

TRAILS 2025

Dr. Shuan-Pei Lin
Dr. Hsiang-Yu Lin

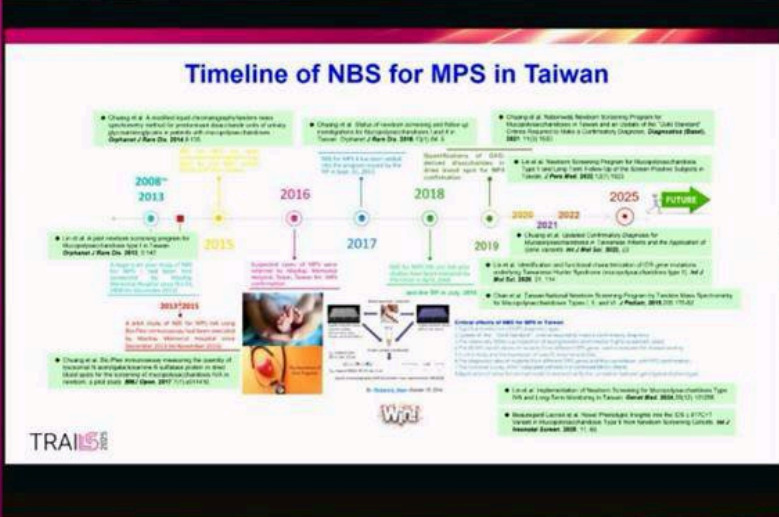
MacKay Memorial Hospital, Taipei, Taiwan

Nationwide Newborn Screening in Taiwan





Timeline of NBS for MPS in Taiwan



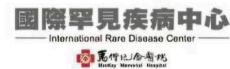






Nationwide Newborn Screening for Mucopolysaccharidoses in Taiwan -10 Years' Experience

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SCIENTIFIC PROGRAM

OCTOBER 28TH • 29TH

TRAILS 2025

4TH EDITION

ALPHA MANNOSIDOSIS AND MUCOPOLYSACCHARIDOSIS:
OPTIMIZING PATIENT JOURNEYS

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*10.10 – 11.00 On-line available tools to reduce diagnostic delays
*M. Fruchart, 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 – *Faculty speaker*

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
- 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*

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. Práhofer, F. Lagler, A. Kerpauskienė, V. Plaiasu*14.45 – 15.15 Closing and remarks – *F. Lagler**Laura Buch (USA)**Mathilde Fruchart (France)**Serena Gasperini (Italy)**Nathalie Guffon (France)**Julia B. Hennermann (Germany)**Alvaro Hermida (Spain)**Agnė Kerpauskienė (Lithuania)**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)**Maximilian Zeyda (Austria)***SCIENTIFIC COORDINATOR***Florian Lagler (Austria)***PATIENT FOR ROUND TABLE***Maria Práhofer (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