

Emma CPM Symposium

Personalized Medicine **Beyond Borders**



10.00 Welcome and opening Prof. dr. Erik Sistermans & Prof. dr. Willem de Vries

10.10 Highlights Emma CPM 2024-2025 Prof. dr. Mieke van Haelst & Prof. dr. Clara van Karnebeek

10.30 EMMA CPM COLLABORATIONS

Moderator: Prof. dr. Vivi Heine

Translating therapies through expert panels and real-world cases

A CRISPR clinic case study in rare retinal disorders

Targeting metabolic dysfunction in neurodevelopmental disorders

Bridging molecular insight and clinical care in **Gyrate Atrophy**

Emma CPM Assistant Professors

NATIONAL COLLABORATIONS 11.45

Moderator: Prof. dr. Clara van Karnebeek

UMD on a mission: Towards treatment for more than 50% of metabolic patients by 2035 *Dr. Hidde Huidekoper (Erasmus MC)*

Personalized therapies for DNA translation disorders: Family Participation is key! *Féline and her mother Marieke*



12.30 Lunch & posters

13.30 **INTERNATIONAL COLLABORATIONS (1)**

Moderator: Prof. dr. Hilgo Bruining

Mitochondrial dysfunction in neurodevelopmental disorders: the Phelan-McDermid paradigm Prof. dr. Antonio Persico (University of Modena)

14.15 Intermezzo How well do you know Emma CPM?

14.30 INTERNATIONAL COLLABORATIONS (2) Moderator: Prof. dr. Riekelt Houtkooper

Molecular therapy for inherited metabolic diseases

Prof. dr. Nicola Brunetti Pierri (University of Napels) Interactive discussion on driving therapeutic 15.15

innovation through collaboration Moderators: Prof. dr. Vivi Heine & Prof. dr. Hilgo Bruining

15.50 Closing

16.00 Coffee break & end of Emma CPM symposium

16.30 **SPINOZA LECTURE**

by Prof. dr. Anne Goriely (University of Oxford)



June 3rd 2025 | 10 - 16 hours | followed by the Spinoza lecture

Amsterdam UMC | AMC | lecture hall 4 Registration before May 27th via QR code

NVK & VKGN accreditation

