



# ICNMD 2026

19<sup>TH</sup> INTERNATIONAL CONGRESS  
ON NEUROMUSCULAR DISEASES

7 - 11 July 2026 **Florence, Italy**

# Program Book

ICNMD.ORG | #ICNMD2026

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# Welcome Letter

I want to extend a warm welcome to the 19th International Congress on Neuromuscular Diseases (ICNMD) and it is with great pleasure and honour to welcome you to Italy in the magnificent city of Florence for this prestigious event.

The ICNMD is recognized worldwide as a leading congress in Neuromuscular Disorders and this edition will continue this legacy with top contributions in education, diagnostic advances and scientific novelties. We are expecting over 2,000 delegates including neurologists, pathologists, psychiatrists, molecular biologists, geneticists and bioinformaticians as well as industry partners who will gather together for ICNMD and make Florence the capital of Neuromuscular pathology updates.

This will be an extraordinary opportunity for all the stakeholders in the field, to meet, exchange ideas, foster fruitful collaborations and catalyze innovation at all levels. We have organized a rich and stimulating programme that reflects the full spectrum of innovation and emerging perspectives in the field of Neuromuscular Disorders.

There will be keynote lectures, plenary and scientific sessions, teaching courses, oral and poster presentations, and specialty curated joint sessions as well as industry supported symposia, all aimed at providing an update on the latest clinical and therapeutic trends. Be sure to participate in the social events and networking opportunities throughout the congress, and hopefully before, in the evenings and after the congress, you will find time to enjoy our beautiful Florence.

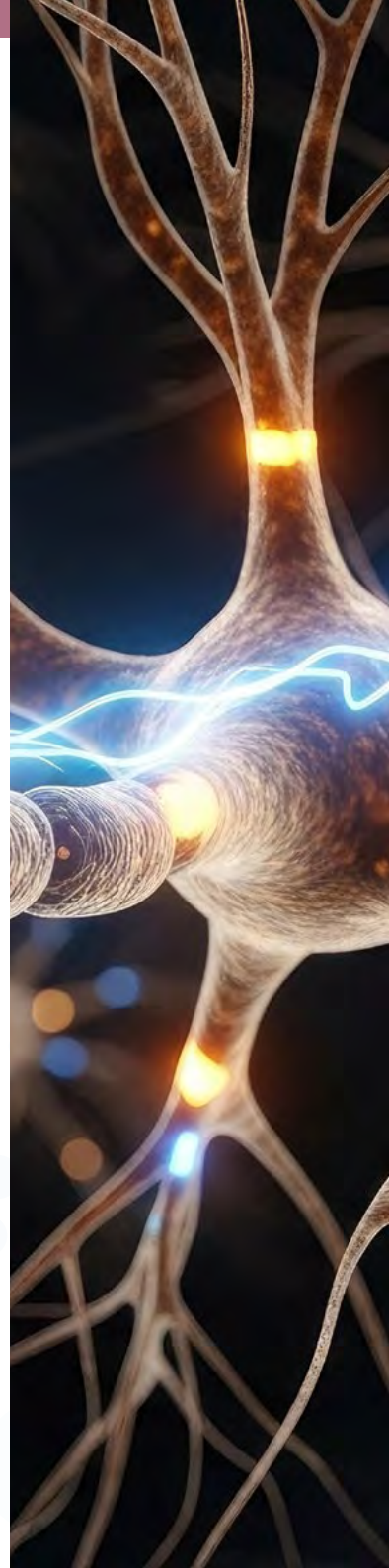
Once again, thank you for being here and Welcome to Florence.

Sincerely,



A handwritten signature in black ink that reads "Antonio Toscano".

**Antonio Toscano,**  
MD, PhD  
**ICNMD 2026**  
**Congress President**  
Professor of Neurology,  
University of Messina



# About ICNMD



The ICNMD International Congress on Neuromuscular Diseases is organized on behalf of the Specialty Group on Neuromuscular Diseases (NMD) of the World Federation of Neurology (WFN).

The aim of the ICNMD Congresses is to offer attendees an updated view on neuromuscular disorders and that networking opportunities increase their international experience and collaborations. The scientific and program committee are invited from all continents around the world to enable this wide spectrum.

Sincerely,



**James B. Dyck, MD**  
**ICNMD 2026 Chair**  
Mayo Clinic Adult Neurology  
and Neurosurgery, Rochester,  
Minnesota

# ICNMD 2026 Committees

## Steering Committee

### **Antonio Toscano**

Congress President  
University of Messina, Italy

### **P. James B. Dyck**

ICNMD Chair  
Mayo Clinic, USA

### **Steven Lewis**

WFN President, USA

### **Wolfgang Grisold**

WFN Past-President, Austria

Chiara Briani

University of Padova, Italy

Claudia Sommer

University of Würzburg, Germany

Fiore Manganelli

University of Naples Federico II, Italy

Gabriele Siciliano

University of Pisa, Italy

Marianne De Visse

Amsterdam UMC, Netherlands

## Program Committee

### **Antonio Toscano**

Congress President & Program Committee  
Co-Chair

### **P. James B. Dyck**

ICNMD Chair & Program Committee Co-Chair

### **Wolfgang Grisold**

WFN Past-President

Benedikt Schoser

Co-Chair, Muscle

Vincenzo Nigro

Co-Chair, Muscle

Giuseppe Lauria

Co-Chair, Peripheral Nerve

Michelle L. Mauer mann

Co-Chair, Peripheral Nerve

Adriano Chiò

Co-Chair, Motor Neuron

Angela Genge

Co-Chair, Motor Neuron

Amelia Evoli

Co-Chair, Neuromuscular Junction

Stephen Reddel

Co-Chair, Neuromuscular Junction



# ICNMD 2026 Committees

## Program Sub-Committees

### Muscle

**Benedikt Schoser – Co-Chair**  
Ludwig Maximilian University,  
Germany

John Vissing  
Copenhagen University  
Hospital, Denmark

Marco Savarese  
Folkhalsan Research Center,  
Finland

**Vincenzo Nigro – Co-Chair**  
University of Campania “Luigi  
Vanvitelli”, Italy

Jorge A. Bevilacqua  
University of Chile and Clinica  
Dávila, Chile

Massimiliano Filosto  
University of Brescia, Italy

Ichizo Nishino  
National Center of Neurology  
and Psychiatry, Japan

Mazen M. Dimachkie  
University of Kansas Medical  
Center, USA

### Peripheral Nerve

**Giuseppe Lauria – Co-Chair**  
IRCCS Istituto Neurologico  
Carlo Besta, Italy

Eduardo Nobile-Orazio  
University of Milan, Italy

Mary Reilly  
University College London, UK

**Michelle L. Mauermann –  
Co-Chair**  
Mayo Clinic, USA

Kleopas A. Kleopa  
Institute of Neurology &  
Genetics, Cyprus

P. James B. Dyck  
Mayo Clinic, USA

David Cornblath  
Johns Hopkins University, USA

Marcelo Rugiero  
Hospital Italiano de Buenos  
Aires, Argentina

Satoshi Kubawara  
Chiba University, Japan

### Neuromuscular Junction

**Amelia Evoli – Co-Chair**  
Università Cattolica del Sacro  
Cuore, Italy

Carlo Antozzi  
IRCCS Istituto Neurologico  
Carlo Besta, Italy

Jan J.G.M Verschuuren  
Leiden University Medical  
Center, Netherlands

**Stephen Reddel – Co-Chair**  
University of Sydney / Concord  
Hospital, Australia

Gil Wolfe  
University at Buffalo, USA

Nils Erik Gilhus  
University of Bergen, Norway

Andreas Meisel  
Charité – Universitätsmedizin  
Berlin, Germany

Hanns Lochmuller  
University of Ottawa / CHEO  
Research Institute, Canada

Pushpa Narayanaswami  
Beth Israel Deaconess  
Medical Center, USA

Henry Kaminski  
George Washington University,  
USA

### Motor Neuron

**Adriano Chiò – Co-Chair**  
University of Turin, Italy

Jonathan Glass  
Emory University, USA

Merit Cudkowicz,  
Massachusetts General  
Hospital / Harvard Medical  
School, USA

**Angela Genge – Co-Chair**  
McGill University Health  
Centre Research Institute,  
Canada

Julian Großkreutz, University  
of Lübeck, Germany

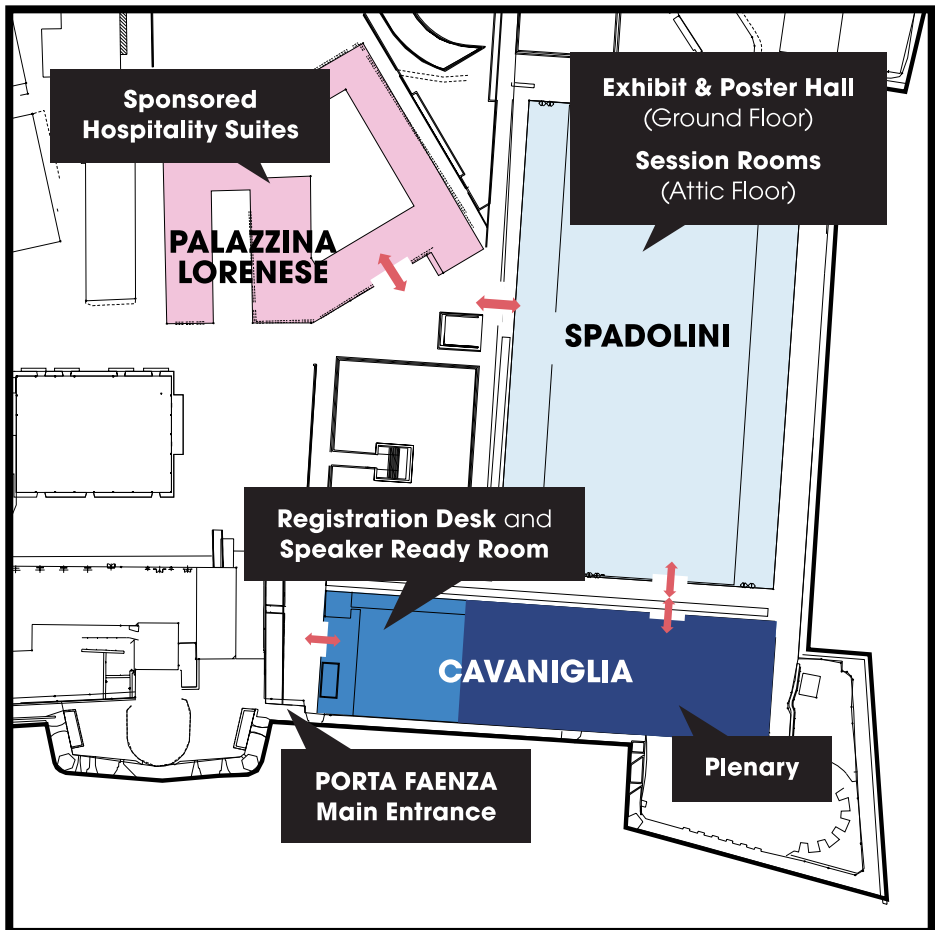
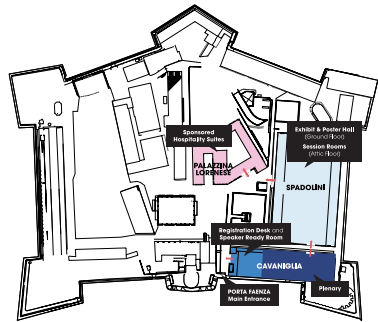
Leonard van den Berg, UMC  
Utrecht, Netherlands

Nicola Ticozzi, University  
of Milan, IRCCS Istituto  
Auxologico Italiano, Italy

Jessica Mandrioli  
University of Modena and  
Reggio Emilia, Italy



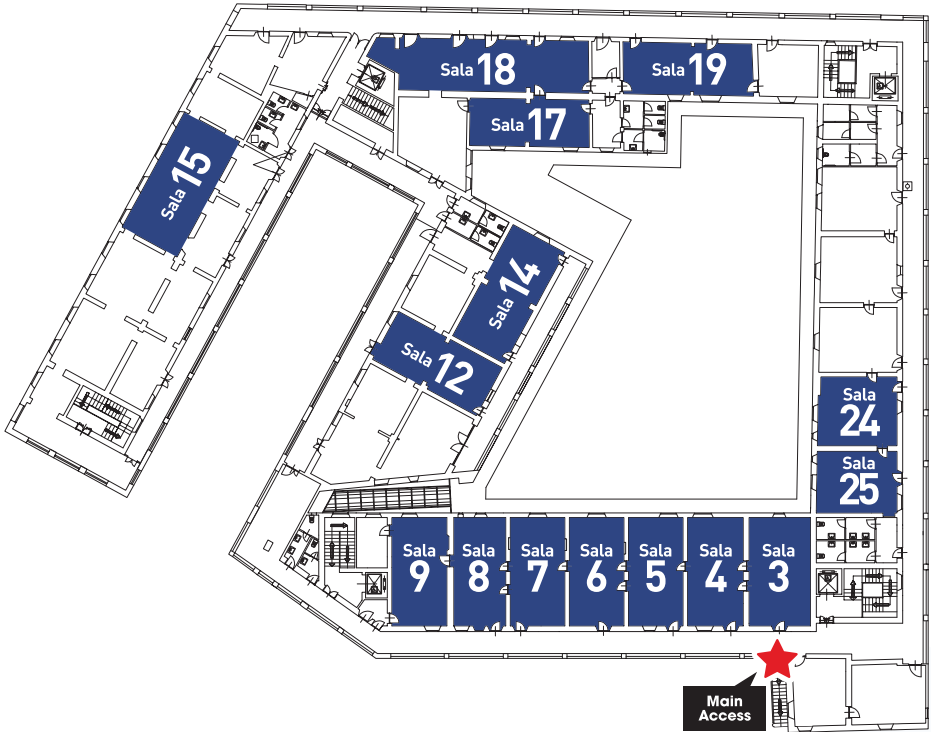
**FIRENZE  
FIERA**



# Venue Floorplan

Palazzina Lorenese 1<sup>st</sup> Floor

## Sponsored Hospitality Suites



# Schedule at a Glance

- Teaching Courses
- Social Functions
- Joint Sessions
- Oral Abstracts
- ◆ Ticketed Sessions
- Muscle Disorders
- Motor Neuron Diseases
- Peripheral Neuropathies
- Neuromuscular Junction Disorders

## Tuesday 7 July

TIME	COURSE NAME	LOCATION
08:00 - 10:00	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC01:</b> Teaching Course - Pain in Neuromuscular Disorders	Hall 1
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC02:</b> Teaching Course - Practical Approach to Myology Phenotypes	Hall 2
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC03:</b> Teaching Course - PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 1	Hall 3
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC04:</b> Teaching Course - The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 1	Hall 4
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC05:</b> Teaching Course - Hands On: Clinical Neurophysiology	Hall 5
10:00 - 10:30	<span style="color: white;">●</span> Morning Break	Spadolini - Attic
08:00 - 10:00	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC06:</b> Teaching Course: Emergencies in Myopathies	Hall 1
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC07:</b> Teaching Course: Genetics in Neuromuscular Disorders	Hall 2
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC08:</b> Teaching Course: PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 2	Hall 3
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC09:</b> Teaching Course: The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 2	Hall 4
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC10:</b> Teaching Course: Myasthenia Gravis	Hall 5
12:30 - 13:30	<span style="color: white;">●</span> Lunch Break	Spadolini - Attic
13:30 - 15:30	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC11:</b> Teaching Course: How to examine a patient with neuromuscular disorders: A Practical Guide	Hall 1
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC12:</b> Teaching Course: What a Biopsy can tell you in the Genomic Era	Hall 2
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC13:</b> Teaching Course: Amyotrophic Lateral Sclerosis in 2026: Clinical Practice, Biomarkers and Precision Medicine	Hall 3
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC14:</b> Teaching Course: Inherited Myopathies - Less Common Disorders	Hall 4
	<span style="color: yellow;">●</span> <span style="color: grey;">◆</span> <b>TC15:</b> Teaching Course - Hands On: Ultrasound	Hall 5
15:30 - 16:00	<span style="color: white;">●</span> Afternoon Break	Spadolini - Attic
17:15 - 18:00	<span style="color: red;">●</span> Opening Ceremony	Cavaniglia - Plenary Room
18:00 - 19:30	<span style="color: red;">●</span> Welcome Reception	Exhibit & Poster Hall

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## Wednesday 8 July

TIME	SESSION NAME	LOCATION
08:30 - 10:30	<span style="color: darkblue;">●</span> <b>PL01:</b> Plenary: Myology 2030	Cavaniglia - Plenary Room
10:30 - 11:15	<span style="color: white;">●</span> Morning Break & Poster Tour 1	Exhibit & Poster Hall
11:15 - 12:45	<span style="color: darkblue;">●</span> <b>SS01:</b> Up-to-date on Muscle Inflammation	Hall 2
	<span style="color: darkblue;">●</span> <b>SS02:</b> Up-to-date on FSHD and OPMD	Hall 5
	<span style="color: cyan;">●</span> <b>SS03:</b> Primary Lateral Sclerosis	Hall 3
	<span style="color: blue;">●</span> <b>SS04:</b> FcRn and Complement Inhibitors in MG Severe Deteriorations	Hall 4
	<span style="color: pink;">●</span> <b>SS05:</b> ICNMD & World Muscle Society Joint Session	Hall 1
12:45 - 14:30	<span style="color: white;">●</span> Lunch & Poster Viewing	Exhibit & Poster Hall
13:15 - 14:15	<span style="color: black;">●</span> Industry-Supported Symposia	Spadolini - Attic
14:30 - 16:00	<span style="color: darkblue;">●</span> <b>SS06:</b> Up-to-date on Metabolic Muscle Disorders	Hall 2
	<span style="color: cyan;">●</span> <b>SS07:</b> Biomarkers of Early Diagnosis in ALS	Hall 3
	<span style="color: darkblue;">●</span> <b>SS08:</b> Tools for the Next Generation Myologists	Hall 5
	<span style="color: red;">●</span> <b>SS09:</b> Advances in TTR Amyloid	Hall 4
	<span style="color: pink;">●</span> <b>SS17:</b> ICNMD, AIM & ASNP Joint Session	Hall 1
16:00 - 16:45	<span style="color: white;">●</span> Afternoon Break & Showcase Theatre	Exhibit & Poster Hall
16:45 - 18:15	<span style="color: darkblue;">●</span> <b>SS11:</b> Up-to-date on Limb Girdle Myopathies	Hall 1
	<span style="color: blue;">●</span> <b>SS12:</b> Early-phase Treatments in MG	Hall 2
	<span style="color: lightblue;">●</span> <b>OS01:</b> Oral Session 1	Hall 3
	<span style="color: lightblue;">●</span> <b>OS02:</b> Oral Session 2	Hall 4
	<span style="color: lightblue;">●</span> <b>OS03:</b> Oral Session 3	Hall 5

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## Thursday 9 July

TIME	SESSION NAME	LOCATION
07:00 - 08:00	● Industry-Supported Symposia	Spadolini - Attic
08:30 - 10:30	● <b>PL02:</b> Plenary: Cutting Edge Updates in Neuropathy	Cavaniglia - Plenary Room
10:30 - 11:15	● Morning Break & Poster Tour 2	Exhibit & Poster Hall
11:15 - 12:45	● <b>SS13:</b> Cancer Related Peripheral Neuropathies	Hall 2
	● <b>SS14:</b> Challenges in MG Treatment	Hall 3
	● <b>SS15:</b> Biomarkers of Disease Progression and Prognosis	Hall 5
	● <b>SS16:</b> Up-to-date on Immune-mediated Neuropathies	Hall 4
	● <b>SS10:</b> ICNMD & Peripheral Nerve Society Joint Session	Hall 1
12:45 - 14:30	● Lunch & Poster Viewing	Exhibit & Poster Hall
13:15 - 14:15	● Industry-Supported Symposia	Spadolini - Attic
14:30 - 16:00	● <b>SS18:</b> Up-to-date on Myotonic Dystrophies	Hall 2
	● <b>SS19:</b> Technologies Updates in Neuropathies (formally Updates in CMT)	Hall 3
	● <b>SS20:</b> Tailoring MG Care for Women	Hall 5
	● <b>SS21:</b> Hereditary Neuropathy Overlap Syndromes	Hall 4
	● <b>SS22:</b> ICNMD & German Nerve Club Joint Session - Nerve Surgery, Diagnostics and Rehabilitation	Hall 1
16:00 - 16:45	● Afternoon Break & Showcase Theatre	Exhibit & Poster Hall
16:45 - 18:15	● <b>SS23:</b> Update on Diagnostic Tools in Neuropathy	Hall 1
	● <b>SS24:</b> Patient Monitoring in NMJ Diseases	Hall 5
	● <b>OS04:</b> Oral Session 4	Hall 2
	● <b>OS05:</b> Oral Session 5	Hall 3
	● <b>OS06:</b> Oral Session 6	Hall 4

# Schedule at a Glance

- Teaching Courses
- Social Functions
- Joint Sessions
- Oral Abstracts
- ◆ Ticketed Sessions
- Muscle Disorders
- Motor Neuron Diseases
- Peripheral Neuropathies
- Neuromuscular Junction Disorders

## Friday 10 July

TIME	SESSION NAME	LOCATION
07:00 - 08:00	● Industry-Supported Symposia	Spadolini - Attic
08:30 - 10:30	● <b>PL03:</b> Plenary: Redefining ALS: A Paradigm Shift in Understanding and Care	Cavaniglia - Plenary Room
10:30 - 11:15	● Morning Break & Poster Tour 3	Exhibit & Poster Hall
11:15 - 12:45	● <b>SS25:</b> New Outcome Measures	Hall 1
	● <b>SS26:</b> ALS Clinical Trials: Novel Approaches Toward an Effective Cure	Hall 2
	● <b>SS27:</b> Diabetic Neuropathies	Hall 3
	● <b>SS28:</b> From Duchenne to Becker Muscular Dystrophies	Hall 4
	● <b>SS29:</b> ICNMD & World Federation of Neurology Joint Session	Hall 5
12:45 - 14:30	● Lunch & Poster Viewing	Exhibit & Poster Hall
13:15 - 14:15	● Industry-Supported Symposia	Spadolini - Attic
14:30 - 16:00	● <b>SS30:</b> Up-to-date Supportive and Palliative Care in Muscle Disorders	Hall 2
	● <b>SS31:</b> A Worldwide Overview of ALS	Hall 3
	● <b>SS32:</b> Updates on ALS Clinical Trials	Hall 5
	● <b>SS33:</b> MG in the Elderly	Hall 4
	● <b>SS34:</b> ICNMD & The Autonomic Disorder Group of the WFN Joint Session	Hall 1
16:00 - 16:45	● Afternoon Break	Exhibit & Poster Hall
16:45 - 18:15	● <b>SS35:</b> Cognition in ALS	Hall 1
	● <b>SS36:</b> Advanced Topics in Peripheral Neuropathy	Hall 2
	● <b>OS07:</b> Oral Session 7	Hall 3
	● <b>OS08:</b> Oral Session 8	Hall 4
	● <b>OS09:</b> Oral Session 9	Hall 5
19:30 - 22:30	●◆ Networking Dinner	Villa Vittoria

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## Saturday 11 July

TIME	SESSION NAME	LOCATION
08:30 - 10:30	<span style="color: blue;">●</span> <b>PL04:</b> Plenary: Translating Advances in Pathophysiology to Clinical Applications in NMJ Diseases	Cavaniglia - Plenary Room
10:30 - 11:00	<span style="color: white;">●</span> Morning Break	Spadolini - Attic
11:00 - 12:30	<span style="color: blue;">●</span> <b>SS37:</b> Defining MG Subtypes	Hall 2
	<span style="color: red;">●</span> <b>SS38:</b> Lepromatous Neuropathy	Hall 3
	<span style="color: darkblue;">●</span> <b>SS39:</b> Up-to-date Fatigue, Ventilation and Sleep Management	Hall 5
	<span style="color: blue;">●</span> <b>SS40:</b> Cancer and Thymoma Related Diseases	Hall 4
	<span style="color: pink;">●</span> <b>SS41:</b> ICNMD & iMyoS Joint Session - Idiopathic Inflammatory Myopathies/Myositis: Optimal Diagnosis and Treatment	Hall 1
12:30 - 14:15	<span style="color: white;">●</span> Lunch Break	Spadolini - Attic
13:00 - 14:00	<span style="color: black;">●</span> Industry-Supported Symposia	Spadolini - Attic
14:15 - 15:45	<span style="color: blue;">●</span> <b>SS42:</b> Translational Research Updates in CMS	Hall 5
	<span style="color: blue;">●</span> <b>SS43:</b> Advances in Understanding Disease Mechanisms in MG	Hall 2
	<span style="color: red;">●</span> <b>SS44:</b> Painful and Small Fiber Neuropathies	Hall 3
	<span style="color: cyan;">●</span> <b>SS45:</b> The Role of Patients and Patient Organizations	Hall 4
	<span style="color: pink;">●</span> <b>SS46:</b> Ageing and Senescence	Hall 1
15:45 - 16:15	<span style="color: red;">●</span> Closing Ceremony	Cavaniglia - Plenary Room

