

SCIENTIFIC COORDINATOR:

Professor Dr. med. BENEDIKT SCHOSER, FEAN

Friedrich Baur Institute
Neurology Clinic and Outpatient Department
LMU University Hospital
Ludwig Maximilian University of Munich

SPEAKERS:

ATALAIA ANTONIO

APHP Pitié Salpêtrière, Inserm U974, Myology Research Center,
Sorbonne University, Paris

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Head of the Translational Myotonic Dystrophy Research Group
at the Institut de Myologie - Paris

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Global Registries Manager - Treat NMD Neuromuscular Network

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Amalia Children's Hospital Radboud University Medical Center
& Donders Institute for Brain Cognition and Behaviour - Nijmegen

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Neurologist University Medical Centre - Maastricht

GEILLE ALAIN

Euro-DyMA: Federation of European Myotonic Dystrophy Associations
Institut de Myologie - Hôpital Pitié-Salpêtrière - Paris

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Sorbonne University, Inserm, Myology Research Center, Paris

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Chief Scientific Officer - Myotonic Dystrophy Foundation

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Neurology Clinic and Clinical Centre for NeuroMuscular Disorders,
S. Chiara Hospital, University of Pisa - Pisa

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University College London Hospitals Nhs Foundation Trust, London

VAN GENT JORG

MD Europe - Nederland

VOHANKA STANISLAV

Assistant professor, Department of Neurology - Masaryk University, Brno

COURSE VENUE

mbw Medienberatung der Wirtschaft GmbH - Munich
Max-Joseph-Straße 5, 80333 Munich, Germany

Industry Representative Attendance Policy

The event is designed primarily for healthcare providers (HCPs) involved in the diagnosis and management of myotonic dystrophy type 1 (DM1), as well as researchers and other professionals seeking to expand their knowledge in this field.

While this event is not specifically tailored to industry representatives, we welcome individuals from companies who wish to attend in a non-promotional capacity to learn and engage with the educational content.

Unrestricted educational grant

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14-15 April 2025
Munich, Germany

mbw Medienberatung der
Wirtschaft Munich, Germany

Euro DM CRN

**Kick-off Meeting
of the European
Clinical Research Network
for Myotonic Dystrophies**

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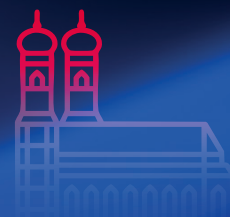
OVERARCHING OBJECTIVES

Facilitate clinical trial implementation across Europe by creating a unified and collaborative framework.

Contribute to the US-based DM-CRN DM1 natural history database to deepen understanding of disease progression and support research worldwide.

Enhance collaboration among European DM experts, fostering knowledge and resource sharing.

Standardize research and clinical protocols to improve outcomes for DM patients across Europe.



Programme

14th April 2025

- 12:00 - 12:45 Registration & Lunch
- 12:45 - 13:15 Welcome and Introduction: On behalf of the kick-off organizing working group
Benedikt Schoser
- 13:15 - 13:45 European landscape on DM
A patient organization view
Alain Gelle
- 13:45 - 14:15 The MDF perspective
Andy Rohrwasser
- 14:15 - 14:30 Q&A
- 14:30 - 14:45 Summary ENMC Workshop on harmonisation of DM registries
Leandre la Fontaine
- 14:45 - 15:15 Update on the END-DM-CRN network USA
Valeria Sansone
- 15:15 - 15:30 DM Registries with focus on the DM Scope
Guillaume Bassez
- 15:30 - 15:45 UK DM registry
Christopher Turner
- 15:45 - 16:00 Italian DM working group
Gabrielle Siciliano
- 16:00 - 16:15 Euro-ERN registry: an overview
Antonio Ataloia
- 16:15 - 16:30 The TREAT-NMD DM registry
Neil Bennett
- 16:30 - 16:45 Coffee Break
- 16:45 - 17:15 Global DM-CRN
Nickolas Johnson
- 17:15 - 17:45 New natural history study in DM1 for myotonia assessments
Jean-Yves Hogrel
- 17:45 - 18:00 Training the new generation of DM experts: the ENTRY-DM doctoral network
Mario Gomes-Pereira
- 18:00 - 18:15 Q&A

15th April 2025

- 09:00 - 09:15 Introduction day 2:
On behalf of the kick-off organizing working group
Benedikt Schoser
- 09:15 - 09:45 PROMs and PROs the Maastricht experience
Karin Faber
- 09:45 - 10:15 Congenital DM - an overview on current trials
Hilde Braakman
- 10:15 - 10:45 Project ANIQ
Jorg van Gent
- 10:45 - 11:00 Q&A
- 11:00 - 11:15 Coffee Break
- 11:15 - 11:30 DM2 Registry an overview
Stanislav Vohanka
- 11:30 - 11:40 Targeting the genetic basis of muscle diseases with the FORCE platform
Douglas Kerr - Dyne Therapeutics
- 11:40 - 12:05 Where Are We Now? Unmet Needs in Rare Neuromuscular Diseases and the Clinical Impact of the FORCE Platform in DM1
Douglas Kerr - Dyne Therapeutics
- 12:05 - 12:15 Q&A
- 12:15 - 12:45 ROUND TABLE: Needs and requests of the industry to the Euro-DM-CRN
Chairperson: *Benedikt Schoser*

Ash Dugar - Dyne Therapeutics
Angela Guerra - Avidity Biosciences
Asif Paker - VRTX
Joseph Thakuria - Arrow Head Pharma
Jenny Shoskes - Pepgen
Judith Walker - Arthex Biotech
Alla Zozulya Weidenfeller - Lupin
Didier Rouy - Sanofi
- 12:45 - 13:45 Alignments of New Clinical Trial Centres to Global DM-CRN session
Open Discussion
- 13:45 - 14:00 Next steps and next meeting
Benedikt Schoser
- 14:00 - 15:00 Lunch and Farewell