



Our 6th Winter School is jointly organised with EPNS this year and will focus on **Challenges of Treating Rare Neurological Diseases.** It will be held **online** from March 12 to 14, 2025.

This training course covers opportunities and challenges of treating rare neurological diseases. It comprises disease-modifying therapies, treatment of movement disorders and non-neurological disease aspects. There will be on day 3 an extra session on case discussion for which participants are invited to submit cases.

ERN-RND has developed a European training requirement for rare neurological diseases (ETR-RND) which has been adopted by the European Union of Medical Specialists. The respective learning objectives according to this ETR-RND are:

- Demonstrate up-to-date knowledge in pharmacological treatment of rare neurological diseases
- Demonstrate up-to-date knowledge about multidisciplinary care and neurorehabilitation and non-pharmacological treatment
- Demonstrate working knowledge of non-invasive stimulation techniques
- Demonstrate knowledge on advanced therapies in the respective disease
- Demonstrate up-to-date knowledge in the surgical treatment if appropriate



# PROGRAMME

#### **Disease Modifying Therapies** 12 March 2025

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CHAIR

TIME (CET)	ΤΟΡΙϹ	SPEAKER
9:00-9:45	Treatment of <b>Metachromatic</b> Leukodystrophies	Francesca Fumagalli IRCCS San Raffaele Scientific Institute, Milan, Italy
9:45-10:30	Leriglitazone Trial in Adrenoleukodystrophy	Fanny Mochel, Caroline Sevin La Pitié-Salpêtrière Hospital and Kremlin-Bicêtre University Hospital, Paris, France
10:30-11:15	Treatment of <b>Aromatic I-amino</b> Acid Decarboxylase (AADC) Deficiency	Thomas Opladen University Children's Hospital Heidelberg, Germany
11:15-11:30	Coffee Break	
11:30-12:15	Disease-modifying Treatment Approaches/Trials in Huntington's Disease (HD)	Bernhard Landwehrmeyer University hospital Ulm, Germany
12:15-13:00	Treatment of <b>Nano-rare</b> Neurological Diseases	Marlen Lauffer Leiden University Medical Center, The Netherlands
13:00-13:30	Q&A, Discussion	

# 13 March 2025 Movement Disorders --

CHAIR

TIME (CET)	ΤΟΡΙϹ	SPEAKER
9:00-9:30	Introduction – Systematics of Movement Disorders Symptoms	Bart van de Warrenburg Radboudumc, Nijmegen, The Netherlands
9:30-10:15	Treatment of <b>Spasticity</b> in children with rare neurological disorders – who, why, when and what?	Annemieke Buizer Amsterdam UMC, The Netherlands
10:15-11:00	Treatment of <b>Dystonia</b>	Norbert Brüggemann University Hospital Schleswig- Holstein, Germany
11:00-11:15	Coffee break	
11:15-12:00	tba	Ronald Buijsen
12:00-12:45	Treatment of Ataxia - Rehabilitation Based	Gabriella Paparella IRCCS Eugenio Medea, Institute for Rehabilitation Medicine, Italy
12:45-13:15	Q&A, Discussion	



14 March 2025Non-neurological Disease Aspects and CasesCHAIR--

TIME (CET)	ΤΟΡΙϹ	SPEAKER
9:00-9:45	Introduction – Systematics of Non-neurological Aspects	Michèl Willemsen Radboudumc, Nijmegen, The Netherlands
9:45-10:30	Speech Treatment in <b>Ataxias</b>	Adam Vogel University of Melbourne, Melbourne, Australia
10:30-11:15	Cognitive and Psychiatric Treatment in Huntington's Disease	Mayke Oosterloo Maastricht University Medical Center, The Netherlands
11:15-11:30	Q&A, Discussion	
11:30-11:45	Coffee Break	
11:45-13:15	<b>Case Presentations</b>	Expert Panel
13:15-13:30	Closing Remarks	

For further information please contact Sophie Ripp (Sophie.ripp@med.uni-tuebingen.de)

## To register click <u>here.</u>

### **PROGRAMME COMMITTEE**

- Michèl Willemsen, Nijmegen (EPNS, ERN-RND)
- Jeroen Vermeulen, Maastrich (EPNS, ERN-RND)
- Barbara Plecko, Graz (EPNS)
- Kathleen Gorman, Dublin (EPNS, ERN-RND)
- Saskia Wortmann, Salzburg (EPNS)
- Caroline Sevin, Paris (EPNS, ERN-RND)
- Dana Craiu, Bucarest (EPNS)
- Holm Graessner, Tübingen (ERN-RND)