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- 08:00 - 10:00**
- **TC01** ◆ **Teaching Course - Pain in Neuromuscular Disorders**  
◆ **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Tiziana Mongini, Italy
  - **TC01.01** Painful Neuropathies  
Grazia Devigili, Italy
  - **TC01.02** Myalgia in Myopathies  
Tiziana Mongini, Italy
  - **TC01.03** Fibromyalgia  
Sara Lazo, El Salvador
- 08:00 - 10:00**
- **TC02** ◆ **Teaching Course - Practical Approach to Myology Phenotypes**  
◆ **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** Marianne de Visser, Netherlands
  - **TC02.01** Dysphagia  
Zohar Argov, Israel
  - **TC02.02** Asymptomatic Hyper CK'emia  
Antonio Toscano, Italy
  - **TC02.03** Scapula Alata (Winging of Scapula)  
Marianne de Visser, Netherlands
- 08:00 - 10:00**
- **TC03** ◆ **Teaching Course - PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 1**  
◆ **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Eduardo Nobile Orazio, Italy
  - **TC03.01** GBS - Acute Polyneuropathies  
Eduardo Nobile-Orazio, Italy
  - **TC03.03** CIDP and Autoimmune Nodopathies  
Marcus Vinicius Pinto, USA
- 08:00 - 10:00**
- **TC04** ◆ **Teaching Course - The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 1**  
◆ **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Heinz Jungbluth, United Kingdom
  - **TC04.01** The Floppy Infant Revisited – from Bedside to Genome  
Gianpaolo Cicala, Italy
  - **TC04.02** The Neuromuscular Assessment of the Floppy Infant  
Jennie Sheehan, United Kingdom
  - **TC04.03** Congenital Myasthenic Syndromes  
Sithara Ramdas, United Kingdom
- 08:00 - 10:00**
- **TC05** ◆ **Teaching Course - Hands On: Clinical Neurophysiology**  
◆ **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** William Litchy, USA
  - **TC05.01** SFEMG: the use in Evaluation of Neuromuscular Disorders  
Anna Rostedt Punga, Sweden, Sweden
  - **TC05.02** Standard Needle Examination  
Rocco Ligouri, Italy
  - **TC05.03** Nerve Conduction Studies of Proximal Nerves (RNS)  
William Litchy, USA

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- 10:30 - 12:30**
- **TC06** ◆ **Teaching Course: Emergencies in Myopathies**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** John Vissing, Denmark
  - **TC06.01** Rhabdomyolysis  
John Vissing, Denmark
  - **TC06.02** Acute Inflammatory/Infective Myopathy  
Mazen Dimachkie, USA
  - **TC06.03** Respiratory Support  
Paolo Banfi, Italy
- 10:30 - 12:30**
- **TC07** ◆ **Teaching Course: Genetics in Neuromuscular Disorders**  
📍 **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** Ichizo Nishino, Japan
  - **TC07.01** The Practicalities of Motor Neuron Disease Genetics  
Vivian Drory, Israel, Israel
  - **TC07.02** OPDM - a Model for Unusual Expansion disorders  
Ichizo Nishino, Japan
  - **TC07.03** Translational Changes - the FSHD Complicated Genetics  
Sabrina Sacconi, France
- 10:30 - 12:30**
- **TC08** ◆ **Teaching Course - PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 2**  
📍 **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Katrin Hahn, Germany
  - **TC08.01** Amyloid Neuropathy  
Katrin Hahn, Germany
  - **TC08.02** Small Fibre Neuropathy  
Grazia Devigili, Italy
  - **TC08.03** Vasculitic Neuropathies  
Judith Spies, Australia
- 10:30 - 12:30**
- **TC09** ◆ **Teaching Course - The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 2**  
📍 **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Heinz Jungbluth, United Kingdom
  - **TC09.01** Acquired Mimics of CMS – TNG, FARIS and JMG  
Ulrike Schara-Schmidt, Germany
  - **TC09.02** Congenital Myopathies and Related Disorders – Clinical Features  
Heinz Jungbluth, United Kingdom
  - **TC09.03** The Congenital Myopathies – Histopathological Features  
Edoardo Malfatti, France, France
  - **TC09.04** Therapeutic Developments and Clinical Trials in the Congenital Myopathies  
Giovanni Baranello, United Kingdom
- 10:30 - 12:30**
- **TC10** ◆ **Teaching Course: Myasthenia Gravis**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Pushpa Narayanaswami, USA
  - **TC10.01** Antibodies in Myasthenia  
Shahar Shelly, Israel
  - **TC10.02** Immune therapy of Myasthenia Gravis  
Pushpa Narayanaswami, USA
  - **TC10.03** Pitfalls in the Diagnosis  
Amelia Evoli, Italy

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- Peripheral Neuropathies
- Neuromuscular Junction Disorders

- 13:30 - 15:30**
- **TC11** ◆ **Teaching Course: How to Examine a Patient with Neuromuscular Disorders: A Practical Guide**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Davide Pareyson, Italy
  - **TC11.01** How to Examine a Patient With a Myopathy  
Pascal Laforet, France
  - **TC11.02** How to Examine a Patient with a Polyneuropathy  
Davide Pareyson, Italy
  - **TC11.03** How to Examine a Patient with a Neuromuscular Junction Disorder  
Lorenzo Maggi, Italy
- 13:30 - 15:30**
- **TC12** ◆ **Teaching Course: What a Biopsy can tell you in the Genomic Era**  
📍 **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** P. James B. Dyck, USA
  - **TC12.01** Muscle Biopsy  
Teresinha Evangelista, France
  - **TC12.02** Nerve Biopsy  
P James Dyck, USA
  - **TC12.03** Skin Biopsy  
Vincenzo Donadio, Italy
- 13:30 - 15:30**
- **TC13** ◆ **Teaching Course: Amyotrophic Lateral Sclerosis in 2026: Clinical Practice, Biomarkers and Precision Medicine**  
📍 **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Andrea Calvo, Italy
  - **TC13.01** Practical Diagnosis and Clinical Management of ALS  
Markus Weber, Switzerland
  - **TC13.02** Biomarker in ALS: Clinical Utility and Implementation  
Julian Großkreutz, Germany
  - **TC13.03** Emerging Therapies and the Future of ALS Trials  
Andrea Calvo, Italy
- 13:30 - 15:30**
- **TC14** ◆ **Teaching Course: Inherited Myopathies - Less Common Disorders**  
📍 **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Anthony Amato, USA
  - **TC14.01** Dominant LGMD  
Anthony Amato, USA
  - **TC14.02** Distal Myopathies  
Marco Savarese, Finland
  - **TC14.03** Late Onset Mitochondrial Myopathies  
Gabriele Siciliano, Italy
- 13:30 - 15:30**
- **TC15** ◆ **Teaching Course - Hands On: Ultrasound**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Stefan Meng, Austria
  - **TC15.01** Cranial Nerves and Trunk  
Stefan Meng, Austria, Austria
  - **TC15.02** Lower Extremities  
Jan-Hendrik Stahl, Germany
- 17:15 - 18:00**
- Opening Ceremony  
📍 **Location:** Cavaniglia Pavilion

- Teaching Courses
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- ✦ Ticketed Sessions
- Muscle Disorders
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- 08:30 - 10:30** ● **PL01 Plenary: Myology 2030**  
 📍 **Location:** Cavaniglia Pavilion  
 ★ **Chair(s):** Benedikt Schoser, Germany and Vincenzo Nigro, Italy
- 08:35 - 09:05 ● **PL01.01** Redefining Muscle Disease Management: A Roadmap to 2030  
 Benedikt Schoser, Germany
- 09:05 - 09:35 ● **PL01.02** Gene Diagnostics in 2030  
 Vincenzo Nigro, Italy
- 09:35 - 10:05 ● **PL01.03** Towards Effective Low-Dose Gene Therapy in Severe Muscular Dystrophy Models using a novel AI-designed AAV  
 Isabelle Richard, France
- 10:30 - 11:15** Morning Break & Poster Tour 1: Muscle and Neuromuscular Junction  
 📍 **Location:** Spadolini - Ground Floor  
 Eva de Boer, Netherlands
- 11:15 - 12:45** ● **SS01 Up-to-date on Muscle Inflammation**  
 📍 **Location:** Spadolini Attic - Hall 2  
 ★ **Chair(s):** Ichizo Nishino, Japan
- 11:20 - 11:40 ● **SS01.01** Myositis Entities: How Many are There and How Do They Differ?  
 Ichizo Nishino, Japan
- 11:40 - 12:00 ● **SS01.02** Classic and Digital Outcome Measures for Inflammatory Myopathies  
 Mazen Dimachkie, USA
- 12:00 - 12:20 ● **SS01.03** Inflammatory Aspects in Hereditary Muscle Diseases  
 Tobias Ruck, Germany
- 11:15 - 12:45** ● **SS02 Up-to-date on FSHD and OPMD**  
 📍 **Location:** Spadolini Attic - Hall 5  
 ★ **Chair(s):** Jeff Statland, USA
- 11:20 - 11:40 ● **SS02.01** FSHD 2026 and 2030  
 Jeffrey Statland, USA
- 11:40 - 12:00 ● **SS02.02** OPMD 2026 and 2030  
 Teresinha Evangelista, France
- 12:00 - 12:20 ● **SS02.03** Muscle Imaging as Outcome Measure 2026 and 2030  
 Kristl Claeys, Belgium
- 11:15 - 12:45** ● **SS03 Primary Lateral Sclerosis**  
 📍 **Location:** Spadolini Attic - Hall 3  
 ★ **Chair(s):** Michael van Es, Netherlands
- 11:20 - 11:50 ● **SS03.01** Genetic Profiling of Primary Lateral Sclerosis  
 Matthew B, Harms, USA
- 11:50 - 12:10 ● **SS03.02** Clinical Profiling of Primary Lateral Sclerosis  
 Michael van Es, Netherlands
- 11:15 - 12:45** ● **SS04 FcRn and Complement Inhibitors in MG Severe Deteriorations**  
 📍 **Location:** Spadolini Attic - Hall 4  
 ★ **Chair(s):** Andreas Meisel, Germany
- 11:20 - 11:40 ● **SS04.01** What we have learnt from Comparative Analyses?  
 Carlo Antozzi, Italy
- 11:40 - 12:00 ● **SS04.02** FcRn Blockers for MG Crisis  
 Shahar Shelly, Israel
- 12:00 - 12:20 ● **SS04.03** Complement Inhibitors for MG Crisis  
 Andreas Meisel, Germany

- Teaching Courses
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- ◆ Ticketed Sessions
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- 11:15 - 12:45** ● **SS05 ICNMD & World Muscle Society Joint Session**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Laurent Servais, United Kingdom
- 11:25 - 11:45 ● **SS05.01** When Drugs are Available... but not for Everyone.  
 Emna Farhat, Tunisia
- 11:45 - 12:05 ● **SS05.02** When war Disrupts Patients Care  
 Natalia Samonenko, Ukraine
- 12:05 - 12:35 ● **SS05.03** Crowdfunding, Lottery, Relocation... Treatment Access Inequity from a Global North Perspective  
 Laurent Servais, United Kingdom
- 12:45 - 14:30** Lunch & Poster Viewing  
📍 **Location:** Spadolini - Ground Floor
- 13:15 - 14:15** ● **Industry-Supported Symposia**  
📍 **Location:** Spadolini - Attic
- 14:30 - 16:00** ● **SS06 Up-to-date on Metabolic Muscle Disorders**  
📍 **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** Antonio Toscano, Italy
- 14:35 - 14:55 ● **SS06.01** Update on Mitochondrial Muscle Disorders  
 Rita Horvath, United Kingdom
- 14:55 - 15:15 ● **SS06.02** Update on Muscle Glycogenoses  
 Antonio Toscano, Italy
- 15:15 - 15:35 ● **SS06.03** Current and Future Therapies in Mitochondrial Myopathies  
 Michelangelo Mancuso, Italy
- 14:30 - 16:00** ● **SS07 Biomarkers of Early Diagnosis in ALS**  
📍 **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Michael van Es, Netherlands
- 14:35 - 14:55 ● **SS07.01** Trial Design  
 Merit Cudkowicz, USA
- 14:55 - 15:15 ● **SS07.02** Neurophysiology  
 Markus Weber, Switzerland
- 15:15 - 15:35 ● **SS07.03** Neuroimaging  
 Julian Grosskreutz, Germany
- 14:30 - 16:00** ● **SS08 Tools for the Next Generation Myologists**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Massimiliano Filosto, Italy
- 14:35 - 14:55 ● **SS08.01** AI for Redefining Natural History in Muscle Disorders  
 Massimiliano Filosto, Italy
- 14:55 - 15:15 ● **SS08.02** AI in Genetic Diagnosis  
 Joanna Polańska, Poland
- 15:15 - 15:35 ● **SS08.03** AI in histopathology?  
 Edoardo Malfatti, France
- 14:30 - 16:00** ● **SS09 Advances in TTR Amyloid**  
📍 **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Laura Obici, Italy
- 14:35 - 14:55 ● **SS09.01** Early Recognition and Diagnosis of TTR Amyloid Neuropathy  
 Michelle Mauermann, USA
- 14:55 - 15:15 ● **SS09.02** Update on Therapies of TTR Neuropathy  
 Laura Obici, Italy
- 15:15 - 15:35 ● **SS09.03** CNS TTR Amyloidosis – an Evolving Challenge  
 Chafic Karam, USA

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**14:30 - 16:00** ● **SS17 ICNMD, AIM & ASNP Joint Session**

📍 **Location:** Spadolini Attic - Hall 1

★ **Chair(s):** Vincenzo Nigro, Italy

14:35 - 14:55 ● **SS17.01** Diagnostic Approach to Peripheral Neuropathy  
Fiore Manganelli, Italy

14:55 - 15:15 ● **SS17.02** Update on Genetic Myasthenic Syndromes  
Lorenzo Maggi, Italy

15:15 - 15:35 ● **SS17.03** Axial Myopathies: Clinical Phenotypes and Differential Diagnosis  
Tiziana Mongini, Italy

**16:00 - 16:45** Afternoon Break & Showcase Theatre

📍 **Location:** Spadolini - Ground Floor

**16:45 - 18:15** ● **SS11 Up.-to-date on Limb Girdle Myopathies**

📍 **Location:** Spadolini Attic - Hall 1

★ **Chair(s):** John Vissing, Denmark

16:45 - 17:05 ● **SS11.01** Emerging Disease-specific Treatments in LGMDs with a Focus on LGMDR9  
John Vissing, Denmark

17:05 - 17:25 ● **SS11.02** Novel Forms of LGMD  
Edmar Zanoteli, Brazil

17:25 - 17:45 ● **SS11.03** Overcoming Challenges in the Molecular Diagnosis of LGMD  
Marco Savarese, Finland

**16:45 - 18:15** ● **SS12 Early-phase Treatments in MG**

📍 **Location:** Spadolini Attic - Hall 2

★ **Chair(s):** James Howard, USA

16:50 - 17:10 ● **SS12.01** DNA CAR/CAAR T Cells  
Aiden Haghikia, Germany

17:10 - 17:30 ● **SS12.02** RNA CAR T Cells  
James Howard, USA

17:30 - 17:50 ● **SS12.03** Trials on B Cell Depleting Agents: Success and Failure  
Raffaele Iorio, Italy

**16:45 - 18:15** ● **OS01 Oral Session 1**

📍 **Location:** Spadolini Attic - Hall 3

★ **Chair(s):** Mazen Dimachkie, USA

16:45 - 17:00 ● **OS01.01** Phase 2/3 Study of a Monoclonal Antibody Targeting KLRG1 in Inclusion Body Myositis  
Namita Goyal, USA

17:00 - 17:15 ● **OS01.02** Genetic Modifier LTBP4 Stratification of Proteomics Points to Interacting IL-23/  
IL-6/IL-17D Pathways in Severity of DMD  
Eric Hoffman, USA

17:15 - 17:30 ● **OS01.03** Targeting Musashi-2 With Antisense Oligonucleotides Mitigates Muscle Pathology  
in Myotonic Dystrophy Type 1  
Dulce Peris-Moreno, Spain

17:30 - 17:45 ● **OS01.04** The Facial-Sparing FSHD Phenotype: A Distinct Entity With Implications for  
Diagnosis, Prognosis, and Trial Design  
Andi Nuredini, Italy

17:45 - 18:00 ● **OS01.05** A Trial of First-Line Add-on Intravenous Immune Globulin in Idiopathic  
Inflammatory Myopathies: TIME IS MUSCLE  
Pinar Özkaynar, Netherlands

18:00 - 18:15 ● **OS01.06** Phase 1/2 Results of an Antisense Oligonucleotide Therapy for Exon-Skipping-  
Amenable DMD  
Giovanni Baranello, United Kingdom

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- 16:45 - 18:15** ● **OS02 Oral Session 2**  
 📍 **Location:** Spadolini Attic - Hall 4  
 ★ **Chair(s):** Jorge A. Bevilacqua, Chile
- 16:45 - 17:00 ● **OS02.01** Integrated Group-Based and Machine Learning Analysis Reveals Sex-Related Clinical Signatures in Autoimmune Myasthenia Gravis  
 Paolo Doksani, Germany
- 17:00 - 17:15 ● **OS02.02** Muscle Transcriptome Profiling Enables In-Depth Characterization of Novel Molecular Pathways Underlying LAMA2-RD Across Disease Severity  
 Veronica Pini, United Kingdom, Italy
- 17:15 - 17:30 ● **OS02.03** Real-World Evaluation of Lamotrigine as an Anti-Myotonic Therapy in Myotonic Dystrophy Type 1  
 Barbara Risi, Italy
- 17:30 - 17:45 ● **OS02.04** Long-Term Safety and Efficacy of an AAV-Based Gene Therapy forX-Linked Myotubular Myopathy  
 Nancy L. Kuntz, USA
- 17:45 - 18:00 ● **OS02.05** An HDAC Inhibitor Reduces Decline of Contractile Cross-Sectional Area and Decreases Fat Infiltration in Duchenne Muscular Dystrophy  
 Eugenio Mercuri, Italy
- 18:00 - 18:15 ● **OS02.06** Characterization of Juvenile-Onset MG in a Nationwide Swedish Cohort  
 Oskar Sunnegardh, Sweden
- 16:45 - 18:15** ● **OS03 Oral Session 3**  
 📍 **Location:** Spadolini Attic - Hall 5  
 ★ **Chair(s):** Vincenzo Nigro, Italy
- 16:45 - 17:00 ● **OS03.01** Advances in Clinical Genetic Epidemiology and Therapy of Nlsgm/nlsgd :(Chanarin-Dorfman) as a Mediterranean Trait  
 Corrado Angelini, Italy
- 17:00 - 17:15 ● **OS03.02** Short Tandem Repeats as Risk Factors and Survival Modifiers of ALS  
 Kang-Yang Jih, Taiwan
- 17:15 - 17:30 ● **OS03.03** Chronology of Subclinical Nerve Hypertrophy in Latin American Hereditary Transthyretin Amyloidosis With Polyneuropathy  
 Edicson Ruiz-Ospina, Colombia, Colombia
- 17:30 - 17:45 ● **OS03.04** Whole Body Imaging Reveals Stage-Dependent Imaging Markers in Calpainopathy and Dysferlinopathy  
 Ai Yamanaka, Japan
- 17:45 - 18:00 ● **OS03.05** A Multicenter Study of CCDC78-Related Myopathy: Clinical and Ultrastructural Insights  
 Diego Lopergolo, Italy
- 18:00 - 18:00 ● **OS03.06** Safety and Efficacy of an Enzyme Replacement Therapy in Infantile-Onset Pompe Disease  
 Andreas Hahn, Germany

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### 07:00 - 08:00 ● Industry-Supported Symposia

📍 Location: Spadolini - Attic

### 08:30 - 10:30 ● PL02 Plenary: Cutting Edge Updates in Neuropathy

📍 Location: Cavaniglia Pavilion

★ Chair(s): Giuseppe Lauria, Italy and Michelle L. Mauermann, USA

08:35 - 09:05 ● PL02.01 Painful Neuropathies: New insights/New treatment targets  
Daniela Menichella, USA

09:05 - 09:35 ● PL02.02 Unraveling Neuromuscular Disorders Caused by Non-Coding Repeat Expansions  
Andrea Cortese, United Kingdom, Italy

09:35 - 10:05 ● PL02.03 Advancing Care in CIDP: Exploring Emerging Treatments  
Pietro Doneddu, Italy

### 10:30 - 11:15 Morning Break & Poster Tour 2: Motor Neuron

📍 Location: Spadolini - Ground Floor

### 11:15 - 12:45 ● SS13 Cancer Related Peripheral Neuropathies

📍 Location: Spadolini Attic - Hall 2

★ Chair(s): Antonio Toscano, Italy

11:20 - 11:45 ● SS13.01 Update in Chemotherapy Induced Neuropathies  
Roser Velasco, Spain

11:45 - 12:05 ● SS13.02 Hematologic Related Neuropathies  
Chiara Briani, Italy

12:05 - 12:25 ● SS13.03 Checkpoint Inhibitor Neuropathies  
Stefano Tamburin, Italy

### 11:15 - 12:45 ● SS14 Challenges in MG Treatment

📍 Location: Spadolini Attic - Hall 3

★ Chair(s): Pushpa Narayanaswami, USA

11:20 - 11:40 ● SS14.01 AEs in MG Treatment  
Michael Hehir, USA

11:40 - 12:00 ● SS14.02 The Cost of Care of MG: Direct and Indirect  
Pushpa Narayanaswami, USA

12:00 - 12:20 ● SS14.03 Can Targeted Treatments be Stopped, and When?  
Isabel Leite, United Kingdom

### 11:15 - 12:45 ● SS15 Biomarkers of Disease Progression and Prognosis

📍 Location: Spadolini Attic - Hall 5

★ Chair(s): Nicola Ticozzi, Italy

11:20 - 11:40 ● SS15.01 Wet Biomarkers  
Federico Verde, Italy

11:40 - 12:00 ● SS15.02 Neurophysiology  
Raffaele Dubbioso, Italy

12:00 - 12:20 ● SS15.03 Neuroimaging  
Julian Grosskreutz, Germany

### 11:15 - 12:45 ● SS16 Up-to-date on Immune-medicated Neuropathies

📍 Location: Spadolini Attic - Hall 4

★ Chair(s): Eduardo Nobile Orazio, Italy

11:20 - 11:40 ● SS16.01 Updates in GBS  
Umapathi Thirugnanam, Singapore

11:40 - 12:00 ● SS16.02 Update on Multifocal Motor Neuropathy  
Eduardo Nobile-Orazio, Italy

12:00 - 12:20 ● SS16.03 Updates in Vasculitic Neuropathy  
Judith Spies, Australia

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- 11:15 - 12:45** ● **SS10 ICNMD & Peripheral Nerve Society Joint Session**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Marcus Pinto, USA
- 11:20 - 11:35 ● **SS10.01** Chemotherapy Induced Peripheral Neuropathy  
 Paola Alberti, Italy
- 11:35 - 11:55 ● **SS10.02** Painful Neuropathies: New Insights/New Treatment targets  
 Claudia Sommer, Germany
- 11:55 - 12:15 ● **SS10.03** Gene Therapy for Inherited Neuropathies – how to Optimally Deliver to Peripheral Nerves  
 Kleopas Kleopa, Cyprus
- 12:15 - 12:35 ● **SS10.04** Antibodies in Autoimmune Neuropathies  
 Kavita Grover, USA
- 12:45 - 14:30** Lunch & Poster Viewing  
📍 **Location:** Spadolini - Ground Floor
- 13:15 - 14:15** ● **Industry-Supported Symposia**  
📍 **Location:** Spadolini - Attic
- 14:30 - 16:00** ● **SS18 Up-to-date on Myotonic Dystrophies**  
📍 **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** Valeria Sansone, Italy
- 14:35 - 14:55 ● **SS18.01** Myotonic Dystrophy Type 1 2026 and 2030  
 Valeria Sansone, Italy
- 14:55 - 15:15 ● **SS18.02** Myotonic Dystrophy Type 2 in 2026 and 2030  
 Nicholas Johnson, USA
- 15:15 - 15:35 ● **SS18.03** Periodic Paralysees and Non-Dystrophic Myotonic Syndromes 2026 and 2030  
 Kristl Claeys, Belgium
- 14:30 - 16:00** ● **SS19 Technologies Updates in Neuropathies (formally Updates in CMT)**  
📍 **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Margheria Marchi, Italy
- 14:35 - 15:05 ● **SS19.02** Artificial Intelligence in Genomics: Opportunities and Challenges  
 Erika Salvi, Italy
- 15:05 - 15:35 ● **SS19.03** The Power of Long Read Sequencing  
 Stefania Magri, Italy
- 14:30 - 16:00** ● **SS20 Tailoring MG Care for Women**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Sarah Hoffmann, Germany
- 14:35 - 14:55 ● **SS20.01** Tailoring MG Care for Women: Sex and Personalized Medicine  
 Sarah Hoffmann, Germany
- 14:55 - 15:15 ● **SS20.02** Planning Pregnancy in Myasthenia Gravis (MG)  
 Nils Erik Gilhus, Norway
- 15:15 - 15:3 ● **SS20.03** Considerations for the Use of New Targeted Drugs in Women of Childbearing Age  
 Pushpa Narayanaswami, USA
- 14:30 - 16:00** ● **SS21 Hereditary Neuropathy Overlap Syndromes**  
📍 **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Davide Pareyson, Italy
- 14:35 - 14:55 ● **SS21.01** Hereditary Neuropathy and Myopathy: Overlapping Features  
 Tanya Stojkovic, France
- 14:55 - 15:15 ● **SS21.02** Ataxic Neuropathies  
 Davide Pareyson, Italy
- 15:15 - 15:35 ● **SS21.02** CMT and Motor Neuronopathies  
 Stefano Previtali, Italy

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- 14:30 - 16:00**    ● **SS22 ICNMD & German Nerve Club Joint Session - Nerve Surgery, Diagnostics and Rehabilitation**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Robert Schmidhammer, Germany
- 14:35 - 15:05    ● **SS22.01** Nerve Surgery the Power of One: Advances With Rare Peripheral Nerve Tumors  
 Robert Spinner, USA
- 15:05 - 15:12    ● **SS22.02** Size does not Matter - Ultrasound Parameters and Patient-Reported Outcomes after Median and Ulnar Nerve Repair  
 Henrik Lauer, Germany
- 15:12 - 15:19    ● **SS22.03** Nerve Ultrasound and its Role in Detecting Neuralgic Amyotrophy  
 Alexander Grimm, Germany
- 15:19 - 15:26    ● **SS22.04** Between Neuralgic Amyotrophy and Torsional Neuropathy – the Role of Surgery  
 Nora Dengler, Germany
- 15:26 - 15:33    ● **SS22.05** The Thoracic Outlet Syndrome  
 Franz Lassner, Germany
- 15:33 - 15:40    ● **SS22.06** The Potential for Gain of Function Under Direct Nerve Stimulation Based on the Long-Term Results of Peroneal Nerve Stimulation  
 Klaus Daniel Martin, Germany
- 15:40 - 15:47    ● **SS22.07** Peripheral Nerve Stimulation as a Therapeutic Option for Severe Neuropathic Pain  
 Walter Demmel, Germany
- 15:47 - 15:54    ● **SS22.08** Addressing Peripheral Neuropathic Pain from Nerve Scarring with Perforator-Based Gliding Tissue Flaps: Two Decades of Clinical Practice  
 Savas Tsolakidis, Austria

