

# TRAILS 2024

3<sup>RD</sup> EDITION

ALPHA MANNOSIDOSIS AND MUCOPOLYSACCHARIDOSIS:  
OPTIMIZING PATIENT JOURNEYS

SCIENTIFIC  
PROGRAM

NOVEMBER 18<sup>TH</sup> - 19<sup>TH</sup>  
3<sup>RD</sup> EDITION 2024  
PRAGUE

Don Giovanni Hotel  
Vínohradská 157a, 130-20  
Prague

# NOVEMBER 18<sup>TH</sup>

08.20 – 08.30 Opening | M. Scarpa, M. Magner

## SESSION I *LSDs PATIENTS JOURNEY, FROM EARLY DIAGNOSIS TO APPROPRIATE TREATMENT*

**Moderators:** M. Scarpa, C. Lampe

08.30 – 09.00 Early diagnosis in LSDs: strategies and impact  
K. Stepien

09.00 – 09.10 Screening initiative for MPS and Alpha-mannosidosis at the regular pediatric examination at the age of 3.5 years in Germany  
C. Lampe

09.10 – 09.40 Newborn screening - best practices from Spanish LSDs scenario  
C. Colón

09.40 – 10.40 Integrated patient approach in LSDs to foster early diagnosis  
S. Rust, L. Buch, L. Berry

10.40 – 10.50 Q&A

10.50 – 11.10 Coffee Break

**Moderators:** M. Scarpa, F. Lagler

11.10 – 11.40 Giving voice to Patients and families treatment goals and expectations  
C. Lampe

11.40 – 12.35 Natural History of LSDs  
Nato V. Vashakmadze, Fatma Tuba Eminoğlu, V. Rovelli

12.35 – 12.50 DISCUSSION – Q&A  
**Moderator:** M. Scarpa  
**Discussants:** All Session Faculty

12.50 – 14.00 Lunch

## SESSION II – FROM SCIENTIFIC EVIDENCE TO CLINICAL PRACTICE

**Moderators:** M. Scarpa, M. Magner

14.00 – 15.00 Different therapeutic goals and options in LSDs in childhood and adulthood  
A. Broomfield, J. B. Hennermann

15.00 – 15.40 ERT long-term results: latest clinical evidence  
N. Guffon

15.40 – 16.00 Coffee Break

16.00 – 16.30 Late onset disease: diagnosis and treatment in adult life  
K. Stepien

16.30 – 17.00 DISCUSSION – Q&A

**Moderators:** M. Scarpa

**Discussants:** All session faculty

# NOVEMBER 19<sup>TH</sup>

## SESSION III CLINICAL PRACTICE

**Moderators:** M. Scarpa, C. Lampe

- 08.20 – 08.30** Introduction and key messages of day 1  
M. Scarpa, C. Lampe
- 08.30 – 09.00** Alpha-mannosidosis management: perspectives from patients and caregivers  
M. Lopez
- 09.00 – 09.50** Emergency and atypical situations in LSDs  
F. Lagler, M. Lindschau
- 09.50 – 10.10** Coffee Break
- 10.10 – 10.30** Shared decision making and integrated care in LSDs  
N. Muschol
- 10.30 – 11.10** Additional perspectives in Alpha-mannosidosis patients management  
**Orthopaedic:** A. Borgo  
**Ophthalmologist:** J. Ashworth  
**Medical and Neuropsychiatrist:** R. Hackett
- 11.10 – 11.30** DISCUSSION – Q&A  
**Moderator:** M. Scarpa  
**Discussants:** All Session Faculty

## SESSION IV AN EYE ON THE FUTURE

**Moderators:** M. Magner, D. Zafeiriou

- 11.30 – 11.55** In-utero ERT: a novel opportunity?  
P. Harmatz
- 11.55 – 12.20** Neurometabolism defects in rare metabolic disorders  
A. Garcia-Gazorla
- 12.20 – 12.45** Gene therapy in LSDs: how far is the revolution?  
**Moderators:** M. Magner, K. Stepien  
**Speaker:** Simon A. Jones
- 12.45 – 13.00** DISCUSSION – Q&A  
**Moderator:** M. Scarpa  
**Discussants:** All Session Faculty
- 13.00 – 13.30** Lunch

## SESSION V NETWORK IN ACTION

**Moderators:** M. Scarpa

- 13.30 – 14.50** Reinforcing the network to generate new data: current challenges and future perspectives  
All Faculty
- 14.50 – 15.15** Conclusions and Take-Home messages  
M. Scarpa

# FACULTY

Jane Ashworth, Manchester (United Kingdom)

Lisa Berry, Cincinnati (United States)

Andrea Borgo, Padova (Italy)

Alexander Broomfield, Manchester (United Kingdom)

Laura Buch, Greenwood (United States)

Cristobal Colon, Vigo (Spain)

Angels Garcia-Cazorla, Barcelona (Spain)

Nathalie Guffon, Lyon (France)

Julia B. Hennermann, Mainz (Germany)

Simon A. Jones, Manchester (United Kingdom)

Florian Lagler, Salzburg (Austria)

Christina Lampe, Giessen (Germany)

Mona Lindschau, Hamburg (Germany)

Monica Lopez, Nashville (United States)

Martin Magner, Prague (Czech Republic)

Nicole Muschol, Hamburg (Germany)

Paul Harmatz, San Francisco (United States)

Richard Hackett, Manchester (United Kingdom)

Valentina Rovelli, Milano (Italy)

Stewart Rust, Manchester (United Kingdom)

Maurizio Scarpa, Udine (Italy)

Karolina Stepien, Manchester (United Kingdom)

Fatma Tuba Eminoğlu, Ankara (Türkiye)

Nato V. Vashakmadze, Mosca (Russia)

Dimitrios Zafeiriou, Tessaloniki (Greece)

# SCIENTIFIC COORDINATOR

Maurizio Scarpa, Udine (Italy)

## **UEMS-EACCME CME:**

For further information, please visit: <https://web.aimgroupinternational.com/2024/trails/>

**An application will be made to the EACCME® for CME accreditation of the event**

**“TRAILS 3<sup>rd</sup> Edition – Alpha Mannosidosis and Mucopolysaccharidosis: optimizing patient journeys”  
Prague, Czech Republic, November 18<sup>th</sup>-19<sup>th</sup> 2024**

**OFFICIAL LANGUAGE:** The official language is English.

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**Organizing Secretariat & CME Provider**



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