann*
- Anaesthesiologic airway assessment – *W. Rhoads & S. Moser*
- Best practice from Brazilian "Casa dos raros" – *R. Giugliani (recorded speech)*

17.30 – 18.15 DISCUSSION - Q&A

Moderator: *F. Lagler*

Discussants: *All Session Faculty*

Session 4 NAVIGATING COMPLEX CLINICAL CASE**Moderator:** *F. Lagler***08.30 – 09.00** Introduction and key messages of day 1
*F. Lagler***09.00 – 12.15** Managing the Unexpected in MPS: Live Simulation of Critical Multidisciplinary Challenges
*F. Lagler, C. Lampe***12.15 – 12.45** DISCUSSION - Q&A**12.45 – 13.30** Lunch**Session 5 RARE AND AWARE****Moderator:** *D. Zafeiriou***13.30 – 14.45** ROUND TABLE: Patients' association groups: emerging stakeholder in promoting access, quality, and continuity of care.**Moderator:** *D. Zafeiriou***Speakers:** *M. Weigl, M. Prähofer, F. Lagler, A. Kerpauskiene, V. Plaiasu***14.45 – 15.15** Closing and remarks – *F. Lagler**Fatma AbdulBaqi Al Jasmi (United Arab Emirates)**Laura Buch (USA)**Mathilde Fruchart (France)**Serena Gasperini (Italy)**Nathalie Guffon (France)**Julia B. Hennermann (Germany)**Alvaro Hermida (Spain)**Agnė Kerpauskiene (Lithuania)**Philip Kunkel, (Germany)**Florian Lagler (Austria)**Christina Lampe (Germany)**Martin Magner (Czech Republic)**Sarar Hamza Elhassan Mohamed (Saudi Arabia)**Shuan-Pei Lin (Taiwan)**Hsiang-Yu Lin (Taiwan)**Petra Oliva (Austria)**Vasilica Plaiasu (Romania)**William Rhoads (USA)**Simon Moser (USA)**Maurizio Scarpa (Italy)**Alessio Signori (Italy)**Karolina Stepien (United Kingdom)**Carolyn Schwartz (USA)**Dimitrios Zafeiriou (Greece)**Maximillian Zeyda (Austria)***SCIENTIFIC COORDINATOR***Florian Lagler (Austria)***PATIENT FOR ROUND TABLE***Maria Prähofer (Austria)**Michaela Weigl (MPS Austria)***ACRONYM:****AM:** Alpha Mannosidosis, **MPS:** Mucopolysaccharidosis,
MDT: Multidisciplinary Team, **LSDs:** Lysosomal Storage Disorders,
ERT: Enzyme Replacement Therapy, **Q&A:** Question & Answer

CME Provider



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Organizing Secretariat

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