**16:00 - 16:45**    Afternoon Break & Showcase Theatre  
📍 **Location:** Spadolini - Ground Floor

- 16:45 - 18:15**    ● **SS23 Update on Diagnostic Tools in Neuropathy**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** P. James B. Dyck, USA
- 16:50 - 17:15    ● **SS23.01** US/MRI in Evaluating/diagnosing Peripheral Neuropathies  
 Stephan Goedee, Netherlands
- 17:15 - 17:35    ● **SS23.02** Role of Nerve Biopsy in Diagnoses of PN  
 P James Dyck, USA
- 17:35 - 17:55    ● **SS23.03** Peripheral Nerve Surgery  
 Robert Spinner, USA
- 16:45 - 18:15**    ● **SS24 Patient Monitoring in NMJ Diseases**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Sithara Ramdas, United Kingdom
- 16:50 - 17:10    ● **SS24.01** CMS: Outcome Measures and Natural History  
 Sithara Ramdas, United Kingdom
- 17:10 - 17:30    ● **SS24.02** Remote Monitoring of MG Patients  
 Maike Stein, Germany
- 17:30 - 17:50    ● **SS24.03** Computer Vision and AI for the Neurological Exam  
 Henry Kaminski, USA

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- 16:45 - 18:15** ● **OS04 Oral Session 4**  
 📍 **Location:** Spadolini Attic - Hall 2  
 ★ **Chair(s):** Amelia Evoli, Italy
- 16:45 - 17:00 ● **OS04.01** Long-Term Remission with Anti-CD19 CAR-T Cell Therapy in Refractory Myasthenia Gravis: A Two-Year Case Series  
 Tobias Hegelmaier, Germany
- 17:00 - 17:15 ● **OS04.02** Comparative Effectiveness of a Conventional Immunosuppressant and a B-Cell-Depleting Therapy in Myasthenia Gravis  
 Wanqing Wu, Sweden
- 17:15 - 17:30 ● **OS04.03** Early First-Week Changes in MG-ADL and MG-QOL-15 During C5 and FcRn-Inhibitors Treatment in Myasthenia Gravis  
 Claudia Vinciguerra, Italy
- 17:30 - 17:45 ● **OS04.04** Repeated Cycles of an FcRn-Targeting Therapy in Antibody-Positive Generalised Myasthenia Gravis  
 Fiammetta Vanoli, Italy
- 17:45 - 18:00 ● **OS04.05** Efficacy of Complement vs. FcRn-Inhibition in Thymoma-Associated Myasthenia Gravis: An Italian Multicentre Real-World Study  
 Alba Cepele, Italy
- 18:00 - 18:15 ● **OS04.06** An si-RNA Based Complement Pathway Inhibitor for gMG: Efficacy, Safety, and Impact on Quality of Life/MGC from the NIMBLE Trial  
 Renato Mantegazza, Italy
- 16:45 - 18:15** ● **OS05 Oral Session 5**  
 📍 **Location:** Spadolini Attic - Hall 3  
 ★ **Chair(s):** Michelle L Mauermann, USA
- 16:45 - 17:00 ● **OS05.01** Prospective Study of an FcRn Inhibitor in Myasthenia Gravis Crisis  
 Shahar Shelly, Israel
- 17:00 - 17:15 ● **OS05.02** TYMS Gene Polymorphisms and Thymic Pathology in Myasthenia Gravis  
 Paulo José Lorenzoni, Brazil
- 17:15 - 17:30 ● **OS05.03** Subclinical Cardiomyopathy in Premanifest Carriers of Late-Onset Hereditary Transthyretin Amyloidosis with Polyneuropathy  
 Te-Wei Wang, Taiwan
- 17:30 - 17:45 ● **OS05.04** Serum Neurofilament Light Chain as a Biomarker of Global Axonal Burden in Immune-Mediated Demyelinating Neuropathies  
 Namjin Heo, Korea, Republic of
- 17:45 - 18:00 ● **OS05.05** Serum Peripherin as a Disease Biomarker in Hereditary Transthyretin Amyloidosis: A Multicenter Cohort Study  
 Guido Primiano, Italy
- 18:00 - 18:15 ● **OS05.06** Static and Dynamic Stabilometric Evaluation and Rehabilitation in Peripheral Neuropathy  
 Edoardo Roveta, Italy
- 16:45 - 18:15** ● **OS06 Oral Session 6**  
 📍 **Location:** Spadolini Attic - Hall 4  
 ★ **Chair(s):** Jessica Mandrioli, Italy
- 16:45 - 17:00 ● **OS06.01** The Landscape of Non-Reference SINE-VNTR-Alus in Amyotrophic Lateral Sclerosis  
 Sulev Koks, Australia
- 17:00 - 17:15 ● **OS06.02** Prognostic Factors in Guillain-Barré Syndrome: A Retrospective Cohort Study in a Tertiary Referral Centre  
 Sonia Messina, Italy
- 17:15 - 17:30 ● **OS06.03** Oxidative Stress-Driven Somatic Mosaicism Marks a Distinct Molecular Subtype of Sporadic ALS  
 Hyunjin Kim, Korea, Republic of
- 17:30 - 17:45 ● **OS06.04** GM1 Neuropathies, From Antibodies to Phenotype  
 Nicolas Dubuisson, United Kingdom, Belgium
- 17:45 - 18:00 ● **OS06.05** Long-Term Exposure to Air Pollution and the Risk and Prognosis of Motor Neuron Disease  
 Jing Wu, Sweden
- 18:00 - 18:15 ● **OS06.06** Rebalancing DNM2 Activity Ameliorates DNM2- and MTMR2-Related Charcot-Marie-Tooth Neuropathies  
 Jocelyn Laporte, France

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### 07:00 - 08:00 ● Industry-Supported Symposia

📍 Location: Spadolini - Attic

### 08:30 - 10:30 ● PL03 Plenary: Redefining ALS: A Paradigm Shift in Understanding and Care

📍 Location: Cavaniglia Pavilion

★ Chair(s): Adriano Chiò, Italy and Angela Genge, Canada

#### 08:35 - 09:05 ● PL03.01 SOD1 Pathology in SOD1 ALS and Sporadic ALS

Pam Shaw, United Kingdom

#### 09:05 - 09:35 ● PL03.02 Unravelling the Presymptomatic Phase of ALS: Clinical, Genetic, and Molecular Advances Toward Early Detection

Andrea Malaspina, United Kingdom

#### 09:35 - 10:05 ● PL03.03 The Global Fight to End ALS

Thanuja Dharmadasa, Australia

### 10:30 - 11:15 Morning Break & Poster Tour 3: Peripheral Neuropathy

📍 Location: Spadolini - Ground Floor

### 11:15 - 12:45 ● SS25 New Outcome Measures

📍 Location: Spadolini Attic - Hall 1

★ Chair(s): Edoardo Spinelli, Italy

#### 11:20 - 11:40 ● SS25.01 Wet Biomarkers

Angela Genge, Canada

#### 11:40 - 12:00 ● SS25.02 Patients' Self Reported Outcomes

Umberto Manera, Italy

#### 12:00 - 12:20 ● SS25.03 Neuroimaging

Edoardo Spinelli, Italy

### 11:15 - 12:45 ● SS26 ALS Clinical Trials: Novel Approaches Toward an Effective Cure

📍 Location: Spadolini Attic - Hall 2

★ Chair(s): Ruben van Eijk, Netherlands

#### 11:20 - 11:40 ● SS26.01 TRICALS

Ruben van Eijk, Netherlands

#### 11:40 - 12:00 ● SS26.02 HEALEY Platform

Senda Ajroud-Driss, USA

#### 12:00 - 12:20 ● SS26.03 ACCESS ALS

Angela Genge, Canada

### 11:15 - 12:45 ● SS27 Diabetic Neuropathies

📍 Location: Spadolini Attic - Hall 3

★ Chair(s): Vincenza Spallone, Italy

#### 11:20 - 11:40 ● SS27.01 Diabetic Neuropathy

Rayaz Malik, Qatar

#### 11:40 - 12:00 ● SS27.02 Diabetic Lumbosacral Radiculoplexus Neuropathy: Recent Updates and Unmet Needs

Marcus Pinto, USA

#### 12:00 - 12:20 ● SS27.03 GLP1 Agonists and Neuropathy

Vincenza Spallone, Italy

### 11:15 - 12:45 ● SS28 From Duchenne to Becker Musclar Dystrophies

📍 Location: Spadolini Attic - Hall 4

★ Chair(s): Carisi Anne Polanczyk, Brazil

#### 11:20 - 11:40 ● SS28.01 Looking forward: Duchenne Muscular Dystrophy in 2030

Francesco Muntoni, United Kingdom

#### 11:40 - 12:00 ● SS28.02 Becker Muscular Dystrophy 2026 and in 2030

Michela Guglieri, United Kingdom

#### 12:00 - 12:00 ● SS28.03 Dystrophinopathies in women 2026 and 2030

Tiziana Mongini, Italy

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**11:15 - 12:45** ● **SS29 ICNMD & World Federation of Neurology Joint Session**

📍 **Location:** Spadolini Attic - Hall 5

★ **Chair(s):** Wolfgang Grisold, Austria

11:20 - 11:40 ● **SS29.01** Neuromuscular Late Effects of Covid  
Maurizio Leone, Italy

11:40 - 12:00 ● **SS29.02** Leprosy  
Pedro Tomaselli, Brazil

12:00 - 12:20 ● **SS29.03** India -Emergencies in Neuromuscular Diseases.  
Sarath Menon, India

**12:45 - 14:30** Lunch & Poster Viewing

📍 **Location:** Spadolini - Ground Floor

**13:15 - 14:15** ● **Industry-Supported Symposia**

📍 **Location:** Spadolini - Attic

**14:30 - 16:00** ● **SS30 Up-to-date Supportive and Palliative Care in Muscle Disorders**

📍 **Location:** Spadolini Attic - Hall 2

★ **Chair(s):** Jorge Bevilacqua, Chile

14:35 - 15:05 ● **SS30.01** Challenges in Supportive and Palliative Neuromuscular care in Developing Countries

Jorge A. Bevilacqua, Chile

15:05 - 15:35 ● **SS30.03** Mental health in Muscle Disorders  
Marianne de Visser, Netherlands

**14:30 - 16:00** ● **SS31 A Worldwide Overview of ALS**

📍 **Location:** Spadolini Attic - Hall 3

★ **Chair(s):** Monica Povedano, Spain

14:35 - 14:55 ● **SS31.01** South America

Monica Povedano, Spain

14:55 - 15:15 ● **SS31.02** South Korea  
Hyunjin Kim, Korea, Republic of

15:15 - 15:35 ● **SS31.03** Asia and Pacific  
Atchayaram Nalini, India

**14:30 - 16:00** ● **SS32 Updates on ALS Clinical Trials**

📍 **Location:** Spadolini Attic - Hall 5

★ **Chair(s):** Angela Genge, Canada

14:35 - 14:55 ● **SS32.01** Harmonization of ALSFRS Update  
Leonard, Van de Berg, Netherlands

14:55 - 15:15 ● **SS32.02** Joint Model is the Future  
Ruben Van Eijk, Netherlands

15:15 - 15:35 ● **SS32.03** New Take on Outcome Measures  
Angela Genge, Canada

**14:30 - 16:00** ● **SS33 MG in the Elderly**

📍 **Location:** Spadolini Attic - Hall 4

★ **Chair(s):** Valentina Damato, Italy

14:35 - 14:55 ● **SS33.01** Epidemiological Changes in MG  
Valentina Damato, Italy

14:55 - 15:15 ● **SS33.02** Immunocellular Phenotyping in MG  
Susanna Brauner, Sweden

15:15 - 15:35 ● **SS33.03** MG Prognosis in Very Late Onset Patients  
Elena Cortes-Vicente, Spain

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**14:30 - 16:00** ● **SS34 ICNMD & The Autonomic Disorder Group of the WFN Joint Session**

📍 **Location:** Spadolini Attic - Hall 1

★ **Chair(s):** Paola Sandroni, USA

- 14:35 - 14:55 ● **SS34.01** Autonomic Testing  
Valerie Iodice, United Kingdom
- 14:55 - 15:15 ● **SS34.02** Dysfunctional Autonomic Disorders  
Paola Sandroni, USA
- 15:15 - 15:45 ● **SS34.03** Autonomic Neuropathies  
Jinwoo Park, Korea, Republic of

**16:00 - 16:45** Afternoon Break

📍 **Location:** Spadolini - Ground Floor

**16:45 - 18:15** ● **SS35 Cognition in ALS**

📍 **Location:** Spadolini Attic - Hall 1

★ **Chair(s):** Dorothee Lulé, Germany

- 16:50 - 17:10 ● **SS35.01** Correlates and Impact of Cognitive Impairment in ALS  
Dorothee Lulé, Germany
- 17:10 - 17:30 ● **SS35.02** Behavioural and Neuropsychiatric Symptoms  
Edoardo Nicolò Aiello, Italy
- 17:30 - 17:50 ● **SS35.03** Social Cognition  
Francesca Palumbo, Italy

**16:45 - 18:15** ● **SS36 Advanced Topics in Peripheral Neuropathy**

📍 **Location:** Spadolini Attic - Hall 2

★ **Chair(s):** Natan Staff, USA

- 16:50 - 17:10 ● **SS36.01** Biomarkers in Chemotherapy Induced Peripheral Neuropathy  
Nathan Staff, USA
- 17:10 - 17:30 ● **SS36.02** Skin Biopsy – Beyond Epidermal Nerve Fiber Density  
Giuseppe Lauria, Italy
- 17:30 - 17:50 ● **SS36.03** Antibody Discovery in Immune Neuropathies  
Divyanshu Dubey, USA

**16:45 - 18:15** ● **OS07 Oral Session 7**

📍 **Location:** Spadolini Attic - Hall 3

★ **Chair(s):** Angela Genge, Canada

- 16:45 - 17:00 ● **OS07.01** Can Amyotrophic Lateral Sclerosis Diagnostic Index (ALSDI) Refine ALS Diagnosis Beyond Gold Coast Criteria?  
Amirul Asyraf Ab Ghapar, Australia
- 17:00 - 17:15 ● **OS07.02** Diagnostic Utility of Ulnar Far Field Potentials in Amyotrophic Lateral Sclerosis  
Aicee Dawn Calma, Australia
- 17:15 - 17:30 ● **OS07.03** Use of Intravenous Immunoglobulin in Participants with Post-Polio Syndrome: The Randomized, Placebo-Controlled FORCE Trial  
Johannes Jakobsen, Denmark
- 17:30 - 17:45 ● **OS07.04** Respiratory Function Improvement in SMA After Long-Term Oral SMN-Splicing Modifier Therapy  
Sonia Messina, Italy
- 17:45 - 18:00 ● **OS07.05** NBS for 5q SMA in Minas Gerais, Brazil: 18 Months of Program Implementation  
Juliana Gurgel Giannetti, Brazil
- 18:00 - 18:15 ● **OS07.06** Treatment Switching from an Intrathecal Antisense Therapy to an Oral SMN Modifying Therapy in Spinal Muscular Atrophy  
Eshgin Maharramov, Turkey

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- 16:45 - 18:15** ● **OS08 Oral Session 8**  
 📍 **Location:** Spadolini Attic - Hall 4  
 ★ **Chair(s):** Marco Savarese, Finland
- 16:45 - 17:00 ● **OS08.01** Human Spinal Cord Organoids Reveal Therapeutic Windows and Ciliary Pathology in Spinal Muscular Atrophy  
 Stefania Corti, Italy
- 17:00 - 17:15 ● **OS08.02** Top Stride Velocity (SV95C) is a Validated Endpoint in DMD Below 4 Years Old  
 Damien Eggenspieler, France
- 17:15 - 17:30 ● **OS08.03** Molecular Imaging of Muscle Involvement in Facioscapulohumeral Muscular Dystrophy Using Multispectral Optoacoustic Tomography  
 Mauro Monforte, Italy, Italy
- 17:30 - 17:45 ● **OS08.04** High Diagnostic Yield in a Large Monocentric Cohort of Patients Affected With Primary Muscle Disorders  
 Dario Ronchi, Italy
- 17:45 - 18:00 ● **OS08.05** Determining Autoimmunity in Seronegative Myasthenic Syndromes: CBA and Human Muscle Cell Assay Results in D-a-CH  
 Paolo Doksan, Germany
- 18:00 - 18:15 ● **OS08.06** One-Year Longitudinal Quantitative Whole-Body Muscle MRI and Functional Outcomes in Adults With FSHD1  
 Matthias Opsomer, Belgium
- 16:45 - 18:15** ● **OS09 Oral Session 9**  
 📍 **Location:** Spadolini Attic - Hall 5  
 ★ **Chair(s):** Pushpa Narayanaswami, USA
- 16:45 - 17:00 ● **OS09.01** Enhancing the Diagnosis of Myasthenia Gravis Using Objective Oculomotor Assessment  
 Fatemeh Rezaia, Australia
- 17:00 - 17:15 ● **OS09.02** Decoding the Mechanisms of Muscle Wasting for Developing a Rna-Based Multitarget Therapeutic Approach  
 Marco Sandri, Italy
- 17:15 - 17:30 ● **OS09.03** Characterization of AChR-Specific CD4+ T Cell Responses in Myasthenia Gravis with HLA-DRB Risk Alleles  
 Mingming Li, Sweden
- 17:30 - 17:45 ● **OS09.04** Diagnostic Support for Myotonic Dystrophy Type 1 (DM1, ORPHA:273) Using German Real-World Data  
 Jana Zschuentzsch, Germany
- 17:45 - 18:00 ● **OS09.05** A Potential Provocation: Adjusting Frontal Area Temperature How Alter Jitter in Myasthenics and Healthy Individuals  
 Selen Ozyurt Kose, Turkey
- 18:00 - 18:15 ● **OS09.06** The Uptake, Intracellular Trafficking and Recycling of FcRn-Blocking Therapeutics in Human Endothelial Cells in Vitro  
 Anthony Shock, United Kingdom

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- 08:30 - 10:30** ● **PL04 Plenary: Translating Advances in Pathophysiology to Clinical Applications in NMJ Diseases**  
 📍 **Location:** Cavaniglia Pavilion  
 ★ **Chair(s):** Amelia Evoli, Italy and Stephen Reddel, Australia
- 08:35 - 09:05 ● **PL04.01** CMS : Update on Diagnosis and Treatment  
 Hanns Lochmuller, Canada
- 09:05 - 09:35 ● **PL04.02** Ready for Change? What the New Guidelines/recommendations Say  
 Carolina Barnett Tapia, Canada
- 09:35 - 10:05 ● **PL04.03** Immune Checkpoint Inhibitor-Related MG  
 Yves Allenbach, France
- 10:30 - 11:00** Morning Break  
 📍 **Location:** Spadolini - Attic
- 11:00 - 12:30** ● **SS37 Defining MG Subtypes**  
 📍 **Location:** Spadolini Attic - Hall 2  
 ★ **Chair(s):** Henry Kaminski, USA
- 11:20 - 11:40 ● **SS37.01** Machine Learning Expands MG ACHR Subtypes  
 Tobias Ruck, Germany
- 11:40 - 12:00 ● **SS37.02** Whole RNA Profile of Response to Thymectomy  
 Henry Kaminski, USA
- 12:00 - 12:20 ● **SS37.03** Metabolically Reprogrammed CD37low/CD98hchi B cells are Linked to Treatment Refractoriness in MG  
 Raffaele Iorio, Italy
- 11:00 - 12:30** ● **SS38 Lepromatous Neuropathy**  
 📍 **Location:** Spadolini Attic - Hall 3  
 ★ **Chair(s):** Pedro Tomaselli, Brazil
- 11:20 - 11:40 ● **SS38.01** Clinical Presentation  
 Pedro Tomaselli, Brazil
- 11:40 - 12:00 ● **SS38.02** Risk Factors and Diagnostic Tools  
 Osvaldo Nascimento, Brazil
- 12:00 - 12:20 ● **SS38.03** Treatment and Complications  
 Joy Vijaayan, Singapore
- 11:00 - 12:30** ● **SS39 Up-to-date Fatigue, Ventilation and Sleep Management**  
 📍 **Location:** Spadolini Attic - Hall 5  
 ★ **Chair(s):** Olimpia Musumeci, Italy
- 11:20 - 11:40 ● **SS39.01** Muscle Fatigue  
 Anna Kostera-Puszczczyk, Poland
- 11:40 - 12:00 ● **SS39.02** Interventional Respiratory Training  
 Stephan Wenninger, Germany
- 12:00 - 12:20 ● **SS39.03** Sleep and Muscle Diseases  
 Olimpia Musumeci, Italy
- 11:00 - 12:30** ● **SS40 Cancer and Thymoma Related Diseases**  
 📍 **Location:** Spadolini Attic - Hall 4  
 ★ **Chair(s):** Gil Wolfe, USA
- 11:20 - 11:40 ● **SS40.01** Update on Lambert-Eaton Myasthenic Syndrome  
 Jan Verschuuren, Netherlands
- 11:40 - 12:00 ● **SS40.02** Autoreactome of Thymoma-Associated Myasthenia Gravis  
 Linda Kusner, USA
- 12:00 - 12:20 ● **SS40.03** Thymoma-Associated Non-MG Syndromes  
 Gil Wolfe, USA

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- 11:00 - 12:30**    ● **SS41 ICNMD & iMyoS Joint Session - Idiopathic Inflammatory Myopathies/Myositis: Optimal Diagnosis and Treatment**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Jens Schmidt, Germany
- 11:20 - 11:40    ● **SS41.01** Diagnostic Strategies for an Optimal Diagnosis of IIM Patients and Mimics  
 Joost Raaphorst, Netherlands
- 12:00 - 12:20    ● **SS41.02** Treatment Options in Newly Diagnosed Patients and Refractory Patients  
 Julie Paik, USA
- 12:20 - 12:40    ● **SS41.03** New IBM Criteria to Enhance Diagnosis, Clinical Trials and Patient Outcomes  
 James Lilleker, United Kingdom
- 12:30 - 14:15**    Lunch Break  
📍 **Location:** Spadolini - Attic
- 13:00 - 14:00**    ● **Industry-Supported Symposia**  
📍 **Location:** Spadolini - Attic
- 14:15 - 15:45**    ● **SS42 Translational Research Updates in CMS**  
📍 **Location:** Spadolini Attic - Hall 5  
★ **Chair(s):** Yin Dong, United Kingdom
- 14:35 - 14:55    ● **SS42.01** New Biomarkers in Congenital Myasthenic Syndromes  
 Andreas Roos, Germany
- 14:55 - 15:15    ● **SS42.02** New Molecular Mechanisms  
 Yin Dong, United Kingdom
- 15:15 - 15:35    ● **SS42.03** Small Molecule Mediated Therapies  
 Sally Spendiff, Canada
- 14:15 - 15:45**    ● **SS43 Advances in Understanding Disease Mechanisms in MG**  
📍 **Location:** Spadolini Attic - Hall 2  
★ **Chair(s):** Patricia Sikorski, USA
- 14:35 - 14:55    ● **SS43.01** Age-Related B Cells  
 Patricia Sikorski, USA
- 14:55 - 15:15    ● **SS43.02** CASPR2 and LGI1 Antibody-Mediated Diseases in MG Patients  
 Gregorio Spagni, Italy
- 15:15 - 15:35    ● **SS43.03** Subunit-Specific Immunodominance in Clinically Distinct Populations With AChR+ Myasthenia Gravis  
 Kfir Oved, Israel
- 14:15 - 15:45**    ● **SS44 Painful and Small Fiber Neuropathies**  
📍 **Location:** Spadolini Attic - Hall 3  
★ **Chair(s):** Margheria Marchi, Italy
- 14:35 - 14:55    ● **SS44.01** Small Fiber Neuropathies  
 Devigili Grazia, Italy
- 14:55 - 15:15    ● **SS44.02** Pain Genetics  
 Margheria Marchi, Italy
- 15:15 - 15:35    ● **SS44.03** Treatment of Neuropathic Pain  
 Andrea Truini, Italy
- 14:15 - 15:45**    ● **SS45 The Role of Patients and Patient Organizations**  
📍 **Location:** Spadolini Attic - Hall 4  
★ **Chair(s):** Senda Ajroud Driss, USA
- 14:20 - 14:50    ● **SS45.02** North America  
 Senda Ajroud Driss, USA
- 14:55 - 15:25    ● **SS45.03** ALS/FTD Journal New Editor's Perspective  
 Matthew B. Harms, USA

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- 14:15 - 15:45** ● **SS46 Ageing and Senescence**  
📍 **Location:** Spadolini Attic - Hall 1  
★ **Chair(s):** Wolfgang Grisold, Austria
- 14:35 - 14:55 ● **SS46.01** Cellular Senescence in Tissue Repair and Dysfunction  
Mikolaj Ogrodnik, Austria
- 14:55 - 15:15 ● **SS46.02** Ageing of peripheral nerves  
Claudia Sommer, Germany
- 15:15 - 15:35 ● **SS46.03** Molecular insights of ageing sarcopenia  
Marco Sandri, Italy
- 15:45 - 16:15** ● Closing Ceremony  
📍 **Location:** Cavaniglia Pavilion

## Tuesday 7 July | 08:00-10:00

### **TC01: Teaching Course - Pain in NMD**

**Description:** Pain is a common and clinically relevant manifestation of several neuromuscular disorders, contributing to functional impairment and diminished quality of life. Beyond motor weakness and disability, pain may result from complex and multifactorial mechanisms, with major differences in prevalence and severity across specific disorders and disease stages. In some conditions, pain is a major feature, requiring an improved recognition of its causes and characteristics. This teaching course aims to provide an overview of the assessment and management of pain in neuropathies and myopathies, highlighting the importance of a multidimensional approach to optimize individualized strategies.

