## **PROGRAM**

#### **KIEROWNIK NAUKOWY**

prof. dr hab. n. med. Anna Kostera-Pruszczyk

#### Friday, June 2, 2023

11:00-11:10	Opening Local Chair: Anna Kostera-Pruszczyk, Poland EAN NMD Panel chairs: <i>Kristl Claeys, Belgium, Gabriele Siciliano, Italy</i>
11:10-13:10	Session I Introduction to neuromuscular disorders Chairman: Anna Kostera-Pruszczyk, Poland, Maria Judit Molnar
11:00-11:25	Clinical patterns in NMDs Benedikt Schoser, Friedrich Baur Institute at the Neurological Clinic and Polyclinic Hospital of the University of Munich Ludwig Maximilian University of Munich, Germany
11:25-11:50	Ultrasound in diagnosis of NMD Stephan Goedee, University Medical Center Utrecht, ERN EURO-NMD, Netherlads

11:50-12:15	Contemporary role of molecular genetics in NMD  Maria Judit Molnar, Clinical Genetics Director of the Institute of Genomic Medicine and Rare  Disorders Semmelweis University, Budapest, Hungary
12:15-12:40	Role of muscle biopsy in era of molecular data in NMD Teresinha Evangelista, Institute of Myology, Head of Morphological Unit, The Pitié-Salpêtrière Hospi ERN EURO-NMD, Paris, France
12:40-13:00	Discussion
13:10-14:10	Lunch break
14:10-16:10	Session II Specific neuromuscular disorders Chairman: Teresinha Evangelista, Maria Jędrzejowska
14:10-14:35	Duchenne muscular dystrophy diagnosis and current treatment Anna Potulska-Chromik, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland

14:35-15:00	DMD: future treatment strategies Valeria Sansone, Nemo Clinical Center, ERN EURO-NMD, Milan, Italy
15:00-15:25	Myofibrillar myopathies Biruta Kierdaszuk, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland
15:25-15:50	Congenital myopathies Maria Jędrzejowska, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Polal
15:50-16:10	Discussion
16:10-16:40	Coffe break
16:40-18:40	Session III Breakthrough therapies in NMD Chairman: Benedikt Schoser, Monika Gos
16:40-17:05	Newborn screening in NMD Monika Gos, Institute of Mother and Child, Warsaw, Poland

17:05-17:30	SMA: real world experience Anna Kostera-Pruszczyk, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland
17:30-17:55	Late onset Pompe disease dr n. med. Grzegorz Witkowski, Institute of Psychiatry and Neurology in Warsaw
17:55-18:20	Gene therapy in selected NMDs Benedikt Schoser, Friedrich Baur Institute at the Neurological Clinic and Polyclinic Hospital of the University of Munich Ludwig Maximilian University of Munich, Germany
18:20-18:40	Discussion

#### Saturday, June 3, 2023

09:50-10:00	Opening of the 2nd day of the conference
	Anna Kostera-Pruszczyk, Poland

# 10:00-11:35 Session IV Special consideration in neuromuscular disease Chairman: Gabriele Siciliano, Anna Łusakowska

10:00-10:25	Why and How to Digitalize Bulbar Motor Dysfunction Patterns in NMD Gabriele Siciliano, Department of Clinical and Experimental Medicine, Neurological Clinic, University of Pisa Medical School, Italy
10:25-10:50	The Role of Digital PROMs in the Data Collection for Real World Evidence Evaluation Peter Balicza, Department of Neurology, Semmelweis University, Budapest, Hungary
10:50-11:15	Role of registries in clinical trial readiness Anna Łusakowska, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland
11:15-11:35	Discussion
11:35-12:05	Coffe break
12:05-14:20	Session V Special consideration in neuromuscular disease Chairman: Nils Erik Gilhus, Kristl Claeys

12:05-12:30	Myasthenia gravis diagnosis Nils Erik Gilhus, University of Bergen and senior consultant at Department of Neurology, Haukeland University Hospital, Norway
12:30-12:55	Myasthenia gravis treatment challenges Anna Kostera-Pruszczyk, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland
12:55-13:20	Inflammatory myopathies Kristl Claeys, Kliniekhoofd Neurologie, Neuromusculaire Ziekten Laboratory for Muscle Diseases and Neuropathies, Leuven, Belgium
13:20-13:45	CIDP and MMN Marta Lipowska, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland
13:45-14:05	Discussion
14:05-14:20	NMD Quiz Anna Frączek, Department of Neurology, Medical University of Warsaw, ERN EURO-NMD, Poland

### 14:20-14:30 Closing EAN Faculty and Local Chair

14:30 Lunch

The organizer reserves the right to make changes to the program
The program includes promotional materials