Chair	Introductions	Tiziana Mongini	Italy
Speaker	Painful neuropathies	Grazia Devigili	Italy
Speaker	Myalgia in myopathies	Tiziana Mongini	Italy
Speaker	Fibromyalgia	Sara Lazo	El Salvador

### **TC02: Teaching Course - Practical Approach to Myology Phenotypes**

**Description:** This course will present differential diagnosis and practical approach to three conditions that physicians encounter in myology/neuromuscular clinics. Dysphagia is a relatively common complaint in various disorders and the participants will be presented with the various diagnostic modalities as well as potential management. Asymptomatic hyper CK'emia is a common situation in clinics and recently guidelines about the approach to it were published and will be presented. Winging of scapula (scapula alata) can be a manifestation of various neuromuscular conditions and may assist in the diagnosis of certain conditions.

Chair	Introductions	Marianne de Visser	Netherland
Speaker	Dysphagia	Zohar Argov	Israel
Speaker	Asymptomatic hyper CK'emia	Antonio Toscano	Italy
Speaker	Scapula Alata (winging of scapula)	Marianne de Visser	Netherland

### **TC03: Teaching Course - PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 1**

**Description:** Rare neuropathies represent an important diagnostic and therapeutic challenge. This joint PNS–ICNMD teaching course will provide an overview of the clinical approach to several rare neuropathies, including CIDP and nodo-paranodopathies, acute polyradiculoneuropathies such as Guillain–Barré syndrome, inherited neuropathies including Charcot–Marie–Tooth disease, amyloid neuropathy, small fibre neuropathy and vasculitic neuropathies. Faculty will discuss key clinical features, diagnostic strategies and current treatment approaches, emphasizing practical algorithms useful in daily clinical practice. The course aims to improve recognition of rare neuropathies and guide clinicians in selecting appropriate diagnostic tests and disease-specific therapies.

Chair	Introductions	Eduardo Nobile Orazio	Italy
Speaker	CIDP and autoimmune nodopathies	Marcus Vinicius Pinto	USA
Speaker	GBS - acute polyneuropathies	Eduardo Nobile Orazio	Italy
Speaker	CMT and related neuropathies	Chiara Pisciotta	Italy

**Tuesday 7 July | 08:00-10:00**

🔗 **TC04: Teaching Course - The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 1**

**Description:** Infantile hypotonia (or the “Floppy Infant Syndrome”) is one of the most common presentations in Paediatric Neurology and has a very wide differential diagnosis, including neuromuscular, neurological and other early-onset genetic and non-genetic disorders. This half day course will cover common causes of the Floppy Infant Syndrome and outline a state-of-the-art diagnostic approach in the age of next generation sequencing. The congenital myasthenic syndromes and the congenital myopathies, two common but genetically diverse groups of early-onset neuromuscular disorders, are amongst the most common causes and will be outlined in detail. Clinical (including muscle imaging and neurophysiology), histopathological and genetic aspects of these conditions will be covered, complemented by an overview of current therapy developments. Illustrative scenarios will highlight practical applications in a clinical setting to consolidate taught knowledge.

Chair	Introductions	Heinz Jungbluth	UK
Speaker	The floppy infant revisited – from bedside to genome	Gianpaolo Cicala	Italy
Speaker	The neuromuscular assessment of the floppy infant	Jennie Sheehan	UK
Speaker	Congenital myasthenic syndromes	Sithara Ramdas	UK

🔗 **TC05: Teaching Course - Hands On: Clinical Neurophysiology**

**Description:** This demonstration and hands-on workshop include a discussion of the rationale and role of electrodiagnostic tools in the evaluation of patients presenting with weakness due to neuromuscular disorders. Participants will observe demonstrations and have the opportunity to practice frequently used techniques for the evaluation of the weak patient: Single Fiber EMG (SFEMG), routine needle examination, and repetitive stimulation of proximal motor nerves.

Chair	Introductions	William Litchy	USA
Speaker	SFEMG: the use in evaluation of Neuromuscular disorders	Anna Rostedt Punga	Sweden
Speaker	Standard Needle Examination	Rocco Ligouri	Italy
Speaker	Nerve Conduction Studies of Proximal Nerves (RNS)	William Litchy	USA

**Tuesday 7 July | 10:30-12:30**

**TC06: Teaching Course: Emergencies in Myopathies**

**Description:** This course will discuss three emergency conditions that need acute involvement of the neuromuscular physician. Rhabdomyolysis can be found in various inherited myopathies as well as in subjects with physiological over activity. The general approach to diagnosis and acute management as well as follow up will be discussed. Inflammatory and infectious myositis can manifest acutely. The participants will be presented with approach to rapid diagnostic as well as therapy under such circumstances. Modes of respiratory support (acute and chronic) will be discussed in the last talk.

Chair	Introductions	John Vissing	Denmark
Speaker	Rhabdomyolysis	John Vissing	Denmark
Speaker	Acute inflammatory/infective myopathy	Mazen Dimachkie	USA
Speaker	Rhabdomyolysis	John Vissing	Denmark

**TC07: Teaching Course: Genetics in Neuromuscular Disorders**

**Description:** This course intends to deliver advanced knowledge of the complicated genetic abnormalities in the NMD field. The various gene mutations associated with ALS will be presented and the importance of identification of mutations to the management and future therapy of this devastating condition. The new findings of genetic defects in oculo-pharyngo- distal myopathy (OPDM), showing that the same expansion mutation in several different genes result in OPDM and the possible disease mechanism will be presented. The complicated post transcriptional modification effect of the genetic defect in facio-scapulo-humeral dystrophy will be described.

Chair	Introductions	Ichizo Nishino	Japan
Speaker	The practicalities of motor neuron disease genetics	Vivian Drory	Israel
Speaker	OPDM- a model for unusual expansion disorders	Ichizo Nishino	Japan
Speaker	Translational changes- the FSHD complicated genetics	Sabrina Sacconi	France

**TC08: Teaching Course: PNS/ICNMD JOINT TC - How to Diagnose and Treat a Patient with a Rare Neuropathy PART 2**

**Description:** Rare neuropathies represent an important diagnostic and therapeutic challenge. This joint PNS–ICNMD teaching course will provide an overview of the clinical approach to several rare neuropathies, including CIDP and nodo-paranodopathies, acute polyradiculoneuropathies such as Guillain–Barré syndrome, inherited neuropathies including Charcot–Marie–Tooth disease, amyloid neuropathy, small fibre neuropathy and vasculitic neuropathies. Faculty will discuss key clinical features, diagnostic strategies and current treatment approaches, emphasizing practical algorithms useful in daily clinical practice. The course aims to improve recognition of rare neuropathies and guide clinicians in selecting appropriate diagnostic tests and disease-specific therapies.

Chair	Introductions	Katrin Hahn	Germany
Speaker	Amyloid neuropathy	Katrin Hahn	Germany
Speaker	Small fibre neuropathy	Grazia Devigili	Italy
Speaker	Vasculitic neuropathies	Judith Spies	Australia

**Tuesday 7 July | 10:30-12:30**

📍 **TC09: Teaching Course: The Floppy Infant - Myasthenic Disorders and Congenital Myopathies PART 2**

**Description:** Infantile hypotonia (or the “Floppy Infant Syndrome”) is one of the most common presentations in Paediatric Neurology and has a very wide differential diagnosis, including neuromuscular, neurological and other early-onset genetic and non-genetic disorders. This half day course will cover common causes of the Floppy Infant Syndrome and outline a state-of-the-art diagnostic approach in the age of next generation sequencing. The congenital myasthenic syndromes and the congenital myopathies, two common but genetically diverse groups of early-onset neuromuscular disorders, are amongst the most common causes and will be outlined in detail. Clinical (including muscle imaging and neurophysiology), histopathological and genetic aspects of these conditions will be covered, complemented by an overview of current therapy developments. Illustrative scenarios will highlight practical applications in a clinical setting to consolidate taught knowledge.


Chair	Introductions	Heinz Jungbluth	UK
Speaker	Acquired mimics of CMS – TNG, FARIS and JMG	Ulrike Schara-Schmidt	Germany
Speaker	Congenital myopathies and related disorders – clinical features	Heinz Jungbluth	UK
Speaker	The congenital myopathies – histopathological features	Edoardo Malfatti	France
Speaker	Therapeutic developments and clinical trials in the congenital myopathies	Giovanni Baranello	UK

📍 **TC10: Teaching Course: Myasthenia Gravis**

**Description:** This is a more basic course about myasthenia gravis (MG). The various antibodies that are found in MG will be correlated with the resulting clinical syndrome and its therapy. The current immunosuppressive and immunomodulation therapies in MG will be overviewed. Pitfall in diagnosis of MG as well as mimics of immune mediated MG will be reviewed.


Chair	Introductions	Pushpa Narrayanaswami	USA
Speaker	Antibodies in Myasthenia	Shahar Shelly	Israel
Speaker	Immune therapy of Myasthenia Gravis	Pushpa Narrayanaswami	USA
Speaker	Pitfalls in the Diagnosis	Amelia Evoli	Italy

**Tuesday 7 July | 13:30-15:30**

 **TC11: Teaching Course: How to examine a patient with neuromuscular disorders: A Practical Guide**

**Description:** Accurate clinical examination remains the cornerstone of diagnosis in neuromuscular medicine. This teaching course will provide a practical approach to the neurological examination of patients with suspected neuromuscular disorders. Experts will review key clinical signs and examination strategies to differentiate between myopathies, polyneuropathies and disorders of the neuromuscular junction. Through practical guidance and illustrative examples, participants will learn how bedside examination can guide diagnostic reasoning and the selection of appropriate investigations. The course aims to strengthen fundamental clinical skills that remain essential even in the era of advanced genetic and molecular diagnostics.

Chair	Introductions	Davide Pareyson	Italy
Speaker	How to examine a patient with a myopathy	Pascal Laforet	France
Speaker	How to examine a patient with a polyneuropathy	Davide Pareyson	Italy
Speaker	How to examine a patient with a neuromuscular junction disorder	Lorenzo Maggi	Italy

 **TC12: Teaching Course: What a Biopsy can tell you in the Genomic Era**

**Description:** Despite major advances in genetic diagnostics, tissue biopsy remains an important tool in the evaluation of neuromuscular disorders. This teaching course will discuss the current role of muscle, nerve and skin biopsy in the era of genomic medicine. Faculty will illustrate how pathological findings can complement genetic testing, clarify uncertain diagnoses and provide insight into disease mechanisms. Particular emphasis will be placed on practical indications, interpretation of pathological findings and integration of biopsy results with clinical and molecular data.

Chair	Introductions	P. James B. Dyck	USA
Speaker	Muscle biopsy	Teresinha Evangelista	France
Speaker	Nerve biopsy	P. James B. Dyck	USA
Speaker	Skin biopsy	Vincenzo Donadio	Italy

 **TC13: Teaching Course: Amyotrophic Lateral Sclerosis in 2026: Clinical Practice, Biomarkers and Precision Medicine**

Chair	Introductions	Andrea Calvo	Italy
Speaker	Practical diagnosis and clinical management of ALS	Markus Weber	Switzerland
Speaker	Biomarker in ALS: clinical utility and implementation	Thomas Meyer	Germany
Speaker	Emerging therapies and the future of ALS trials	Andrea Calvo	Italy

**Tuesday 7 July | 13:30-15:30**

🔑 **TC14: Teaching Course: Inherited Myopathies - Less Common Disorders**

Description: Dominant limb girdle dystrophies are less common compared with the recessive forms but their recognition has several important aspects. Distal myopathies is an entity that is missed initially by non myology experts and the approach to this syndrome will be discussed. Late onset mitochondrial disorders are under recognized as causes of fatigue and weakness and those will be presented.

Chair	Introductions	Anthony Amato	USA
Speaker	Dominant LGMD	Anthony Amato	USA
Speaker	Distal Myopathies	Marco Savarese	Finland
Speaker	Late Onset Mitochondrial myopathies	Gabriele Siciliano	Italy

🔑 **TC15: Teaching Course - Hands On: Ultrasound**

Description: Dominant limb girdle dystrophies are less common compared with the recessive forms but their recognition has several important aspects. Distal myopathies is an entity that is missed initially by non myology experts and the approach to this syndrome will be discussed. Late onset mitochondrial disorders are under recognized as causes of fatigue and weakness and those will be presented.

Chair	Introductions	Stefan Meng	Austria
Speaker	Cranial nerves and Trunk	Stefan Meng	Austria
Speaker	Lower Extremities	Jan-Hendrik Stahl	Germany

# Joint Sessions

ICNMD is excited to be partnering with various organizations and have developed Joint Sessions highlighting important topics to be presented throughout the ICNMD Congress.

## Wednesday 8 July | 11:15 - 12:45

### ● SS05: ICNMD & World Muscle Society Joint Session

**Description:** The session will focus on inequalities in access to treatment, their consequences, and the solutions that have been proposed. We will examine the situation in Tunisia, a country with a strong healthcare system but limited resources for innovative therapies. Tunisia is the last country in North Africa without access to disease-modifying therapies for spinal Muscular Atrophy. We will also examine the situation in Ukraine, where access to healthcare and treatments has been disrupted in some regions by the war. Finally, we will explore the consequences of inequitable access to treatment from the perspective of a high-income country. Speakers will focus not only on the specific challenges faced by their countries, but also on the solutions that have been implemented or could be introduced to address these issues.

5 min	Chair	Introductions	Laurent Servais	Italy
20 min	Speaker	When drugs are available... but not for everyone	Emna Farhat	Tunisia
20 min	Speaker	When war disrupts patients care	Nataliia Samonenko	Ukraine
30 min	Speaker	Crowdfunding, lottery, relocation... Treatment access inequity from a global north perspective	Laurent Servais	Italy
25 min	Q&A			

# Joint Sessions

Wednesday 8 July | 14:30 - 16:00

● **SS17: ICNMD, AIM & ASNP Joint Session- Update in Neuromuscular Disorders: The Italian Contribution**

**Description:** This joint session of the ICNMD and the German Nerve Club brings together leading international experts in peripheral nerve disorders to discuss recent advances in nerve surgery, diagnostic techniques, and rehabilitation strategies. The program covers the full spectrum of modern peripheral nerve care, from rare peripheral nerve tumors and innovative surgical approaches to the latest developments in nerve imaging, ultrasound diagnostics, and functional recovery.

Presentations will explore the role of high-resolution nerve ultrasound in the diagnosis and management of conditions such as neuralgic amyotrophy, outcomes following peripheral nerve repair, and the surgical treatment of complex neuropathies. Further topics include thoracic outlet syndrome, peripheral nerve stimulation for motor recovery and neuropathic pain, and reconstructive techniques aimed at improving long-term patient outcomes.

By integrating perspectives from neurology, neurosurgery, rehabilitation medicine, and translational research, this session highlights multidisciplinary approaches to optimizing diagnosis, treatment, and recovery in patients with peripheral nerve disorders. Attendees will gain insights into emerging technologies, evidence-based surgical interventions, and future directions in peripheral nerve care.

5 min	Chair	Introductions	Vincenzo Nigro	Italy
20 min	Speaker	Diagnostic approach to peripheral neuropathy	Fiore Manganelli	Italy
20 min	Speaker	Update on Genetic Myasthenic syndromes	Lorenzo Maggi	Italy
20 min	Speaker	Axial myopathies: clinical phenotypes and differential diagnosis	Tiziana Mongini	Italy
25 min	Q&A			

# Joint Sessions

Thursday 9 July | 11:15 - 12:45

● **SS10: ICNMD & Peripheral Nerve Society Joint Session**

**Description:** This special joint symposium brings together the International Congress on Neuromuscular Diseases (ICNMD) and the Peripheral Nerve Society (PNS)—the world’s largest medical society dedicated to the study of peripheral nerve disorders. In this collaborative session, distinguished members of the PNS will deliver expert updates and comprehensive educational content focusing on the latest advancements in autoimmune, genetic, and toxic neuropathies.

5 min	Chair	Introductions	Marcus Pinto	USA
20 min	Speaker	Painful Neuropathies: New insights/New treatment targets	Claudia Somner	Germany
20 min	Speaker	Chemotherapy induced peripheral neuropathy	Paola Alberti	Italy
20 min	Speaker	Gene therapy for inherited neuropathies – how to optimally deliver to peripheral nerves	Kleopas Kleopa	Cyprus
30 min	Speaker	Antibodies in autoimmune neuropathies	Kavita Grover	USA
5 min	Q&A			

# Joint Sessions

Thursday 9 July | 14:30 - 16:00

● **SS22: ICNMD & German Nerve Club Joint Session - Nerve surgery, diagnostics and rehabilitation**

**Description:** This joint session of the ICNMD and the German Nerve Club brings together leading international experts in peripheral nerve disorders to discuss recent advances in nerve surgery, diagnostic techniques, and rehabilitation strategies. The program covers the full spectrum of modern peripheral nerve care, from rare peripheral nerve tumors and innovative surgical approaches to the latest developments in nerve imaging, ultrasound diagnostics, and functional recovery.

Presentations will explore the role of high-resolution nerve ultrasound in the diagnosis and management of conditions such as neuralgic amyotrophy, outcomes following peripheral nerve repair, and the surgical treatment of complex neuropathies. Further topics include thoracic outlet syndrome, peripheral nerve stimulation for motor recovery and neuropathic pain, and reconstructive techniques aimed at improving long-term patient outcomes.

By integrating perspectives from neurology, neurosurgery, rehabilitation medicine, and translational research, this session highlights multidisciplinary approaches to optimizing diagnosis, treatment, and recovery in patients with peripheral nerve disorders. Attendees will gain insights into emerging technologies, evidence-based surgical interventions, and future directions in peripheral nerve care.

5 min	Chair	Introductions	Robert Schmidhammer	USA
30 min	Speaker	Nerve Surgery The power of one: advances with rare peripheral nerve tumors	Robert Spinner	USA
7 min	Speaker	Size does not matter - Ultrasound Parameters and Patient-Reported Outcomes after Median and Ulnar Nerve Repair	Henrik Lauer	Germany
7 min	Speaker	Nerve ultrasound and its role in detecting neuralgic amyotrophy	Alexander Grimm	Germany
7 min	Speaker	Between neuralgic amyotrophy and torsional neuropathy – the role of surgery	Nora Dengler	Germany
7 min	Speaker	The Thoracic Outlet Syndrome	Franz Lassner	Germany
7 min	Speaker	The potential for gain of function under direct nerve stimulation based on the long-term results of peroneal nerve stimulation	K.D Martin	Germany
7 min	Speaker	Peripheral Nerve Stimulation as a Therapeutic Option for Severe Neuropathic Pain	Walter Demmel	Germany
7 min	Speaker	Addressing Peripheral Neuropathic Pain from Nerve Scarring with Perforator-Based Gliding Tissue Flaps: Two Decades of Clinical Practice	Savas Tsolakidis	Germany

# Joint Sessions

## Friday 10 July | 11:15 - 12:45

### ● SS29: ICNMD & World Federation of Neurology Joint Session

**Description:** The World federation of Neurology promotes and supports research and treatment of NM disease. Following the congress tradition, one session is cohosted with ICNMD to make aware and promote important global topics. This session will include an analysis of neuromuscular symptoms of Covid, the globally important topic of leprosy and NM emergencies in Asia

5 min	Chair	Introductions		
20 min	Speaker	Neuromuscular late effects of Covid	Maurizio Leone	Italy
20 min	Speaker	Leprosy	Pedro Tomaselli	Brazil
20 min	Speaker	India: Emergencies in NM diseases	Sarath Menon	India
25 min	Q&A			

## Friday 10 July | 14:30 - 16:00

### ● SS34: ICNMD & The Autonomic Disorder Group of the WFN Joint Session

**Description:** Symptoms attributed to autonomic dysfunction are common, and underlying causes are often misdiagnosed as primary autonomic failure. Cardiovascular, gastrointestinal, rheumatologic, immune, and functional conditions (e.g., PPPD, ME/CFS, pelvic floor disorders) can mimic these presentations. Some patients experience a cluster of symptoms—orthostatic intolerance, fatigue, pain, cognitive issues, and dysregulation—that, while individually non-dangerous, can be highly disabling. Managing symptoms in isolation is ineffective; evidence supports a coordinated, multidisciplinary approach for better outcomes, though optimal strategies remain under discussion.

min	Chair	Introductions	Paola Sandroni	USA
20 min	Speaker	Autonomic testing	Valerie Iodice	UK
20 min	Speaker	Dysfunctional autonomic disorders	Paola Sandroni	USA
20 min	Speaker	Autonomic neuropathies	Jin-Woo Park	Korea
25 min	Q&A			

# Joint Sessions

Saturday 11 July | 11:15 - 12:45

## ● SS41: ICNMD & iMyoS Joint Session - Idiopathic inflammatory myopathies/Myositis: optimal diagnosis and treatment

**Description:** Inflammatory myopathies -myositis- include dermatomyositis (DM), anti-synthetase syndrome (ASyS), immune-mediated-necrotizing-myopathy (IMNM), overlap myositis and inclusion body myositis. Apart from muscle weakness, varying degrees of extramuscular involvement such as interstitial lung disease (ILD) can occur and even lead to death.

iMyoS is a global, interdisciplinary society for Rheumatologists, Neurologists, Neuropathologists and many other specialties that are involved in diagnosing and treating myositis. The society aims to harmonize the development of best international standards of care for myositis and organizes two yearly global conferences on myositis [GCOM]. The most recent GCOM meeting took place in Lisbon in March 2026.

During this joint iMyos-ICNMD session on myositis three topics will be discussed: diagnostic strategies for an optimal diagnosis of IIM patients and mimics, treatment options in newly diagnosed patients and refractory patients and the new IBM criteria to enhance diagnosis, clinical trials and patient outcomes.

5 min	Chair	Introductions	Jens Schmidt	Germany
20 min	Speaker	Diagnostic strategies for an optimal diagnosis of IIM patients and mimics	Joost Raaphorst	Netherlands
20 min	Speaker	Treatment options in newly diagnosed patients and refractory patients	Julie Paik	USA
20 min	Speaker	New IBM criteria to enhance diagnosis, clinical trials and patient outcomes	James Lilleker	UK
25 min	Q&A			

Saturday 11 July | 14:30 - 16:00

## ● SS46: Ageing and Senescence

**Description:** The Ageing of the neuromuscular system is gaining importance. Most commonly, Sarcopenia and the cancer associated cachexia are referenced. Increasingly the assessment of Sarcopenia in the elderly is aging importance and several associations, also as with cognitive decline have been noted. Also peripheral nerves age, which important not only for motor , but also sensory functions, in relation to coordination and balance. This session discusses several aspects on the ageing on the NM system.

5 min	Chair	Introductions	Wolfgang Grisold	Austria
20 min	Speaker	Senescence of the NM system	Mikolaj Ogrodnik	Austria
20 min	Speaker	Ageing of peripheral nerves	Claudia Sommer	Germany
20 min	Speaker	Molecular insights of ageing sarcopenia	Marco Sandri	Italy
25 min	Q&A			

● Muscle Disorders

● Motor Neuron Diseases

● Peripheral Neuropathies

● Neuromuscular Junction Disorders



### Benedikt Schoser

#### ● PL01.01 Redefining Muscle Disease Management: A Roadmap to 2030

Professor Schoser is a neurologist, neurophysiologist, neurointensivist, palliative medicine doctor, and muscle pathologist. He is a professor of Neurology at the Friedrich-Baur-Institute, Ludwig-Maximilians-University Munich. He became an EAN fellow in 2017 and serves on various panels, including the Educational, Rare Disease, and Scientific Muscle and Neuromuscular panels. He co-chaired the EAN/UEMS Educational Board. As a long-term member of the World Muscle Society, he organized its teaching course for 10 years. Since 2015, he

has chaired the European Pompe Consortium EPOC, and since 2024, the EuroDyMA scientific advisory board. He authored over 410 peer-reviewed publications, mainly on myotonic dystrophies and glycogen storage diseases, with a focus on multisystemic neuromuscular disorders, translational research, and therapy.



### Vincenzo Nigro

#### ● PL01.02 Gene Diagnostics in 2030

Full professor of Medical Genetics at the Department of "Precision Medicine" of the "University "Luigi Vanvitelli" of Naples, Italy" and Associate Investigator of the Telethon Institute of Genetics and Medicine (TIGEM). Born in Naples (Italy), graduated in Medicine. He published >350 articles in peer reviewed journals. Among the most significant results, the identification of the causes of many Mendelian disorders, especially of the neuromuscular diseases. He directs the Unit of Medical Genetics and is coordinator of the Telethon

Undiagnosed Program that provides discovery of new and unrecognized genetic disorders.



### Isabelle Richard

#### ● PL01.03 Towards effective low-dose gene therapy in severe muscular dystrophy models using a novel AI-designed AAV

Dr. Isabelle Richard, PhD, is a Research Director at the CNRS and leads the Muscular Dystrophies team within INSERM U951 at Genethon, France. As a human geneticist, she has participated in identifying disease causing genes and uncovering key molecular and cellular mechanisms involved in many forms of muscular dystrophies (MD), laying essential foundations for gene therapy strategies. Her team has demonstrated proof of concept for AAV-mediated gene

therapy across six forms of MD. She currently focuses on improving the safety and efficacy of these therapies by studying host-vector interactions and optimizing vector design using protein modeling and artificial intelligence. Her research has led to the launch of clinical trials for two forms of MD, with additional therapeutic candidates in development. To accelerate clinical translation, she co-founded the biotech company Atamy Therapeutics in 2021. She has published over 205 scientific publications.



### Daniela Menichella

#### ● PL02.01 Painful Neuropathies: New insights/New treatment targets

Dr. Daniela Maria Menichella received her M.D. and Ph.D. from the University of Milan and completed postdoctoral training at Wayne State University and Harvard Medical School. She completed her Neurology residency at Northwestern University, where she is currently Associate Professor of Neurology and Pharmacology. She directs the Peripheral Neuropathy Multidisciplinary Clinic and the Charcot-Marie-Tooth Association Center of Excellence and is actively involved in NIH NeuroNext. Dr. Menichella serves as Principal Investigator of the

Northwestern University Clinical Hub within the NIH-funded Early Phase Pain Investigation Clinical Network (EPPIC-Net). As PI of an NIH-funded laboratory, her research focuses on molecular and cellular mechanisms of neuropathic pain and axonal degeneration, particularly in painful diabetic neuropathy. Her work integrates advanced preclinical models with human tissue studies and precision-medicine approaches to identify biomarkers and advance targeted therapies for peripheral neuropathies.



### Andrea Cortese

#### ● PL02.02 Unraveling Neuromuscular Disorders Caused by Non-Coding Repeat Expansions

Andrea Cortese is an academic neurologist at the UCL Queen Square Institute of Neurology and the Carlo Besta Neurological Institute, University of Milan. His research focuses on the identification and modelling of novel causes of neuromuscular and neurogenetic diseases, with a particular emphasis on repeat expansion disorders. His main scientific contributions include the identification of the RFC1 repeat expansion causing CANVAS and sensory neuropathy, the

discovery of SORD mutations as a common cause of inherited neuropathy, and the identification of a CGG repeat expansion causing myopathy. More recently, his research has expanded to understanding the disease mechanisms leading to progressive neurodegeneration associated with RFC1 expansions.



### Pietro Doneddu

#### ● PL02.03 Advancing Care in CIDP: Exploring Emerging Treatments

Pietro Emiliano Doneddu, MD, is a neurologist, researcher, and Professor at Humanitas University (Hunimed), affiliated with Humanitas Clinical and Research Hospital in Milan, Italy. He trained at the University of Sassari and undertook further specialization in neuromuscular diseases and peripheral neuropathies at the Catholic University of the Sacred Heart, Rome, and King's College Hospital, London.

His research focuses on immune-mediated neuropathies, particularly CIDP and MMN, spanning clinical epidemiology, electrophysiology, biomarkers, and diagnostic criteria. He coordinates the Italian CIDP/MMN study group and collaborates in international research initiatives.

Dr. Doneddu serves on the boards of the Peripheral Nerve Society (PNS) and the Italian Peripheral Nerve Society (ASNP), and has authored numerous peer-reviewed publications aimed at improving diagnosis and treatment of peripheral nerve disorders.



### Pam Shaw

#### ● PL03.01 SOD1 pathology in SOD1 ALS and Sporadic ALS

Pamela Shaw is Professor of Neurology, University of Sheffield and Director of the Sheffield Institute for Translational Neuroscience (SITraN and the Sheffield Care and Research Centre for Motor Neuron Disorders. Her work investigates genetic, molecular and neurochemical mechanisms underlying ALS/MND; identifies new therapeutic targets and translates new neuroprotective and symptomatic treatment approaches into the clinic, including genetic therapy approaches. She has authored more than 630 publications (H-index 131). Her research is funded by NIHR, the UK

Medical Research Council, MND Association, MyName'5 Doddie Foundation, Fight MND and Department of Defense, EU and biotech/ pharmaceutical industry partners. She has received multiple awards for her work including: DBE for Services to Neuroscience; AAN Sheila Essey Award; International ALS/MND Alliance Forbes Norris Award; Royal College of Physicians Jean Hunter Award; FMedSci; Fellowship American Association for the Advancement of Science; ABN Medallist-2019; Queen's Anniversary Award-2020; British Neuroscience Association Outstanding Contribution Award-2022.



### Andrea Malaspina

#### ● PL03.02 Unravelling the Presymptomatic Phase of ALS: Clinical, Genetic, and Molecular Advances Toward Early Detection

Professor Andrea Malaspina is a Consultant Neurologist and Professor of Neurology at the UCL Queen Square Institute of Neurology, one of the world's leading centres for neurological care and research. With over 25 years of clinical experience in general neurology, he specialises in neuromuscular disorders and neurodegenerative diseases, including motor neurone disease (MND/ALS). Professor Malaspina is Director of the BRC UCL Queen Square MND Centre, where he leads

a multidisciplinary programme of clinical care and research dedicated to improving outcomes for people living with motor neurone disease and related neuromuscular conditions. His research focuses on biomarkers, neuroinflammation and mechanisms of neurodegeneration, helping to develop new diagnostic tools and therapeutic strategies. He leads major clinical trial platforms, UK and world-wide translational research initiatives aimed at accelerating treatment development for neurodegenerative diseases.



### Thanuja Dharmadasa

#### ● PL03.03 The Global Fight to End ALS

Dr Thanuja Dharmadasa [MBBS Hons 1, FRACP, PhD] is a Neurologist & Clinical Neurophysiologist and The Deputy Head of Neurodegeneration and Immunology Research (The Florey Institute), Australia. She was awarded the Jim Lance Young Investigator's Award (2017) and Peter Bancroft Prize (2019) for her PhD thesis (University of Sydney) and received a prestigious NHMRC Early Career Fellowship to continue this work at The University of Oxford (UK). She now runs a Motor & Nerve Disorders Clinical Research Service at the Royal

Melbourne Hospital, Victoria [Australia]. As a clinician-scientist, her clinical research explores clinical and advanced neurophysiological and neuroimaging techniques in patients to understand disease mechanisms, with a particular focus in motor neuron disease (MND). Having worked as an MND clinician at several MND care centres both nationally and internationally, she also has a keen interest on strategies surrounding the implementation and optimisation of best care for patients living with the disease. diagnosis and treatment of peripheral nerve disorders.

● Muscle Disorders

● Motor Neuron Diseases

● Peripheral Neuropathies

● Neuromuscular Junction Disorders



### Hanns Lochmuller

#### ● PL04.01 CMS : Update on Diagnosis and Treatment

Hanns is a neurologist and clinical academic specializing in genetic neuromuscular disorders and rare disease. He is a Senior Scientist at the Children's Hospital of Eastern Ontario (CHEO) Research Institute and the Ottawa Hospital Research Institute. He also holds appointments as Professor of Neurology in the University of Ottawa Faculty of Medicine and the Department of Medicine, Division of Neurology at The Ottawa Hospital. He is the co-director of the University of Ottawa Centre for Neuromuscular Disease and is

affiliated with the University's Brain and Mind Research Institute and Department of Cellular and Molecular Medicine.



### Carolina Barnett Tapia

#### ● PL04.02 Ready for Change? What the New Guidelines/ recommendations Say

Dr. Carolina Barnett-Tapia is Associate Professor in the Department of Medicine, Division of Neurology and the Institute of Health Policy, Management and Evaluation, University of Toronto. She is a clinician-scientist at the University Health Network, Toronto, Ontario. Dr. Barnett-Tapia's main research interest is patient-centered outcomes and patient preference elicitation in patients with Neuromuscular Disorders and Neurofibromatosis. She developed the Myasthenia

Gravis Impairment Index (MGII) a novel, patient-centred measure of disease severity. She has received funding from the US Department of Defense and Canadian Cancer Society, MGNet and Muscular Dystrophy Canada. She is member of the scientific council of the Myasthenia Gravis Foundation of America, of the editorial board of Neurology (Neuromuscular section), of the PRO section of the REiNS consortium, and of the scientific leadership group for the international MG base registry.



### Yves Allenbach

#### ● PL04.03 Immune Checkpoint Inhibitor-Related MG

Yves Allenbach is a Professor of Clinical Immunology and Internal Medicine at Sorbonne University and expert in autoimmune myopathies. He obtained his MD and PhD in immunology from Sorbonne University, followed by advanced clinical training in internal medicine and immunology, and a postdoctoral fellowship at Humboldt University of Berlin focusing on immunopathology.

His research focuses on inflammatory myopathies and immune-related adverse events, particularly immune checkpoint inhibitor-

related myotoxicity. He has led multiple national and international projects, including prospective multicenter cohorts and randomized trials, and has secured several competitive grants as principal investigator. His work integrates clinical phenotyping, biomarker discovery, and translational immunology.

He collaborates extensively with international partners and has organized consensus initiatives in myositis. He is an active member of several scientific societies and serves on national and international scientific committees.

# General Information

## Access/Security

Name Badges will be provided to all delegates and participants and can be picked up at the ICNMD 2026 Registration Desk. Please wear and ensure your name badge is visible at all times as it is your admission pass to all Plenary and Concurrent sessions, the Exhibit Hall and Social Events. Delegates will not be able to access the congress venue and meeting space without their badge. There is a €60.00 reprint fee for any lost or misplaced badges.

## Official Language

The official language of the ICNMD 2026 Congress is English. All sessions will be conducted in English.

## Lost Property

Please report any lost or unattended items immediately to the ICNMD 2026 Registration Desk. Should you lose anything while at the congress, please enquire at the Registration Desk where any recovered lost property will be held. At the end of the conference, all unclaimed lost and found items will be given to The Fortezza da Basso Congress Centre.

## Photographer & Videographer

An official photographer & videographer will be present during the Congress. By registering for the ICNMD 2026 Congress, you agree your image may be taken. Imagery taken may be used for marketing purposes for future ICNMD Congresses and Events.

## Registration Desk

📍 **Location:** Cavaniglia

<b>Monday 6 July</b>	14:00 - 18:00
<b>Tuesday 7 July</b>	07:00 - 19:30
<b>Wednesday 8 July</b>	07:30 - 17:30
<b>Thursday 9 July</b>	07:00 - 17:30
<b>Friday 10 July</b>	07:00 - 18:00
<b>*Luggage check available</b>	
<b>Saturday 11 July</b>	08:00 - 14:30
<b>*Luggage check available</b>	

## Refreshment Breaks

### Wednesday 8 July – Friday 10 July

📍 **Location:** Spadolini – Ground Floor (Exhibit & Poster Hall)

Morning Break	10:30 - 11:15
Lunch Break	12:45 - 14:30
Afternoon Break	16:00 - 16:45

### Saturday 11 July

📍 **Location:** Spadolini – Attic

Morning Break	10:30 - 11:00
Lunch Break	12:30 - 14:15

In addition to these Refreshment and Lunch Breaks, water stations will be available in the Exhibit Hall during Exhibit Hours.

### Water Stations Supported by:



## Speaker Ready Room Hours

📍 **Location:** Cavaniglia

<b>Monday 6 July</b>	14:00 - 18:00
<b>Tuesday 7 July</b>	07:00 - 18:00
<b>Wednesday 8 July</b>	07:30 - 17:30
<b>Thursday 9 July</b>	07:00 - 17:30
<b>Friday 10 July</b>	07:00 - 18:00
<b>Saturday 11 July</b>	08:00 - 14:30

## WiFi

Network: ICNMD Password: icnmd2026

### WiFi Supported by:



## Disclaimer

All reasonable endeavors will be made to hold the ICNMD 2026 Congress and to present the program as scheduled under circumstances which assure the comfort and safety of the Congress Participants. However, ICNMD and their respective directors, officers, employees, representatives or agents, shall not be liable in any manner whatsoever to any person as a result of the cancellation of the Congress or any of the arrangements, programs or events connected therewith; nor shall any of the foregoing entities or persons be liable in any manner whatsoever for any loss, injury, damage or inconvenience which may be suffered by any person while travelling to or from, or during such person's presence in, Italy in connection with the Congress. Participants are advised to consider procuring their own insurance against any such occurrences.

# Switching therapy in late-onset Pompe disease (LOPD): Bridging the gap between data and what really matters to patients

## OUR EXPERT PANEL



Prof Antonio Toscano  
Professor of Neurology,  
Italy



Dr Nadine van der Beek  
Neurologist,  
the Netherlands



Prof Olimpia Musumeci  
Professor of Neurology,  
Italy



Dr Menekşe Öztürk  
Clinician Scientist in  
Neuromuscular Disorders,  
Germany

Our expert panel invites you to join them around the kitchen table as we explore switching therapy in LOPD, with a focus on how we can bridge the gap between the clinical data and what really matters to patients.

Whilst sharing long-term data, case studies and patient insights, our expert panel will be challenging you to consider:

- Are we missing what matters more to adults with LOPD?
- How can we apply learnings on long-term efficacy from the open-label extension data?
- In an era of multiple options, how do we approach switching therapy?

Join us for an immersive experience where we invite you to really think about what matters more—the coffee is brewing!

This Satellite Symposium is organised and sponsored by Amicus Therapeutics and is intended for registered healthcare professionals. Amicus Therapeutics' products will be discussed at this meeting.

This presentation was approved by the Program Committee as an independent activity held in conjunction with the 19th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2026.



Advancing research & Supporting patients to plan for tomorrow

**Johnson & Johnson**  
The Health & Care Company

# Social Events



## Opening Ceremony

**Tuesday 7 July**

17:15 - 18:00

Cavaniglia Pavilion

Access: All registered delegates

Description: The Opening Ceremony will feature a local performance and Welcome Remarks. Following the Opening Ceremony, guests are invited to the Welcome Reception to have the opportunity to mix and mingle with colleagues and friends.



## Welcome Reception

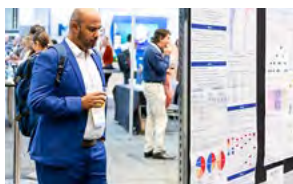
**Tuesday 7 July**

18:00 - 19:30

Spadolini – Ground Floor (Exhibit & Poster Hall)

Access: Registered Delegates – Additional tickets can be purchased at €85.00 each.

Description: Join your fellow peers on the first evening for a drink to kick off the 19th ICNMD!



## Poster Tours

**Wednesday 8 July - Friday 10 July**

10:30 - 11:15 (During Morning Break)

Spadolini – Ground Floor (Exhibit & Poster Hall)

Access: All registered delegates

Description: During the morning coffee break, be sure to come to the Exhibit Hall and meet with our Poster Presenters.



Wednesday 8 July	Thursday 9 July	Friday 10 July
<b>Poster Tour 1:</b> Muscle and Neuromuscular Junction	<b>Poster Tour 2:</b> Motor Neuron	<b>Poster Tour 3:</b> Peripheral Neuropathy



Scan for Directions

## Networking Dinner

**Friday 10 July**

19:30 - 22:30

Villa Vittoria (V.le Filippo Strozzi, 2, 50123 Presso FI, Italy)

Access: Pre-Registration & Ticket required – €125.00

Description: For the final evening, join other delegates at Villa Vittoria, steps away from the main congress centre for a fun casual evening to celebrate yet another fantastic and engaging ICNMD Congress.

## Closing Ceremony

**Saturday 11 July**

15:45 - 16:15

Cavaniglia Pavilion

Access: All registered delegates

Description: The Closing Ceremony will celebrate the success of the Congress and mark the inauguration of the incoming ICNMD Congress President. Closing remarks followed by a feature on the next host city will cap off the congress and set the stage for the 20th ICNMD Congress in 2027.





# DYNE THERAPEUTICS IS ON A MISSION TO DELIVER FUNCTIONAL IMPROVEMENT FOR PEOPLE LIVING WITH GENETICALLY DRIVEN NEUROMUSCULAR DISEASES

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We are developing therapeutics that target muscle and the central nervous system (CNS) to address the root cause of disease across multiple organ systems. Dyne is advancing clinical programs for Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1), as well as preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease.

We are committed to listening to and learning from the communities that we aim to serve, including individuals and families living with these diseases. Their stories inspire us and help us to deliver what patients value most: functional improvement.

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**WANT TO LEARN MORE ABOUT  
DYNE AND OUR SCIENCE?  
SIGN UP HERE AND STAY IN TOUCH**



If you would like to receive updates from Dyne Therapeutics in the future, please take a moment to sign up via this QR code, to provide your consent.

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**VISIT BOOTH #101 TO LEARN MORE  
ABOUT US AND TO EXPLORE OUR  
EDUCATIONAL DIGITAL CONTENT  
IN DMD AND DM1:**

**Understanding how  
DM1 affects the brain**


**What is a  
spliceopathy?**

**One disease,  
multiple symptoms**

**Think you  
know DM1?**

**Understanding  
dystrophin in DMD**

**Motor function  
assessments in DMD**



**PNS**  
PERIPHERAL NERVE  
SOCIETY

**CHICAGO**  
**8-11 MAY, 2027**  
**SAVE THE DATE!**

**2027 ANNUAL MEETING**  
**Hilton Chicago • Chicago, Illinois, USA**

Wednesday 8 July | 16:05 - 16:20



## ST01 – Satellos Bioscience

**From Regeneration to Function: Advancing Muscle Repair with SAT-3247**

**Presenter:** Wildon Farwell, MD, MPH, Satellos Chief Medical Officer

**Description:** Satellos Bioscience operates on a simple but powerful premise: in muscular dystrophies the problem isn't only muscle damage, but also the lack of effective muscle regeneration and repair. In this showcase theater, Satellos Chief Medical Officer Dr. Wildon Farwell will discuss 1) a critical but underappreciated role of dystrophin in muscle function and degeneration, 2) our small-molecule (SAT-3247) approach to harnessing a separate, dystrophin-independent mechanism (AAK1) within satellite cells to enhance muscle regeneration, 3) our emerging data in people living with Duchenne muscular dystrophy (DMD), and 4) our clinical development program in DMD and potential utility of SAT-3247 in other muscle disorders.

Wednesday 8 July | 16:25 - 16:40



## ST02 – BridgeBio

**Interim analysis from ongoing Phase 3 FORTIFY study of BBP-418 in patients with limb girdle muscular dystrophy 2I/R9 meets efficacy endpoints**

**Presenter:** Douglas M. Sproute, MD MSc, Chief Medical Officer, BridgeBio

**Description:** FORTIFY (NCT05775848) is a randomized, double-blind, placebo-controlled Phase 3 trial. This prespecified 1-year interim analysis evaluated biochemical, functional, and safety endpoints in patients with LGMD2I/R9. The primary endpoint was the change from baseline in glycosylated aDG at 3 months; key secondary endpoints included serum creatine kinase (CK), 100-meter timed test (100MTT) velocity, and forced vital capacity (FVC). Patient reported outcomes were also assessed.

Thursday 9 July | 16:05 - 16:20



## ST03 – Lupin Neurosciences Specialty & ICNMD 2026 Scientific Theatre

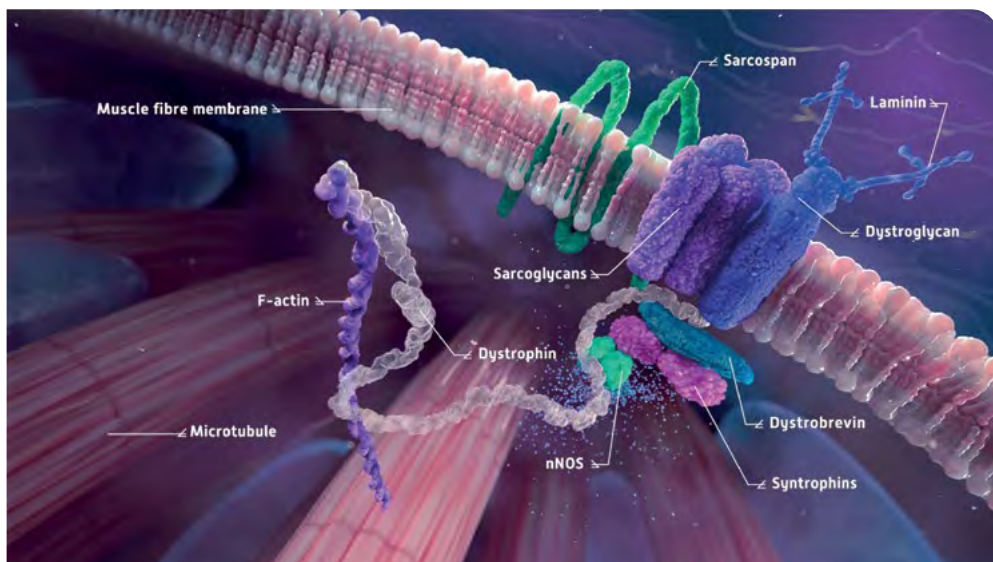
**Channelopathies in childhood: the mosaic of myotonia**

**Presenter:** Dr. Pinki Munot, Department of Neurology, Great Ormond Street Hospital (GOSH), NHS Foundation Trust, London, UK

**Description:** This session discusses the physiological and clinical attributes of skeletal muscle channelopathies. It explains the heterogeneity of non-dystrophic myotonia in children, including symptoms, patient impact, disease burden, and clinical management. development program in DMD and potential utility of SAT-3247 in other muscle disorders.

**ITF-RareDiseases.com is now LIVE:**  
a comprehensive online resource designed for Duchenne  
muscular dystrophy specialists

Expand your knowledge, find practical information and stay updated  
with the latest topics and international conferences in DMD



*Video illustrating the pathological cascade of events triggered by the disassembly of the DAPC in DMD  
(available in the DMD section of the ITF-RareDiseases website)*

**Access [www.ITFRareDiseases.com](http://www.ITFRareDiseases.com)**

**DMD:** Duchenne muscular dystrophy; **DAPC:** Dystrophin-associated protein complex.

A Novartis-sponsored symposium  
at the **International Congress  
on Neuromuscular Diseases**

7–11 July 2026

# The transforming SMA landscape

Understanding the unmet needs  
and ongoing clinical experience

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Spadolini Attic: Room 4, Fortezza da Basso, Florence, Italy  
**Wednesday 8 July 2026, 13:15–14:15 CEST**

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Please join Prof. Eugenio Mercuri, Dr. Felipe Franco da Graça, and Dr. Luca Labianca as they explore how registries are helping us to understand the evolving natural history in SMA, the changing unmet needs, and current and future approaches to scoliosis management.



**Prof. Eugenio  
Mercuri (Chair)**

Rome, Italy

Advancing evidence and care in SMA



**Dr. Felipe  
Franco da Graça**

Campinas, São Paulo, Brazil

Seeing unmet needs in SMA differently:  
Insights and opportunities



**Dr. Luca  
Labianca**

Rome, Italy

Innovations and insights in scoliosis  
management in SMA

SMA, spinal muscular atrophy.

This presentation was approved by the Program Committee as an independent activity held in conjunction with the 19th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2026.

This symposium is organized and funded by Novartis and is intended for an audience of non-UK HCPs and non-US HCPs within the context of the ICNMD Congress 2026 in Florence, Italy.

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 **NOVARTIS**

# Industry-Supported Symposia

Wednesday 8 July | Lunch Symposium 13:15 - 14:15



## ISS01 – Peripheral neuropathies unmasked: Decoding pathophysiology and overcoming challenges

- 📍 **Location:** Spadolini Attic – Room 3
- **Theme:** MMN / CIDP

### Presenters:

- **Chair/ Speaker:** Prof. Claudia Sommer (Claudia Sommer, MD - Universitätsklinikum Würzburg, Würzburg, Germany)
- **Chair:** Prof. Hans Dieter Katzberg (Hans Katzberg MD, MSc, FRCPC, FAAN - Professor of Medicine, University of Toronto / Krembil Family Chair in Neurology / Division Head, Neurology at University Health Network and Sinai Health)
- **Speaker:** Prof. Pietro Emiliano Doneddu (Pietro Emiliano Doneddu, MD - Associate Professor, Neuromuscular and Neuroimmunology Unit, IRCCS Humanitas Research Hospital, Milano, Italy / Department of Biomedical Sciences, Humanitas University, Milan, Italy)
- **Speaker:** Prof. Channa Adithya Ashubodha Hewamadduma (Channa Hewamadduma, MBBS, MRCP, MRCP, PhD - Sheffield Institute for Translational Neuroscience (SITraN), School of Medicine and Population Health, University of Sheffield. UK / Academic Neuromuscular Unit, Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield, UK)

**Description:** Join us for an engaging symposium exploring peripheral neuropathies through expert-led presentations and moderated discussions. This symposium will examine the underlying pathophysiology of peripheral neuropathies, with a focus on MMN and CIDP, including the role of autoantibodies and complement, alongside current diagnostic challenges and unmet needs. Integrated discussions and Q&A will help translate mechanistic insights into clinical approaches that may reduce disease burden.

### Learning Objectives:

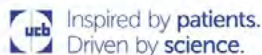
- Explore the heterogeneous mechanisms of autoantibody- and complement-mediated nerve injury in MMN and CIDP
- Discuss diagnosis challenges and the progressive burden of disease of MMN
- Highlight the burden of disease and unmet treatment needs in CIDP

### Agenda:

13:15 - 13:19	Introduction: Setting the scene	Prof. Claudia Sommer/ Prof. Hans Dieter Katzberg
13:19 - 13:23	The suspects: Overview of peripheral neuropathies	Prof. Claudia Sommer
13:23 - 13:41	The clues: Pathophysiology deep dive – Autoantibodies and complement in MMN and CIDP	Prof. Claudia Sommer/ Prof. Pietro Emiliano Doneddu
13:41 - 13:51	The loose ends: Overcoming challenges in MMN	Prof. Channa Adithya Ashubodha Hewamadduma
13:51 - 14:01	The loose ends: Overcoming challenges in CIDP	Prof. Hans Dieter Katzberg
14:01 - 14:15	Moderated discussion and Q&A	All

# Industry-Supported Symposia

Wednesday 8 July | Lunch Symposium 13:15 - 14:15



## ISS02 – Clarity is critical: Key factors driving treatment decisions with targeted therapies in generalised Myasthenia Gravis (gMG)

📍 **Location:** Spadolini Attic – Room 1

● **Theme:** MG

### Presenters:

- **Chair/ Speaker:** Prof. Pushpa Narayanaswami (Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, MA, USA)
- **Speaker:** Prof. Anna Rostedt Punga (Uppsala University, Uppsala, Sweden)
- **Speaker:** Dr. Vincenzo Di Stefano (University of Palermo, Palermo, Italy)
- **Speaker:** Prof. Suraj Muley (HonorHealth Neurology, Scottsdale, AZ, USA)

**Description:** This symposium provides an opportunity to explore the key factors driving treatment decisions with targeted therapies in generalised myasthenia gravis (gMG) through an engaging ‘choose your own story’ case study format. Featuring Professor Pushpa Narayanaswami as Chair, the session will discuss best practice strategies when managing patients with gMG, including the use of current guidelines, the role of current and emerging biomarkers, and clinical experience. Our expert faculty will guide the audience through the latest clinical data, and its relevance to key patient factors, to provide clarity when selecting appropriate targeted therapies in gMG, as part of individualised, patient-centred care.

### Learning Objectives:

- Describe current best practice strategies in the management of generalised Myasthenia Gravis (gMG), including use of local and international guidelines/recommendations.
- Discuss the role of established and emerging biomarkers in MG in treatment decisions.
- Provide clarity to HCPs, through the use of the latest clinical data and expert guidance, when selecting appropriate targeted therapies, including anti-FcRn and C5 inhibitors, for patients with gMG.
- Demonstrate how recent data for targeted treatments support their role in optimising gMG patient management by addressing specific patient needs as part of individualised patient-centred treatment selection.

### Agenda:

13:15 - 13:20	Best practice treatment strategies in gMG	Prof. Pushpa Narayanaswami
13:20 - 13:30	Advancing MG care: The need for objective biomarkers	Prof. Anna Rostedt Punga
13:30 - 14:10	Targeted treatment selection: Facilitating patient choice	Dr. Vincenzo Di Stefano/ Prof. Suraj Muley
	Muley Discussions facilitated	Prof. Pushpa Narayanaswami
14:10 - 14:15	Transforming gMG care, today	Prof. Pushpa Narayanaswami

# Industry-Supported Symposia

Wednesday 8 July | Symposium 13:15 - 14:15



## ISS03 – Functional Improvement: Moving beyond dystrophin in DMD

- 📍 **Location:** Spadolini Attic – Room 2
- **Theme:** DMD

### Presenters:

- **Chair/ Speaker:** Prof. Liesbeth De Waele (University Hospitals Leuven & Department of Development and Regeneration, KU Leuven, Belgium)
- **Chair/ Speaker:** Prof. Perry Shieh (Departments of Neurology and Pediatrics David Geffen School of Medicine at UCLA University of California, Los Angeles)

**Description:** In this Industry Symposium, world-leading experts will reframe expectations for functional improvement in Duchenne muscular dystrophy (DMD), reflect on the evolving clinical landscape and ongoing efforts to address unmet needs with investigational therapeutic zecicent rostudirsen. An important conversation will be held around transforming our approach to patient outcomes in DMD where dystrophin quantity, quality, and distribution form the basis for functional improvement that may be meaningful to patients and families.

### Learning Objectives:

Highlight remaining unmet therapeutic needs in DMD and the important attributes of dystrophin-producing therapies that drive functional improvement. Explore the evolving definition of treatment success in DMD, from dystrophin expression to clinical endpoints that can be contextualized to outcomes that are meaningful for individuals with DMD and their families. Spotlight clinical data from the DELIVER trial of zecicent rostudirsen that demonstrate functional improvement in DMD. Discuss evolving therapeutic goals in DMD, from slowing decline to measurable, meaningful functional improvement.

### Agenda:

	Welcome	Prof. Perry Shieh
	Unmet needs in DMD	Prof. Perry Shieh
	Resetting expectations for functional improvement	Prof. Perry Shieh
	DELIVER trial data: functional outcomes in focus	Prof. Liesbeth De Waele
	Moving the needle in DMD	Prof. Liesbeth De Waele
	Audience Q&A and close	All

# Industry-Supported Symposia

Wednesday 8 July | Lunch Symposium 13:15 - 14:15

**ISS04 – The transforming SMA landscape: Understanding the unmet needs and ongoing clinical experience**



📍 **Location:** Spadolini Attic – Room 4  
● **Theme:** SMA

## Presenters:

- **Chair/ Speaker:** Prof. Eugenio Mercuri (Professor of Paediatric Neurology Head, Paediatric Neurology Unit Gemelli University Hospital Catholic University Rome, Italy)
- **Speaker:** Dr. Felipe Franco da Graça (Neurologist and Clinical Neurophysiologist University of Campinas Hospital (HCUNICAMP) Campinas, São Paulo, Brazil)
- **Speaker:** Dr. Luca Labianca (Orthopaedics and Traumatology Paediatric Orthopaedics UPMC Salvator Mundi International Hospital Rome, Italy)

## Agenda:

13:15 - 13:30	Advancing evidence and care in SMA	Prof. Eugenio Mercuri
13:30 - 13:45	Seeing unmet needs in SMA differently: Insights and opportunities	Dr. Felipe Franco da Graça
13:45 - 14:00	Innovations and insights in scoliosis management in SMA	Dr. Luca Labianca
14:00 - 14:15	Key takeaways, discussion, and closing remarks	All

# Industry-Supported Symposia

Thursday 9 July | Morning Symposium 07:00 - 08:00

ISS05 – Perspectives on gMG: Evolving Clinical Practice and Emerging Targets



📍 **Location:** Spadolini Attic – Room 4  
● **Theme:** MG

## Presenters:

- **Chair/ Speaker:** Dr. Stephen Reddel (Sydney Neurology, Brain & Mind Centre, University of Sydney, Australia)
- **Speaker:** Dr. Carolina Barnett-Tapia (University of Toronto, Toronto, Canada)
- **Speaker:** Dr. Raffaele Iorio (Gemelli University Hospital, Rome, Italy)

## Agenda:

07:00 - 07:05	Welcome and introduction	Dr. Stephen Reddel
07:05 - 07:10	gMG Pathopharmacology: A Brief Overview	Dr. Stephen Reddel
07:10 - 07:20	Patients Living with gMG Are Doing Better — But Are They Doing Well?	Dr. Carolina Barnett-Tapia
07:20 - 07:30	Panel discussion- gMG Management in Practice & Where Challenges Remain	All
07:30 - 07:40	The Potential for BTK Inhibition to Modulate B Cell and Macrophage Activity in gMG	Dr. Raffaele Iorio
07:40 - 07:50	The Potential for Factor B Inhibition to Modulate the Alternative Complement Pathway in gMG	Dr. Stephen Reddel
07:50 - 08:00	Panel discussion - Future Directions in gMG Audience/ Q&A and Close	All

# Industry-Supported Symposia

Thursday 9 July | Morning Symposium 07:00 - 08:00



## ISS06 – Redefining Duchenne: The Impact of Multidisciplinary Approaches and Emerging Therapies

- 📍 **Location:** Spadolini Attic – Room 1
- **Theme:** DMD

### Presenters:

- **Chair/ Speaker:** Prof. Eugenio Mercuri (Gemelli Hospital Catholic University, Rome, Italy)
- **Speaker:** Prof. Andreas Hahn (Universitätsklinikum Gießen (UKGM), Germany)
- **Speaker:** Prof. Michela Guglieri (Newcastle University and Newcastle Hospitals NHS Foundation Trust, UK)

**Description:** Survival and standards of care in Duchenne muscular dystrophy (DMD) have evolved substantially, reshaping the clinical journey from early childhood into adulthood. This symposium will explore how improvements in life expectancy have increased the importance of coordinated, multidisciplinary care across all disease stages. Expert faculty will examine the role of the multidisciplinary team in managing the growing complexity of Duchenne, highlighting practical approaches to optimising long-term outcomes.

The session will also review the long-term benefits and recognised limitations of corticosteroid therapy in Duchenne, drawing on key clinical evidence. Finally, emerging data on the evolving standard of care regimens will be discussed, with a focus on their potential impact on long-term disease trajectory as well as clinically relevant outcomes such as growth and skeletal health. Together, these perspectives aim to challenge current assumptions and stimulate discussion on whether—and how—we may be beginning to change the long-term trajectory of Duchenne.

### Learning Objectives:

- Describe the evolving Duchenne care landscape, including improved survival and the increasing importance of coordinated multidisciplinary management
- Review the long-term benefits and limitations of corticosteroid therapy in Duchenne, including key clinical evidence across disease stages
- Discuss emerging data on the evolving standard of care, including potential impact on growth and skeletal health

### Agenda:

07:00 - 07:05	Welcome and Introductions.	Prof. Eugenio Mercuri
07:05 - 07:25	Coordinating complexity: The MDT across the Duchenne journey.	Prof. Andreas Hahn
07:25 - 07:45	Long-term outcomes in Duchenne: Are we changing the trajectory?	Prof. Michela Guglieri
07:45 - 08:00	Audience Q&A and close.	Prof. Eugenio Mercuri

# Industry-Supported Symposia

Thursday 9 July | Lunch Symposium 13:15 - 14:15



## ISS07 – Givinostat: Bridging Clinical Evidence to Real-World Practice in Duchenne Muscular Dystrophy

📍 **Location:** Spadolini Attic – Room 1

● **Theme:** DMD

### Presenters:

- **Chair/ Speaker:** Dr. Erik Niks (Pediatric and Adult Neurologist, Leiden University Medical Center, Leiden, Netherlands)
- **Speaker:** Prof. Dr. Eugenio Mercuri (Pediatric Neurology Institute, Catholic University and Nemo Pediatrico, Fondazione Policlinico Gemelli IRCCS, Rome, Italy)
- **Speaker:** Dr. Perry Shieh (Professor of Neurology and Pediatrics, University of California Los Angeles, Los Angeles, California, USA)

**Description:** This 60-minute session explores the translation of givinostat from pivotal clinical trials to real-world clinical practice in Duchenne muscular dystrophy (DMD). It will cover the scientific foundation of histone deacetylase (HDAC) inhibition, Phase 3 EPIDYS efficacy and safety data, long-term functional outcomes from the open-label extension (OLE) study, and practical management insights from the Italian Early Access Program (EAP) and emerging US clinical experience.

### Learning Objectives:

- Review the mechanism of action of HDAC inhibition and its role in targeting DMD pathology
- Analyze safety and efficacy results from the Phase 3 EPIDYS trial and long-term OLE data
- Discuss real-world safety and dose management strategies from the Italian EAP cohort
- Evaluate practical considerations for implementing weight-based dosing and monitoring (platelets, triglycerides, and cardiac parameters) in accordance with the summary of product characteristics (SmPC) guidance

### Agenda:

13:15 - 13:30	DMD Therapeutic Landscape and the Phase 3 EPIDYS Study	Dr. Erik Niks
13:30 - 13:50	Long-Term Extension Data, Real-World Experience, and Practical Management Considerations	Prof. Dr. Eugenio Mercuri
13:50 - 14:05	US Clinical Practice Experience With Givinostat	Dr. Perry Shieh
14:05 - 14:15	Panel Discussion and Audience Q&A	All

# Industry-Supported Symposia

Thursday 9 July | Lunch Symposium 13:15 - 14:15



## ISS08 – Complement Component 5 Inhibitor Therapies (C5ITs) in Generalised Myasthenia Gravis (gMG): From Established Evidence to Latest Clinical Data

- 📍 **Location:** Spadolini Attic – Room 2
- **Theme:** MG

### Presenters:

- **Chair/ Speaker:** Dr. Sabrina Sacconi (CHU de Nice, France)
- **Speaker:** Dr. Elena Sacconi (UO Neurologia Azienda Ospedaliero Universitaria di Parma)

**Description:** To explore how the complement component 5 inhibitors, eculizumab and ravulizumab, have changed the treatment paradigm for patients with anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalised myasthenia gravis (gMG). We will investigate long-term efficacy and safety data for eculizumab and ravulizumab as treatments for adults with AChR-Ab+ gMG from clinical trials to a real-world setting, discuss the implementation of recent guidelines and how they impact clinical decision-making, and describe the burden associated with gMG and how eculizumab and ravulizumab helped to improve the patient experience.

### Learning Objectives:

- Understand the burden of illness and unmet needs for patients with AChR-Ab+ gMG
- Discuss the long-term effectiveness and safety of eculizumab and ravulizumab as treatments for AChR-Ab+ gMG
- Explore gMG patient cases who were treated with ravulizumab

### Agenda:

	The Impact of gMG: Understanding Disease Burden and Unmet Needs	Dr. Elena Sacconi
	From Clinical Trials to Clinical Practice: Long-Term Effectiveness and Safety of C5ITs in gMG	Dr. Sabrina Sacconi

# Industry-Supported Symposia

Thursday 9 July | Lunch Symposium 13:15 - 14:15



**ISS09 – A new era in SMA: improving outcomes across every stage of SMA care**

📍 **Location:** Spadolini Attic – Room 3

● **Theme:** SMA

## Presenters:

- **Chair/ Speaker:** Prof. Giovanni Baranello (Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, NIHR Great Ormond Street Hospital Biomedical Research Centre and Great Ormond Street Hospital for Children NHS Foundation Trust, London, UK)
- **Speaker:** Dr. Felix Kleefeld (Department of Neurology, BG University Hospital Bergmannsheil, Ruhr University Bochum, Bochum, Germany)
- **Speaker:** Prof. Valeria Sansone (NeMO Clinical Center, Neurorehabilitation Unit, University of Milan, Milan, Italy)

**Description:** Despite recent advancements, residual unmet needs remain across the population of people living with SMA, from pre-symptomatic infants to adults. This symposium will discuss:

- How infants with severe SMA phenotypes may experience limited benefit from pre-symptomatic treatment
- The changing SMA population and their unmet needs in the era of DMTs
- The importance of expanding outcomes beyond motor scales to more accurately assess disease burden and treatment effect

## Learning Objectives:

- Understand the importance of pre-symptomatic treatment of SMA to improve long-term treatment outcomes, and highlight the treatment gaps for those with severe phenotypes
- Discuss the developments made in SMA over the past decade, and highlight residual treatment/care gaps
- Highlight the need to reconsider treatment goals for all people living with SMA (including children and adults), with a particular focus on expanding beyond motor outcomes (e.g. quality of life and cognition)

## Agenda:

13:15 - 13:20	Welcome and introduction	Prof. Giovanni Baranello
13:20 - 13:32	Defining the therapeutic window: understanding the importance of pre-symptomatic SMA intervention	Prof. Giovanni Baranello
13:32 - 13:44	Understanding the transforming SMA landscape: exploring the remaining treatment gaps	Dr. Felix Kleefeld
13:44 - 13:56	From clinical trials to lived experience: embracing the full story of people with SMA	Prof. Valeria Sansone
13:56 - 14:10	Panel discussion and Q&A	All
14:10 - 14:15	Closing Remarks	All

# Industry-Supported Symposia

Thursday 9 July | Lunch Symposium 13:15 - 14:15



ISS10 – Connections in CIDP: From Clinical Need to Future Care

📍 **Location:** Spadolini Attic – Room 4

● **Theme:** CIDP

## Presenters:

- **Chair/ Speaker:** Prof. Pietro Doneddu (Neuromuscular and Neuroimmunology Unit IRCCS Humanitas Research Hospital, Milan)
- **Speaker:** Dr. Chafic Karam (Professor of Neurology at Penn Medicine, PA, USA)
- **Speaker:** Prof. Simon Rinaldi (Nuffield Department of Clinical Neurosciences, Oxford, UK)

**Description:** A clinically-anchored CIDP story that starts with unmet need in clinical practice (partial/inadequate responders, refractory patients, residual disability) using a patient case fil-rouge that connects that need to mechanistic understanding focusing on the role of complement in axonal injury, and finally to innovations and where the field is going (biomarkers/precision medicine, e.g., NfL; practical ways to identify and monitor risk of ongoing axonal damage)

## Learning Objectives:

- Identify key unmet needs in CIDP, including residual disability, inadequate response, and refractory disease, and their impact on management goals in real-world practice
- Explain how CIDP pathobiology, including complement-mediated immune mechanisms, demyelination, and axonal injury, may contribute to residual disability
- Review emerging approaches, biomarkers and clinical measures that may enable more targeted care

## Agenda:

13:15 - 13:20	Setting the Scene:The Current State of CIDP	Prof. Pietro Doneddu
13:20 - 13:35	Understanding the Gaps: Unmet Needs in Clinical Practice	Dr. Chafic Karam
13:35 - 13:50	Connecting the Dots:The Role of Complement in CIDP Pathobiology	Prof. Pietro Doneddu
13:50 - 14:03	The Path Ahead: Biomarkers in CIDP	Prof. Simon Rinaldi
14:03 - 14:15	Bringing it Together: Panel Discussion and Q&A	All

# Industry-Supported Symposia

Friday 10 July | Morning Symposium 07:00 - 08:00

## ISS11 – Exon skipping in DMD: The Entrada EEV approach and early clinical data



- 📍 **Location:** Spadolini Attic – Room 4
- **Theme:** DMD

### Presenters:

- **Chair:** Dr. Christine Clemson (Entrada Therapeutics, Boston, MA, USA)
- **Speaker:** Dr. Natarajan Sethuraman (Entrada Therapeutics, Boston, MA, USA)
- **Speaker:** Dr. Erik Niks (Department of Neurology, Leiden University Medical Center, Leiden, Netherlands)
- **Speaker:** Dr. Laurent Servais (MDUK Oxford Neuromuscular Centre & NIHR Oxford Biomedical Research, University of Oxford, Oxford, UK, and Neuromuscular Reference Centre, University Hospital of Liège, Liège, Belgium)
- **Speaker:** Enrico Iovene (Patient Advocate, Italy)

**Description:** This interactive symposium will provide a patient perspective on living with Duchenne muscular dystrophy (DMD) as well as an overview of the technology behind Entrada’s Endosomal Escape Vehicle (EEV™)-based exon-skipping investigational therapies for DMD. In addition, we will review new clinical data from the ELEVATE-44-201 study in patients living with DMD.

### Learning Objectives:

- Understand the patient experience — Gain perspective on the lived experience of patients living with Duchenne muscular dystrophy (DMD) and their families
- Understand EEV Platform technology — Describe the mechanism of Entrada’s Endosomal Escape Vehicle (EEV™) Platform for intracellular delivery and its application to the DMD pipeline
- Describe the ELEVATE-44-201 and ELEVATE-45-201 study designs — Summarize the rationales, designs, and patient populations of the ELEVATE-44-201 and ELEVATE-45-201 Phase 1/2 clinical studies for patients living with DMD amenable to exon 44 or 45 skipping
- Present early clinical findings — Review and discuss the first-in-patient clinical data from the ELEVATE-44-201 study, including safety and preliminary efficacy signals
- Evaluate the therapeutic landscape — Engage in a multidisciplinary discussion on the implications of these findings for the broader DMD treatment landscape and future research directions

### Agenda:

07:00 - 07:05	Welcome and Introductions	Dr. Christine Clemson
07:05 - 07:10	The Patient Perspective: Living with MDM - Patient / family advocate	Enrico Lovene
07:10 - 07:20	EEV Platform technology: Intracellular delivery and the Entrada DMD pipeline	Dr. Natarajan Sethuraman
07:20 - 07:30	The ELEVATE-44-201 and ELEVATE-45-201 studies: Designs, rationales and patient populations	Dr. Erik Niks
07:30 - 07:40	Early clinical data from the ELEVATE-44-201 study	Dr. Laurent Servais
07:40 - 07:55	Panel discussion and Q&A	All
07:55 - 08:00	Closing remarks	Dr. Christine Clemson

# Industry-Supported Symposia

Friday 10 July | Morning Symposium 07:00 - 08:00



## ISS12 – Targeting Pathogenic B Cells in Generalized Myasthenia Gravis Through Upstream BAFF/APRIL Inhibition

📍 **Location:** Spadolini Attic – Room 1

● **Theme:** MG

### Presenters:

- **Chair:** Prof. Heinz Wiendl (Department of Neurology and Neurophysiology, University Medical Center Freiburg, Freiburg, Germany)
- **Speaker:** Prof. Dr. Kristl Claeys (Kliniekhoofd Neurologie, Neuromusculaire Ziekten, Laboratory for Muscle Diseases and Neuropathies, UZ Leuven, Leuven, Belgium)
- **Speaker:** Dr. Ali A. Habib (University of California, Irvine, Orange, CA, USA)

**Description:** Join us for an engaging presentation exploring the role of B cells and the cytokines BAFF and APRIL in the pathophysiology of myasthenia gravis. Discover the science behind telitaccept, an investigational therapy in generalized myasthenia gravis, and learn about UPSTREAM MG, a global Phase 3 clinical trial currently underway.

### Learning Objectives:

Understand the role of B-cell dysregulation, including BAFF and APRIL signaling, in the pathogenesis of myasthenia gravis and the rationale for targeting these pathways Review the efficacy and safety findings from the Phase 3 trial of telitaccept conducted in Chinese patients with generalized myasthenia gravis Discuss the design and objectives of the UPSTREAM MG Global Phase 3 trial in patients with generalized myasthenia gravis

### Agenda:

07:00 - 07:05	Welcome and Introductions	Prof. Heinz Wiendl
07:05 - 07:20	B-Cell Biology and the Role of BAFF/APRIL in Myasthenia Gravis Pathophysiology	Prof. Heinz Wiendl
07:20 - 07:35	Telitaccept and Existing Clinical Evidence in Patients with Generalized Myasthenia Gravis	Prof. Dr. Kristl Claeys
07:35 - 07:50	The UPSTREAM MG Study: Design and Objectives of a Global Phase 3 Program	Dr. Ali A. Habib
07:50 - 08:00	Panel Q&A	All

# Industry-Supported Symposia

Friday 10 July | Lunch Symposium 13:15 - 14:15



**ISS13 – IgG fragment-mediated FcRn blockade: A path to early, patient-centered innovation in gMG**

📍 **Location:** Spadolini Attic – Room 3  
● **Theme:** MG

## Presenters:

- **Chair/ Speaker:** Prof. Kristl Claeys (University Hospitals Leuven and KU Leuven, Belgium)
- **Chair/ Speaker:** Prof. Tobias Ruck (BG University Hospital Bergmannsheil Bochum, Germany)
- **Speaker:** Prof. Raffaele Iorio (Fondazione Policlinico Universitario A. Gemelli and Università Cattolica del Sacro Cuore, Italy)
- **Speaker:** Prof. Carolina Barnett-Tapia (University of Toronto and Toronto General Hospital, Canada)

**Description:** An interactive symposium, exploring gMG management. Through expert dialogue and case-based discussion, this symposium will examine how early intervention, disease and treatment biology, individualized dosing, and monitoring can inform clinical decision-making and shape patient outcomes.

## Learning Objectives:

- Understand the rationale for early intervention in gMG
- Examine mechanistic and clinical differentiation of an Fc fragment-based therapy
- Apply individualized, evidence-based approaches through case discussions

## Agenda:

13:15 - 13:18	Welcome & introduction	Prof. Kristl Claeys
13:18 - 13:30	Timing: Early intervention in gMG	Prof. Tobias Ruck
13:30 - 13:42	Precision: Fragment-based FcRn IgG modulation	Prof. Kristl Claeys
13:42 - 13:50	Convenience: Individualized dosing with pre filled syringe	Prof. Raffaele Iorio
13:50 - 14:05	Patient-centered care: Interactive case studies	Prof. Carolina Barnett-Tapia
14:05 - 14:15	Audience Q&A	All

# Industry-Supported Symposia

Friday 10 July | Lunch Symposium 13:15 - 14:15

**ISS14 – Advancing TK2d management: From natural history to clinical evidence and real-world opportunities**



📍 **Location:** Spadolini Attic – Room 1  
● **Theme:** TK2D

## Presenters:

- **Chair/ Speaker:** Prof. Caterina Garone (Department of Medical and Surgical Sciences, Alma Mater Studiorum, University of Bologna, Bologna, Italy)
- **Speaker:** Prof. Yolanda Cámara (Mitochondrial and Neuromuscular Pathology Group, Vall d'Hebron Research Institute/CIBERER, Barcelona, Spain)
- **Speaker:** Prof. Cristina Domínguez-González (University Hospital 12 de Octubre, National Reference Center for Rare Neuromuscular Diseases, ERN EURO-NMD imas12 Research Institute, CIBERER Madrid, Spain)

**Description:** This symposium will provide a comprehensive overview of thymidine kinase 2 deficiency (TK2d), examining the pathophysiology of the disease and its associated morbidity and mortality. Faculty will explore the molecular mechanisms underlying TK2d, including the role of nucleoside metabolism and mitochondrial DNA maintenance, to highlight why early recognition and timely intervention are critical for improving patient outcomes. Emerging clinical data will be presented to contextualise evolving approaches to patient characterisation and treatment, drawing on evidence from recent studies to inform expectations across different patient populations. Through expert discussion and real-world case presentations spanning both adult and paediatric patients, the session will address practical considerations for diagnosis, treatment initiation, and ongoing monitoring, with the aim of supporting a multidisciplinary approach to care for patients living with TK2d.

## Learning Objectives:

- Explain the pathophysiological mechanisms underlying TK2d, including the role of nucleoside metabolism and mitochondrial DNA maintenance
- Discuss emerging evidence on patient characterisation and treatment approaches in TK2d and their implications for clinical management
- Apply clinical insights from real-world cases to improve diagnosis, treatment initiation, and monitoring of patients with TK2d

## Agenda:

13:15 - 13:20	Welcome and introduction	13:50–14:05: Identifying and treating patients in the real world: Instructive cases	Prof. Caterina Garone
13:20 - 13:35	Looking more closely at the pathophysiology of TK2d		Prof. Yolanda Cámara
13:35 - 13:50	New perspectives in patient characterisation and treatment		Prof. Cristina Domínguez-González
13:50 - 14:05	Identifying and treating patients in the real world: Instructive cases		Prof. Caterina Garone
14:05 - 14:15	Panel discussion and closing remarks		All

# Industry-Supported Symposia

Friday 10 July | Lunch Symposium 13:15 - 14:15



ISS15 – Shaping the journey ahead: The importance of starting early in SMA

📍 **Location:** Spadolini Attic – Room 4  
● **Theme:** SMA

## Presenters:

- **Chair/ Speaker:** Prof. Giovanni Baranello (Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health; Great Ormond Street Hospital NHS Foundation Trust London, UK)
- **Speaker:** Dr. Teresa Moreno (Unidade de Neuropediatria, Centro Hospitalar Universitario Lisboa Norte, Lisbon, Portugal)
- **Speaker:** Prof. Liesbeth De Waele (Department of Pediatric Neurology, University Hospitals Leuven, Leuven, Belgium)

# Industry-Supported Symposia

Friday 10 July | Lunch Symposium 13:15 - 14:15



## ISS16 – Switching therapy in late-onset Pompe disease (LOPD): Bridging the gap between data and what really matters to patients

📍 **Location:** Spadolini Attic – Room 2

● **Theme:** Pompe

### Presenters:

- **Chair/ Speaker:** Prof. Antonio Toscano (Professor of Neurology at the University of Messina, Italy, and Head of the Neurology and Neuromuscular Disorders Unit. Member of the EAN Programme Committee since 2019.)
- **Speaker:** Dr. Menekşe Öztürk (Clinician Scientist in Neuromuscular Disorders at BG Universitätsklinikum Bergmannsheil in Germany, and a Project Program Coordinator for the B2B-RARE initiative.)
- **Speaker:** Prof. Olimpia Musumeci (Professor of Neurology at the University of Messina, Italy, and responsible for the Reference Centre for Rare Neurological and Neuromuscular Disorders.)
- **Speaker:** Dr. Nadine van der Beek (Neurologist at the department of Neurology at Erasmus MC. Board member of the Spierziekten centrum Nederland, and a co-chair of the muscle working group of the European Reference Network for Neuromuscular Diseases (EURO-NMD).)

**Description:** Our expert panel invites you to join them around the kitchen table as we explore switching therapy in LOPD, with a focus on how we can bridge the gap between the clinical data and what really matters to patients. Whilst sharing long-term data, case studies and patient insights, our expert panel will be challenging you to consider:

- Are we missing what matters more to adults with LOPD?
- How can we apply learnings on long-term efficacy from the open-label extension data?
- In an era of multiple options, how do we approach switching therapy? Join us for an immersive experience where we invite you to really think about what matters more—the coffee is brewing! This Satellite Symposium is organised and sponsored by Amicus Therapeutics and is intended for registered healthcare professionals.

### Learning Objectives:

By the end of this symposium, delegates will be able to:

- Understand the current treatment landscape and their options in late-onset Pompe disease (LOPD)
- Interpret long-term efficacy and safety data for second-generation therapies, including open-label extension findings
- Evaluate when and why to consider switching therapy in clinical practice
- Apply real-world experience and case-based insights to treatment decision-making
- Incorporate patient perspectives to better align clinical decisions with what matters most to adults with LOPD

### Agenda:

	An invitation from our host	Prof. Antonio Toscano
	Understanding the treatment landscape in LOPD	Dr. Menekşe Öztürk
	Long-term efficacy and safety of second-generation therapies in LOPD	Prof. Olimpia Musumeci
	Switching therapy in practice: real-world experience from Erasmus MC (Netherlands)	Dr. Nadine van der Beek
	Patient case discussions and Q&A	All
	Summary and closing remarks	All

# Industry-Supported Symposia

Saturday 11 July | Lunch Symposium 13:00 - 14:00

**Johnson & Johnson**

**ISS17 – Begin at the beginning: reimagine immunotherapy in generalized myasthenia gravis**

📍 **Location:** Spadolini Attic – Room 1

● **Theme:** MG

## Presenters:

- **Chair/ Speaker:** Prof. Heinz Wiendl (Medical Center – University of Freiburg, Freiburg, Germany)
- **Speaker:** Dr Elena Cortés Vicente (Hospital de la Santa Creu i Sant Pau, Barcelona, Spain)
- **Speaker:** Dr Shaha Shelly (Rambam Health Care Campus, Haifa, Israel)

**Description:** This symposium links generalized myasthenia gravis (gMG) immunopathology to therapeutic intervention, with a focus on neonatal fragment crystallizable receptor (FcRn) blockers and nipocalimab. It covers immunopathology and autoantibody subtypes and the clinical impact of different mechanisms of FcRn blockade will be discussed. Through clinical cases and real-world scenarios, attendees will receive practical guidance on how nipocalimab could be integrated into guideline-consistent care pathways to achieve key patient-centred goals in the management of gMG; these goals include fewer exacerbations, stable function, improved quality of life, and reduced steroid dependence.

## Learning Objectives:

After this symposium, participants should be able to:

- Explain how gMG pathophysiology relates to the mechanism of action and clinical effects of current and emerging therapies
- Evaluate the role of FcRn blockers in achieving sustained gMG control and reducing the treatment burden across diverse patient populations
- Apply practical insights for enhancing safe and guideline-consistent patient care to support individualized treatment decisions in gMG

## Agenda:

13:00 - 13:05	Welcome and introduction	Prof. Heinz Wiendl (DE)
13:05 - 13:20	Learning immunopathology via therapeutic intervention	Dr Elena Cortés Vicente (ES)
13:20 - 13:35	From principles to practice: using nipocalimab to achieve sustained gMG control	Prof. Heinz Wiendl (DE)
13:35 - 13:50	Real world decisions in gMG: case based use	Dr Shaha Shelly (IL)
13:50 - 14:00	Q&A and closing	Prof. Heinz Wiendl (DE)

# Industry-Supported Symposia

Saturday 11 July | Lunch Symposium 13:00 - 14:00



## ISS18 – Beyond symptom control: Evolving approaches in MG

📍 **Location:** Spadolini Attic – Room 4

● **Theme:** MG

### Presenters:

- **Chair/ Speaker:** Dr. Katherine Buzzard (Eastern Health Multiple Sclerosis and Neuroimmunology Service)
- **Speaker:** Dr. Raffaele Iorio (Policlinico Universitario Agostino Gemelli)
- **Speaker:** Dr. Tobias Ruck (Bergmannsheil University Hospital)

**Description:** Despite advancements in the myasthenia gravis (MG) treatment landscape, many people living with MG continue to experience significant disease and treatment burden, even when symptoms appear controlled. This symposium brings together leading experts to discuss how well clinical measures reflect patient experience, examine how effectively current approaches address underlying disease mechanisms and explore how emerging upstream strategies may potentially go beyond symptom management and improve patient outcomes.

### Learning Objectives:

- Consider the burden of myasthenia gravis (MG) and highlight the unmet needs despite available therapies
- Outline the pathophysiology of MG and assess whether current treatments target the drivers of disease
- Explore whether targeting mechanisms upstream of the neuromuscular junction could potentially offer an alternative option for disease control

### Agenda:

	Welcome and introduction	Dr. Katherine Buzzard
	The hidden burden of controlled MG	Dr. Raffaele Iorio
	Are we addressing the drivers of the disease?	Dr. Tobias Ruck
	What would it take to change the immune trajectory in MG?	Dr. Katherine Buzzard
	Panel discussion	All
	Q&A and close	All

# Industry-Supported Symposia

Saturday 11 July | Lunch Symposium 13:00 - 14:00



## ISS19 – Precision in Motion: Exploring the Potential of siRNA in Facioscapulohumeral Muscular Dystrophy (FSHD) and Myotonic Dystrophy (DM)

📍 **Location:** Spadolini Attic – Room 3

● **Theme:** FSHD/DM

### Presenters:

- **Chair:** John Graef, Executive Director, Research Partnerships, Sarepta Therapeutics, Inc
- **Speaker:** Richard Roxburgh, FRACP, PhD, Neurologist, Auckland City Hospital; Associate Professor, University of Auckland, Auckland, New Zealand
- **Speaker:** Suzanne Jean Hodgkinson, FRACP, PhD, Associate Professor, University of New South Wales; Senior Neurologist, Department of Neurology; Director, Multiple Sclerosis Clinic, Liverpool Hospital; Immune Tolerance Laboratory, Ingham Institute for Applied Medical Research, New South Wales, Australia

**Description:** Sarepta Therapeutics invites you to join them for a scientific presentation exploring the potential of siRNA-based approaches in neuromuscular diseases. This session will discuss the scientific rationale behind siRNA-based therapeutic approaches in neuromuscular conditions. Intended for Healthcare Professionals.

### Agenda:

13:00 - 13:15	Overview of TRIM siRNA Platform	John Graef
13:15 - 13:20	Facioscapulohumeral Muscular Dystrophy Type (FSHD) Disease State Background	Richard Roxburgh, FRACP, PhD
13:20 - 13:30	Data Reporting from SRP-1001-101	Richard Roxburgh, FRACP, PhD
13:30 - 13:35	Myotonic Dystrophy (DM) Disease State Background	Suzanne Hodgkinson, FRACP, PhD
13:35 - 13:45	Data Reporting from SRP-1003-101	Suzanne Hodgkinson, FRACP, PhD
13:45 - 14:00	Audience Q&A	

Sanofi Industry-Supported Medical Symposium at ICNMD 2026

# CONNECTIONS

## in CIDP

From Clinical Need  
to Future Care

Exploring clinical gaps and what's next in CIDP management



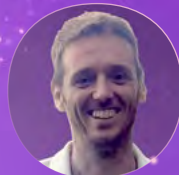
**Pietro Doneddu, MD**

Neuromuscular and Neuroimmunology Unit,  
IRCCS Humanitas Research Hospital;  
Department of Biomedical Sciences,  
Humanitas University,  
Milan, Italy



**Chafic Karam, MD**

Department of Neurology,  
University of Pennsylvania,  
Pennsylvania, USA



**Simon Rinaldi, MBChB, PhD**

Nuffield Department of Clinical Neurosciences,  
University of Oxford and Oxford University  
Hospitals NHS Foundation Trust,  
Oxford, UK

Please join the faculty for a discussion on:



- Unmet needs in CIDP care, including residual disability and clinical goals in real-world practice
- The role of complement in CIDP pathobiology and residual disability
- Emerging approaches, biomarkers and clinical measures for more targeted care



**SAVE THE DATE!**

**Thursday,  
July 9, 2026  
13:15 – 14:15 CEST**

**ADD TO CALENDAR** ↗

CIDP, chronic inflammatory demyelinating polyneuropathy; CME, continuous medical education; ICNMD, International Congress on Neuromuscular Diseases. This presentation was approved by the Program Committee as an independent activity held in conjunction with the 19th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2026. This is a non-promotional medical symposium sponsored by Sanofi. The content was developed by Sanofi in consultation with scientific experts for scientific exchange purposes and is not eligible for CME credits. ©2026 Sanofi. All rights reserved. Sanofi is a registered trademark of Sanofi or an affiliate. 450 Water Street, Cambridge, MA 02141. <https://www.sanofi.com/en/contact>. MAT-GLB-2602331 – 1.0 – 05/2026

**sanofi**



**Come and learn  
more about Takeda's  
commitment to CIDP  
at Booth 121**

# Exhibit & Poster Hall Information

**Location:** Spadolini – Ground Floor

<b>Tuesday 7 July</b>	18:00 - 19:30 (Welcome Reception)
<b>Wednesday 8 July</b>	10:30 - 16:45 10:30 - 11:15 (Poster Tour 1: Muscle and Neuromuscular Junction)
<b>Thursday 9 July</b>	10:30 - 16:45 10:30 - 11:15 (Poster Tour 2: Motor Neuron)
<b>Friday 10 July</b>	10:30 - 16:45 10:30 - 11:15 (Poster Tour 3: Peripheral Neuropathy)

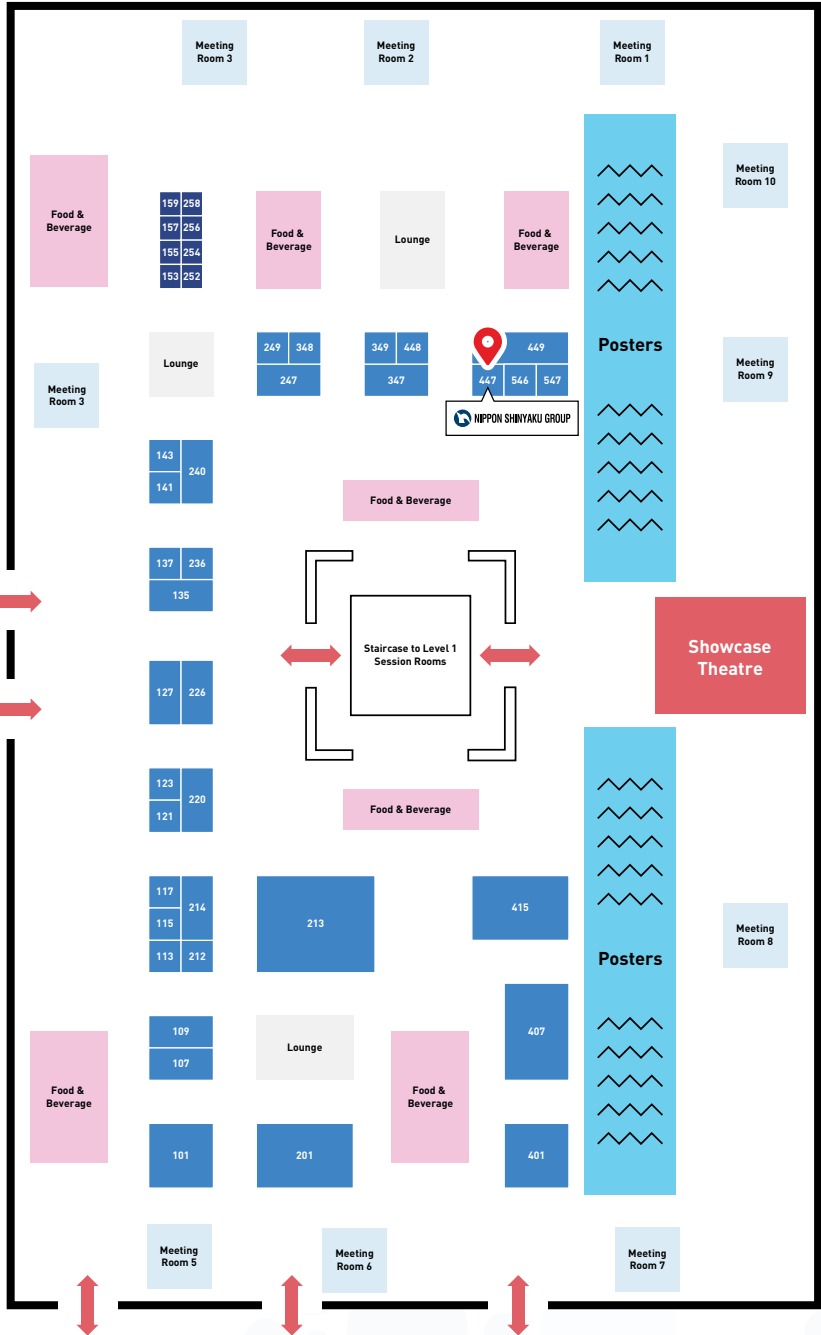
Poster Tours supported by:



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# Exhibit & Poster Hall Floorplan



# Exhibitor Information



## Alexion, AstraZeneca Rare Disease

★ Booth #107

🌐 [alexion.com](http://alexion.com)

Alexion, AstraZeneca Rare Disease, is the group within AstraZeneca focused on rare diseases, created following the 2021 acquisition of Alexion Pharmaceuticals, Inc. As a leader in rare diseases for over 30 years, Alexion is focused on serving patients and families affected by rare diseases and devastating conditions. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries.



## Amgen

★ Booth #449

🌐 [amgen.com](http://amgen.com)

Amgen harnesses the best of biology and technology to fight the world's toughest diseases, and make people's lives easier, fuller and longer. We helped establish the biotechnology industry, and we remain on the cutting-edge of innovation, using technology and human genetic data to push beyond what's known today.



## Amicus Therapeutics

★ Booth #212

🌐 [amicusrx.com](http://amicusrx.com)

BioMarin is a leading, global rare disease company focused on delivering medicines for people living with genetically defined conditions. With the close of the Amicus acquisition, our portfolio has expanded to include therapies for Fabry disease and Pompe disease, expanding our ability to reach more people living with rare genetic conditions.



## argenx

★ Booth #401

🌐 [argenx.com](http://argenx.com)

argenx is a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases. Partnering with patients, healthcare professionals, and leading academic researchers, argenx aims to translate immunology breakthroughs into a world-class portfolio of accessible novel antibody-based medicines for patients living with chronic immune-mediated diseases.

# Exhibitor Information



## ATOM International

★ Booth #137

🌐 [atom-international.org](http://atom-international.org)

ATOM International provides functional testing training and oversight in the support of rare neuromuscular disorders. Our global team of physiotherapists help ensure consistency and confidence in study data via standardized training and comprehensive oversight and documentation.



## BioMarin

★ Booth #113

🌐 [biomarin.com](http://biomarin.com)

BioMarin is a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. Founded in 1997, the San Rafael, California-based company has a proven track record of innovation, with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, BioMarin seeks to unleash the full potential of genetic science by pursuing category-defining medicines that have a profound impact on patients.



## BridgeBio

★ Booth #240

🌐 [bridgebio.com](http://bridgebio.com)

BridgeBio exists to develop transformative medicines for genetic conditions. Millions of people worldwide living with genetic conditions lack treatment options, often because drug development for small patient populations can be commercially challenging. We aim to bridge the gap between advancements in genetic science and meaningful medicines for underserved patient populations. Our decentralized, hub-and-spoke model is designed for speed, precision, and scalability. Autonomous and empowered teams focus on individual conditions, while a central hub provides the clinical, regulatory, and commercial capabilities needed to bring innovation to market.



## DEYMED Diagnostic

★ Booth #123

🌐 [deymed.com](http://deymed.com)

DEYMED Diagnostic is a leading Czech company with an established global existence, specialising in advanced medical diagnostic and treatment technologies for neurophysiology and psychiatry. Founded in 1997, DEYMED has expanded its product range over the years to include EEG, EMG, TMS (Transcranial Magnetic Stimulation), PSG (Polysomnography), and BFB (Biofeedback) systems, trusted by healthcare providers worldwide for their precision, reliability, and ease of use.

# Exhibitor Information



## Dyne Therapeutics

★ Booth #101

📍 [dyne-tx.com](http://dyne-tx.com)

Dyne Therapeutics is focused on delivering functional improvement for people living with genetically driven neuromuscular diseases. We are developing therapeutics that target muscle and the central nervous system (CNS) to address the root cause of disease. The company is advancing clinical programs for Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1) as well as a preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease. At Dyne, we are on a mission to deliver functional improvement for individuals, families and communities.



## Edgewise Therapeutics

★ Booth #117

📍 [edgewisetx.com](http://edgewisetx.com)

Edgewise Therapeutics is a leading muscle disease biopharmaceutical company developing novel therapeutics for muscular dystrophies and serious cardiac conditions. Guided by our approach to targeting the muscle as an organ, we have combined our foundational expertise in muscle biology and small molecule drug discovery to build our proprietary, muscle-focused platform. The entire team at Edgewise is dedicated to our mission: changing the lives of patients and families affected by serious muscle diseases.



## Entrada Therapeutics

★ Booth #135

📍 [entradatx.com](http://entradatx.com)

Entrada Therapeutics is a clinical-stage biopharmaceutical company aiming to transform the lives of patients by establishing a new class of intracellular therapeutics. Entrada's research and development includes several neuromuscular programs.



## ERN EURO-NMD

★ Booth #256

📍 [ern-euro-nmd.eu](http://ern-euro-nmd.eu)

ERN EURO-NMD (European Reference Network for Rare Neuromuscular Diseases) is a EU-funded virtual network that connects 80 expert centers across 25 countries, supporting collaboration between clinicians, researchers, and patient representatives to improve care and advance knowledge in the field of neuromuscular diseases.

# Exhibitor Information



## EURO-DM-CRN

★ Booth #159

🌐 [euro-dmcrn.org](https://euro-dmcrn.org)

The EURO-DM-CRN is a new European network dedicated to advancing clinical research in Myotonic Dystrophies. It harmonizes research practices and streamlines collaborative trials across Europe. By uniting clinicians and researchers, the network aims to accelerate therapeutic development and strengthen Europe's role in global research efforts.



## European Neuromuscular Centre (ENMC)

★ Booth #254

🌐 [enmc.org](https://enmc.org)

The European Neuromuscular Centre (ENMC) was founded in 1992 by a group of European patient associations that dedicated itself to bring leading researchers and clinicians from all over the world together. To achieve this goal, ENMC applies a concept unique in the scientific world, which consists of organising and financing workshops on application basis.



## FSHD Society

★ Booth #258

🌐 [fshdsociety.org](https://fshdsociety.org)

The FSHD Society is the world's leading patient-driven organization dedicated to facioscapulohumeral muscular dystrophy (FSHD). We accelerate research, improve care, empower patients and families, and build community worldwide. Through education, advocacy, and collaboration, we work to bring effective treatments and a cure to everyone affected by FSHD.



## GBS|CIDP Foundation International

★ Booth #157

🌐 [gbs-cidp.org](https://gbs-cidp.org)

The GBS | CIDP Foundation International is a global nonprofit organization supporting individuals and their families affected by Guillain-Barre' syndrome (GBS), chronic inflammatory demyelinating polyneuropathy (CIDP), Multifocal Motor Neuropathy (MMN) and related conditions through a commitment to support, education, research and advocacy. Our vision is for very person affected by GBS, CIDP, MMN, or related syndromes to have access to early and accurate diagnosis, appropriate treatment, and knowledgeable support services.

# Exhibitor Information



## ICNMD 2027, Chiba, Japan

★ Booth #448

🌐 [icnmd.org/future-congresses/#icnmd2027](http://icnmd.org/future-congresses/#icnmd2027)

The 20th International Congress on Neuromuscular Diseases will take place in Chiba, Japan, 6-10 July 2027. Known for its dynamic blend of modern innovation and cultural heritage, Chiba offers an exceptional venue for scientific exchange and international collaboration. Participants can look forward to world-class facilities, easy access from Tokyo, and the opportunity to experience Japan's renowned hospitality and rich scientific community.



## Italfarmaco

★ Booth #201

🌐 [italfarmaco.com](http://italfarmaco.com)

Italfarmaco is a global pharmaceutical company operating in over 90 countries and committed to improving patients' lives through pioneering solutions in women's health, neurology, cardiovascular, and rare conditions. Its rare disease unit is advancing therapies for Duchenne muscular dystrophy and amyotrophic lateral sclerosis, while also developing candidates for Becker muscular dystrophy and polycythemia vera. (EU-ITFRD-25-00006)

## Johnson & Johnson

## Johnson & Johnson

★ Booth #415

🌐 [jnj.com/innovativemedicine/emea/](http://jnj.com/innovativemedicine/emea/)

At Johnson & Johnson, we believe health is everything. Our strength in healthcare innovation empowers us to build a world where complex diseases are prevented, treated, and cured, where treatments are smarter and less invasive, and solutions are personal. Through our expertise in Innovative Medicine and MedTech, we are uniquely positioned to innovate across the full spectrum of healthcare solutions today to deliver the breakthroughs of tomorrow, and profoundly impact health for humanity.



## LFB

★ Booth #249

🌐 [groupe-lfb.com](http://groupe-lfb.com)

LFB is a French biopharmaceutical group that develops, manufactures and markets plasma-derived medicinal products and recombinant proteins for the treatment of patients with serious and often rare diseases, in immunology, haemostasis, and intensive care. Created in 1994, LFB today is a leading European company providing plasma-derived medicines to healthcare professionals.

# Exhibitor Information



## Lupin Neurosciences

★ Booth #347

🌐 [lupin-neurosciences.com](http://lupin-neurosciences.com)

Lupin Neurosciences, a specialty division of Lupin, is focusing on neuromuscular disorders following the launch of the company's first Orphan Drug, NaMuscla® (mexiletine), licensed in Europe and UK for the treatment of non-dystrophic myotonia in adults. The division's aim is to develop treatment solutions within neuromuscular and movement disorder areas.



## Merck

★ Booth #220

🌐 [merckgroup.com](http://merckgroup.com)

Merck has a long-standing legacy in neurology and immunology and aims to improve the lives of patients by addressing areas of unmet medical needs. In addition to Merck's commitment to MS, the company pipeline focuses on discovering potential new therapies in neuroinflammatory and immune-mediated diseases, including SLE, gMG, and NMOSD.



## MGBase Registry

★ Booth #153

🌐 [mgbase.org](http://mgbase.org)

The MGBase Registry, supported by the MSBase Foundation, is a global collaborative registry for clinicians in Myasthenia Gravis (MG). It enables collection, tracking and evaluation of patient outcomes through free, secure, web-based tools accessible anywhere on any device, offering scientific support to advance research and improve understanding of MG worldwide.



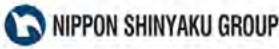
## NADMED

★ Booth #547

🌐 [nadmed.com](http://nadmed.com)

NADMED is a biotech company that offers NAD measuring technology. NADMED method is based on solid research at the University of Helsinki. The method is the first to measure all four NADs quickly and efficiently with the accuracy of mass spectrometry. NADMED aims to be the standard in NAD measuring.

# Exhibitor Information



## Nippon Shinyaku Group

★ Booth #447

🌐 [nippon-shinyaku.co.jp](http://nippon-shinyaku.co.jp)

Nippon Shinyaku is a global, patient centric healthcare company based in Kyoto, Japan, with over a century of history. Guided by our philosophy of helping people lead healthier, happier lives, we develop innovative medicines for Duchenne muscular dystrophy and other rare and serious diseases, addressing high unmet medical needs through science driven research and global collaboration.



## Novartis Pharma AG

★ Booth #407

🌐 [novartis.com](http://novartis.com)

Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach nearly 300 million people worldwide.



## Optics11 Life

★ Booth #546

🌐 [optics11life.com](http://optics11life.com)

Optics11 Life helps neuromuscular researchers move beyond structural readouts by measuring muscle function directly. Our tools quantify force generation, stiffness, and viscoelasticity in cells, tissues, and 3D muscle models under near-physiological conditions. This gives faster, more functional insight into disease mechanisms, therapy response, and model quality for neuromuscular disease research.



## Regeneron Pharmaceuticals

★ Booth #348

🌐 [regeneron.com](http://regeneron.com)

Regeneron is a leading biotechnology company that invents, develops and commercializes life-transforming medicines for serious diseases. Founded and led by physician-scientists, our unique ability to repeatedly translate science into medicine has led to numerous approved treatments and product candidates in development.

# Exhibitor Information



## Sage Publishing

★ Booth #155

🌐 [journals.sagepub.com](https://journals.sagepub.com)

Published by Sage, the gold open access Journal of Neuromuscular Diseases is proud to partner with ICNMD in advancing neuromuscular research and clinical care. The journal publishes basic, translational and clinical research across genetics, pathogenesis, diagnosis and therapeutics for acquired and inherited neuromuscular disorders, supporting scientific discovery and improved patient outcomes.



## Sanofi

★ Sanofi Booth #127 | Sanofi Medical Booth #226

🌐 [sanofi.com/en/patients/understanding-diseases-conditions](https://sanofi.com/en/patients/understanding-diseases-conditions)

Sanofi's Specialty Care business focuses on neurology, rare diseases, rare blood disorders, oncology, and immunology. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. Our approach is shaped by our experience developing highly specialized treatments and forging close relationships with physician and patient communities. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world.



## Santhera Pharmaceutical

★ Booth #109

🌐 [santhera.com](https://santhera.com)

Santhera Pharmaceutical is a Swiss commercial stage biopharmaceutical company committed to developing and commercializing innovative medicines for patients living with rare and other diseases with high unmet medical needs. We are focusing on the development of treatments for neuromuscular diseases that currently lack treatment options, such as Duchenne muscular dystrophy (DMD).



## Sarepta Therapeutics

★ Booth #247

🌐 [sarepta.com](https://sarepta.com)

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold a leadership position in Duchenne muscular dystrophy (Duchenne) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases.

# Exhibitor Information



## Satellos

★ Booth #115

📍 [satellos.com](http://satellos.com)

Satellos is a clinical-stage drug development company focused on restoring natural muscle repair and regeneration in degenerative muscle diseases. The company's first-in-class small molecule drug, SAT-3247, targets AAK1 to replace dystrophin-associated signaling in muscle stem cells and is currently in clinical development as a potential disease-modifying treatment for DMD.



## Scholar Rock

★ Booth #236

📍 [scholarrock.com](http://scholarrock.com)

Scholar Rock is a late-stage biopharmaceutical company on a mission to discover, develop, and deliver life-changing therapies for serious diseases with high unmet need. As a global leader in transforming growth factor beta (TGFB) superfamily biology, the company is focused on advancing innovative treatments where protein growth factors are fundamental.

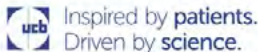


## Takeda Pharmaceuticals

★ Booth #121

📍 [takeda.com](http://takeda.com)

Takeda is focused on creating better health for people and a brighter future for the world. We aim to discover and deliver life-transforming treatments in our core therapeutic and business areas, including gastrointestinal and inflammation, rare diseases, plasma-derived therapies, oncology, neuroscience and vaccines.



## UCB Biopharma

★ Booth #213

📍 [ucb.com](http://ucb.com)

UCB Biopharma aims to improve lives by addressing disease challenges through innovative research in neurology, immunology, and more. Guided by patient perspectives, UCB delivers meaningful solutions globally, fostering collaboration, diversity, and care for people, communities, and the planet, from its Belgian headquarters to nearly 40 countries.

# Exhibitor Information



## University of Rochester Center for Health and Technology (CHeT)

★ Booth #252

🌐 [urmc.rochester.edu/chet](http://urmc.rochester.edu/chet)

University of Rochester Center for Health and Technology (CHeT) Outcomes team develops and validates highly sensitive, disease-specific, regulatory-grade patient and caregiver reported outcome measures for therapeutic trials and drug labelling claims. Our Health Indexes span 40+ neurological, neuromuscular, and movement disorders, have been used in over 100 clinical studies, and translated worldwide into over 40 languages. With extraordinary patient focus, Amicus strives to redefine expectations in rare disease.



## Vor Biopharma

★ Booth #214

🌐 [vorbio.com](http://vorbio.com)

Vor Bio is a clinical-stage biotechnology company rapidly advancing telitaccept, designed to modulate the immune system and deliver meaningful treatment for people living with autoimmune disease. By targeting the BAFF/APRIL pathway, Vor Bio aims to address disease at its source in serious B-cell driven conditions to improve patients' daily lives.



## World Federation of Neurology (WFN)

★ Booth #349

🌐 [wfneurology.org](http://wfneurology.org)

The World Federation of Neurology (WFN) is an association of neurological member societies representing 126 professional societies in all regions of the world. The mission of the WFN is to foster quality neurology and brain health worldwide, a goal we seek to achieve by promoting global neurological education and training.

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[scholarrock.com](https://scholarrock.com)



## **PP01.01 Investigating Intravenous Efgartigimod in Juvenile Generalized Myasthenia Gravis: Results from the ADAPT JR Study**

Dr. Sithara Ramdas<sup>1,2</sup>

<sup>1</sup>Department of Paediatric Neurology, John Radcliffe Hospital, Oxford, United Kingdom.

<sup>2</sup>MDUK Oxford Neuromuscular Centre, Department of Paediatrics, University of Oxford, Oxford, United Kingdom

## **PP01.02 Specific Myostatin Inhibition Ameliorates Sarcopenia and Extends Lifespan in $\alpha$ -Klotho-Null Mice**

Assoc. Prof. Yutaka Ohsawa<sup>1</sup>

<sup>1</sup>Kawasaki Medical School, 577 Matsushima, Kurashiki, Okayama 701-0192, Japan

## **PP01.03 Clinical Correlates of Scoliosis in Spinal Muscular Atrophy Treated with Disease-Modifying Therapies: A Pilot-Observational Study**

Dr. Barbara Risj<sup>1,2</sup>

<sup>1</sup>NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy, Brescia, Italy. <sup>2</sup>Department of Molecular and Translational Medicine, University of Brescia, Brescia, Italy, Brescia, Italy

## **PP01.04 Diagnostic Challenges in Autosomal Dominant Multiple Acyl-CoA Dehydrogenase Deficiency: A Case Report**

Dr. Barbara Risj<sup>1,2</sup>

<sup>1</sup>NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy. <sup>2</sup>Department of Molecular and Translational Medicine, University of Brescia, Brescia, Italy

## **PP01.05 Late-Onset Acute Respiratory Failure Revealing SELENON-Related Myopathy: A Case Report**

Dr. Barbara Risj<sup>1,2</sup>

<sup>1</sup>NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy.

<sup>2</sup>Department of Molecular and Translational Medicine, University of Brescia, Brescia, Italy

## **PP01.06 Congenital Myasthenic Syndrome: 3 Different Phenotypes and Genotypes in 8 Moroccan Families**

Prof. Birouk Nazha<sup>1</sup>

<sup>1</sup>Department of Neurophysiology, hospital of specialities, university hospital Ibn Sina, Mohamed V university, Rabat, Morocco

## **PP01.07 Joint Contractures as Prominent Clinical Feature in Congenital Myopathy Related to K7del TPM2 Gene Mutation**

Prof. Birouk Nazha<sup>1</sup>

<sup>1</sup>Neurophysiology department, Hospital of specialities, Ibn Sina University Hospital, Mohamed V University, Rabat, Morocco

## **PP01.08 Proteomic Insights into Infantile Nemaline Myopathy Reveal Dysregulated Proteins and Cellular Pathways**

Assoc. Prof. Carola Hedberg-Oldfors<sup>1</sup>,

<sup>1</sup>Department of Laboratory Medicine, University of Gothenburg, Gothenburg, Sweden

## **PP01.09 Biallelic SNAPC4 Variants Associated with Lower Motor Neuron Disease**

Dr. Chiara Fiorillo<sup>1,2</sup>

<sup>1</sup>Department of Neuroscience, University of, Genova, Italy. <sup>2</sup>Pediatric Neurology, Gaslini Children Hospital, Genova, Italy

## **PP01.10 Expanding the Phenotypic Spectrum of FKTN Mutations: A Case of Generalized Epilepsy, Neurodevelopmental Delay, and HyperCKemia**

Prof. Chiara Fiorillo

IRCCS Giannina Gaslini, Genova, Italy

**PP01.11 Clinical and Genetic Characterization of Dominant COL12A1- Related Myopathies Across European Centers**

Prof. Chiara Fiorillo<sup>2,1</sup>

<sup>1</sup>Department of Neuroscience, University of Genova, Genova, Italy. <sup>2</sup>Unit of Paediatric Neurology and Muscle Disorders, IRCCS Istituto G.Gaslini, Genova, Italy

**PP01.12 MYBPC1-Related Congenital Myopathy With Tremor Associated With Cerebellar Atrophy and Adult-Onset Cardiac Involvement**

Dr. Cristina Muntean Firanescu<sup>1</sup>,

<sup>1</sup>Department of Neurology, Karolinska University Hospital, Stockholm, Sweden

**PP01.13 RYR1 Variants in Pediatric Myopathies: Clinical Diversity and Diagnostic Uncertainty**

Dr. Gordana Kovacevic<sup>1,2</sup>

<sup>1</sup>Neurology Department, Mother and Child Health Care Institute of Serbia "Dr Vukan Cupic", Belgrade, Serbia. <sup>2</sup>Faculty of Medicine, University of Belgrade, Belgrade, Serbia

**PP01.14 One-Year Follow-Up of Muscle MRI in LAMA2-Related Muscular Dystrophy and SELENON-Related Congenital Myopathy**

Ms. Ilse de Laat

Radboud university medical center, Nijmegen, Netherlands

**PP01.15 MEF2A Modulation as a Therapeutic Strategy for LAMA2-Related Muscular Dystrophy**

Dr. Julie Yue Yuan<sup>1</sup>,

<sup>1</sup>Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

**PP01.16 DNM2-Related Centronuclear Myopathy With Electrical Myotonia**

Prof. Jungim Suk

Department of Neurology, Catholic University of Daegu, School of Medicine, Daegu, Korea, Republic of

**PP01.17 Clinical Characteristics of Patients With Congenital Myopathies From a 2-Year Prospective Natural History Study (READYCOM)**

Ms. Sanne A.J.H. van de Camp<sup>1</sup>

<sup>1</sup>Radboud university medical center, Nijmegen, Netherlands

**PP01.18 Beyond Muscle: Anterior Horn Cell Involvement in The Phenotypic Spectrum of Collagen VI - Related Disorders**

Dr. Thainá Louise Rodrigues<sup>1</sup>

<sup>1</sup>Centro Universitário FMABC, Santo André, Brazil

**PP01.19 Exploring Novel Modifier Genes for LAMA2-RD by *in Vitro* and *in Vivo* studies**

Dr. Veronica Pini<sup>1,2</sup>

<sup>1</sup>Neuromuscular Repair Unit, Institute of Experimental Neurology, Ospedale San Raffaele, Milan, Italy. <sup>2</sup>Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

**PP01.20 Expanding the Phenotype of TUBA4A-Related Myopathy: Two Unrelated Korean Families**

Dr. Yunjung Choi<sup>1</sup>

<sup>1</sup>Department of Neurology, Sanggye Paik Hospital, Inje University College of Medicine, Seoul, Korea, Republic of

## **PP01.21 Characterization of an *In Vitro* C2c12 Stable Model for *plin4*-Related Myopathy**

Dr. Alessandra Carnazzi<sup>1</sup>

<sup>1</sup>Neuroimmunology and Neuromuscular Diseases Unit, Fondazione IRCCS Istituto Neurologico “Carlo Besta”, Milan, Italy

## **PP01.22 MicroRNA Profiling in Patients With *plin4*-Related Myopathy**

Ms. Eliana Iannibelli<sup>1</sup>,

<sup>1</sup>Neuroimmunology and Neuromuscular Diseases Unit – Fondazione IRCCS Istituto Neurologico Carlo Besta, Milan, Italy

## **PP01.23 Desmin-Related Myopathy in a Korean Patient with Distal Myopathy and Atrial Fibrillation**

Dr. Hyung Jun Park<sup>1</sup>

<sup>1</sup>Department of Neurology, Gangnam Severance Hospital, Yonsei University College of Medicine, Seoul, Korea, Republic of

## **PP01.24 Expansion of the Clinical and Genetic Spectrum of hnRNPA1-Associated Distal Myopathy in Two Korean Families**

Dr. Hyung Jun Park<sup>1</sup>

<sup>1</sup>Department of Neurology, Gangnam Severance Hospital, Yonsei University College of Medicine, Seoul, Korea, Republic of

## **PP01.25 Myotonia in PLIN4-Related Myopathy: Investigating the Role of Ion Channels**

Dr. Paola Laghetti<sup>1</sup>

<sup>1</sup>Section of Pharmacology, Department of Precision and Regenerative Medicine and Ionian Area, School of Medicine, University of Bari “Aldo Moro”, Bari, Italy

## **PP01.26 H2S-Based Strategies for Duchenne Muscular Dystrophy**

Assoc. Prof. Agnieszka Łoboda<sup>1</sup>

<sup>1</sup>Jagiellonian University, Kraków, Poland

## **PP01.27 Assessment of the Impact of Modifier Genes on the Clinical Course of Ambulatory DMD Patients**

Dr. Ana Kosac<sup>1,2</sup>

<sup>1</sup>Clinic of neurology and psychiatry for children and youth, Belgrade, Serbia. <sup>2</sup>School of Medicine, University of Belgrade, Belgrade, Serbia

## **PP01.28 Cell-Cell Communication Reveals Novel Mechanistic Insights into Duchenne Muscular Dystrophy**

Miss Annabel Sen,

UCLA, Los Angeles, United States

## **PP01.29 Automated Patch-Clamp Electrophysiology for Drug Discovery and Genotype-Phenotype Correlation in Skeletal Muscle and Pain Disorders**

Prof. Annamaria De Luca

Department of Pharmacy-Drug Sciences, University of Bari, Bari, Italy

## **PP01.30 Dysregulation of LKB1 in Duchenne Muscular Dystrophy: Disease Specificity and Epigenetic Regulation by HDAC Inhibitors**

Prof. Annamaria De Luca<sup>1</sup>

<sup>1</sup>Department of Pharmacy – Drug Sciences, University of Bari “Aldo Moro”, Bari, Italy

## **PP01.31 Patient-Derived Skeletal Muscle-on-Chip as a Platform for Pre-Clinical Studies in Duchenne Muscular Dystrophy**

Prof. Annamaria De Luca<sup>1</sup>

<sup>1</sup>Department of Pharmacy – Drug Sciences, University of Bari “Aldo Moro”, Bari, Italy

**PP01.32 Altered Sphingomyelin and Phosphocholine Profiles in Duchenne Cardiac Organoids**

Ms. Carolina Ferri<sup>2</sup>

<sup>2</sup>University of Pisa, Pisa, Italy. <sup>3</sup>Istituto di Fisiologia Clinica, Consiglio Nazionale delle Ricerche, Pisa, Italy

**PP01.33 Use of Psychopharmacological Treatments in Children and Adolescents With Duchenne Muscular Dystrophy: A Single-Centre Experience**

Dr. Chiara Brusa<sup>1,2</sup>

<sup>1</sup>Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom. <sup>2</sup>Dubowitz Neuromuscular Centre, Great Ormond Street Hospital for Children, London, United Kingdom

**PP01.34 Expanding the Phenotypic Spectrum of SYT2-Related Disorders: A Case Report of Distal Weakness and Fatigue**

Dr. Chiara Panicucci<sup>4</sup>

<sup>4</sup>Unit of Pediatric Neurology, IRCCS Istituto Giannina Gaslini, Genoa, Italy

**PP01.35 Efficacy and Safety of Neridronate for the Treatment of Fragility Fractures in Duchenne Muscular Dystrophy**

Dr. Chiara Panicucci<sup>1</sup>

<sup>1</sup>Centre of Translational and Experimental Myology, IRCCS Istituto Giannina Gaslini, Genova, Italy

**PP01.36 Prospective Evaluation of Sleep-Disordered Breathing and Functional Assessment in Duchenne Muscular Dystrophy: DystREST Study**

Dr. Chiara Panicucci<sup>5</sup>

<sup>5</sup>Centre of Translational and Experimental Myology, IRCCS Istituto Giannina Gaslini, Genova, Italy

**PP01.37 Preclinical Efficacy of ENTR-601-51 for the Treatment of Exon 51 Skip-Amenable Duchenne Muscular Dystrophy**

Dr. Christopher M. Brennan,

Entrada Therapeutics, Boston, United States

**PP01.38 Psychometric Validation of the DuMAND Checklist in Boys With Duchenne Muscular Dystrophy: A Belgian Cohort**

Ms. Eline Cuveele<sup>1,2</sup>

<sup>1</sup>University Hospitals Leuven, Leuven, Belgium.

<sup>2</sup>KU Leuven, Leuven, Belgium

**PP01.39 The DuMAND Checklist: Behavioral Patterns in Typically Developing Boys and Boys with Duchenne Muscular Dystrophy**

Ms. Eline Cuveele<sup>1,2</sup>

<sup>1</sup>University Hospitals Leuven, Leuven, Belgium.

<sup>2</sup>KU Leuven, Leuven, Belgium

**PP01.40 Mapping the Mind in Duchenne Muscular Dystrophy: The DuMAND Network**

Ms. Eline Cuveele<sup>1,2</sup>

<sup>1</sup>University Hospitals Leuven, Leuven, Belgium.

<sup>2</sup>KU Leuven, Leuven, Belgium

**PP01.41 Efficacy and Safety of Golodirsen and Casimersen Compared With Placebo in Duchenne Muscular Dystrophy (ESSENCE)**

Prof. Eugenio Mercuri<sup>11</sup>

<sup>11</sup>Pediatric Neurology Unit, Università Cattolica del Sacro Cuore Roma, Rome, Italy

**PP01.42 Genetic Modifier Associations With Steroid Safety Outcomes in Duchenne Muscular Dystrophy**

Ms. Fatemeh Ahmadiharchegani<sup>1</sup>,

<sup>1</sup>Carleton University, Ottawa, Canada

## **PP01.43 Real-World Ataluren Outcomes in 7 nmDMD Patients**

Prof. Flávia Nardes

UFRJ, Rio de Janeiro, Brazil

## **PP01.44 Characterisation of Anti-AAV9 Seropositivity in Zolgensma Treated SMA Patients**

Prof. Francesco Muntoni<sup>1</sup>

<sup>1</sup>University College London, London, United Kingdom

## **PP01.45 Neuronal Micro-Dystrophin Gene Therapy Improves Emotional Behaviour and Restores Dystrophin Interactors in *mdx52* mouse Model**

Prof. Francesco Muntoni<sup>1,2</sup>

<sup>1</sup>Genetic Therapy Accelerator Centre, UCL Queen Square Institute of Neurology, London, United Kingdom. <sup>2</sup>Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

## **PP01.46 Phase 2 Study of Vesteteplirsen in Patients With Duchenne Muscular Dystrophy: MOMENTUM Part B Results**

Dr. Giovanni Baranello<sup>11,12</sup>

<sup>11</sup>Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health Great Ormond Street Hospital NHS Foundation Trust, London, United Kingdom. <sup>12</sup>National Institute for Health Research Great Ormond Street Hospital Biomedical Research Centre, London, United Kingdom

## **PP01.47 SMAD6 Heterozygous Loss-of-Function Variant as a Potential Modifier of Duchenne Muscular Dystrophy (DMD)**

Dr. Giulio Gadaleta

Neuromuscular Unit, Department of Neuroscience "Rita Levi Montalcini", University of Turin, Turin, Italy

## **PP01.48 Patient Experiences With DMD and Impacts of PMO Therapies - Interviews With Caregivers From REAL-DMD**

Mrs Ivana F. Audhya<sup>1</sup>

<sup>1</sup>Sarepta Therapeutics, Inc, Cambridge, MA, United States

## **PP01.49 Digital Transformation of Korean Articulation and Phonology Assessment: Reliability and Validity Study of New Instrument**

Asst. Prof. Jee Hyun Suh<sup>1</sup>

<sup>1</sup>Seoul National University Bundang Hospital, Seongnam-si, Korea, Republic of

## **PP01.50 Management of Hip Subluxation Using Hip Bracing in Patients with Fukuyama Congenital Muscular Dystrophy**

Asst. Prof. Jee Hyun Suh

Seoul National University Bundang Hospital, Seongnam-si, Korea, Republic of

## **PP01.51 Considerations for Assessing Efficacy in Steroid-Naïve Interventional Trials in Duchenne Muscular Dystrophy**

Ms. Kaitey Guite<sup>1</sup>

<sup>1</sup>Carleton University, Ottawa, Canada

## **PP01.52 Long-Term Real-World Outcomes Following Onasemnogene Apeparovect Monotherapy for Patients With SMA: Updated Findings From RESTORE**

Prof. Laurent Servais<sup>1,2</sup>

<sup>1</sup>Department of Paediatrics, MDUK Oxford Neuromuscular Centre & NIHR Oxford Biomedical Research Centre, University of Oxford, Oxford, United Kingdom. <sup>2</sup>Department of Pediatrics, Neuromuscular Reference Center, University and University Hospital of Liège, Liège, Belgium

# Poster Listing

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## **PP01.53 ENTR-601-44-201: First-in-Patient Results From a Phase 1/2 Study Evaluating ENTR-601-44 in Exon 44 Skip-Amenable DMD**

Dr. Laurent Servais<sup>1,2</sup>

<sup>1</sup>Department of Paediatrics, MDUK Oxford Neuromuscular Centre & NIHR Oxford Biomedical Research Centre, University of Oxford, Oxford, United Kingdom. <sup>2</sup>Department of Paediatrics, Neuromuscular Reference Centers, University Hospital of Liège, Liège, Belgium

## **PP01.54 Effects of NP12 Treatment on Skeletal and Cardiac Muscle in Thermoneutrally Housed Mdx Mice**

Mr. Luc Wasilewicz<sup>1</sup>

<sup>1</sup>Brock University, St.Catharines, Canada

## **PP01.55 Development of the Duchenne Muscular Dystrophy Natural History Italian Registry: Next-Generation EU-PNRR Study**

Dr. Luca Kunstler<sup>1</sup>

<sup>1</sup>Fondazione Policlinico Universitario Agostino Gemelli IRCCS, Rome, Italy

## **PP01.56 Patient Characteristics and Use of Vamorolone in Adult Patients With DMD in Germany and Austria**

Dr. Marcus Erdler<sup>1</sup>

<sup>1</sup>Klinik Donaustadt, Dept. of Neurology, Vienna, Austria

## **PP01.57 The DMD-NEEDS Study: Real-World Management of Duchenne Muscular Dystrophy in Spain.**

Mrs Maria Branas<sup>2</sup>

<sup>2</sup>Roche Farma, Madrid, Spain

## **PP01.58 Clinical and Genetic Characterization of Duchenne Muscular Dystrophy in a Chilean Cohort**

Asst. Prof. Maria de los Angeles Beytia<sup>1,2</sup>

<sup>1</sup>Pontificia Universidad Católica de Chile, División de Pediatría, Unidad de neurología pediátrica, Santiago, Chile. <sup>2</sup>Hospital Dr. Sótero del Río, Unidad de Neurología pediátrica, Santiago, Chile

## **PP01.59 FORZETTO, a Phase 3 Study of Z-Rostudirsén in Ambulatory Males With Exon 51 Amenable DMD**

Dr. Maria Naylor

Dyne Therapeutics, Waltham, United States

## **PP01.60 RNA-Based Combinatorial Multi-Target Therapy for the Treatment of Duchenne Muscular Dystrophy**

Dr. Mathieu Horeau<sup>1,2</sup>

<sup>1</sup>Veneto Institute of Molecular Medicine, Padova, Italy. <sup>2</sup>Università di Padova, Department of Biomedical Sciences, Padova, Italy

## **PP01.61 Bone-Health and Fracture Risk in FSHD1: A 12-Months Longitudinal Multi-Modal Imaging and Biomarker Study**

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<sup>1</sup>Laboratory for Muscle Diseases and Neuropathies, Department of Neurosciences, KU Leuven, and Leuven Brain Institute (LBI), Leuven, Belgium. <sup>2</sup>Department of Neurology, University Hospitals Leuven, Leuven, Belgium

## **PP01.62 Multimodal Assessment of Bone Health and Functional Status in Adults With Duchenne Muscular Dystrophy**

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**PP01.63 Volumetric Brain Differences in Duchenne Muscular Dystrophy: What Happens When Accounting for Puberty?**

Mr. Matthias Schelfhout<sup>1</sup>

<sup>1</sup>Department of Development and Regeneration, KU Leuven, Leuven, Belgium

**PP01.64 Outlining the Neuroimaging Landscape in Duchenne and Becker Muscular Dystrophy: A Systematic Review**

Mr. Matthias Schelfhout<sup>1</sup>

<sup>1</sup>Department of Development and Regeneration, KU Leuven, Leuven, Belgium

**PP01.65 A New Beginning for Duchenne: Genomic Newborn Screening and Integrated Models of Early Care**

Dr. Michelle Lorentzos<sup>1,2</sup>

<sup>1</sup>Sydney Children's Hospitals Network, Sydney, Australia. <sup>2</sup>The University of Sydney, Sydney, Australia

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Dr. Miriam Hiebeler<sup>1</sup>,

<sup>1</sup>Friedrich-Baur-Institute, LMU, Munich, Germany

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Dr. Paolo Bettica<sup>3</sup>

<sup>3</sup>Italfarmaco S.p.A., Milan, Italy

**PP01.68 Update on the INSPIRE DUCHENNE Phase 1/2 Study of SGT-003 Microdystrophin Gene Therapy**

Prof. Perry Shieh<sup>1</sup>

<sup>1</sup>University of California, Los Angeles, Los Angeles, United States

**PP01.69 A Novel Tandem Quadruplication of Exons 8-40 in the DMD Gene: Phenotype and Genotype Characterization**

Prof. Sonia Nouioua<sup>1</sup>

<sup>1</sup>Neurology Department, Cherchell Hospital, Chercherll, Tipaza, Algeria

**PP01.70 Xp21 Contiguous Gene Deletion Syndrome Presenting as Atypical Duchenne Muscular Dystrophy With Adrenal Insufficiency**

Prof. Sonia Nouioua<sup>1</sup>

<sup>1</sup>Department of neurology EHS Cherchell, Tipaza, Algeria

**PP01.71 Zeleciment Rostudirsen Increased Dystrophin and Led to Functional Improvement in Clinical Measures in DELIVER Trial**

Dr. Stefano Previtali<sup>1,2</sup>

<sup>1</sup>IRCCS San Raffaele Scientific Institute, Milan, Italy. <sup>2</sup>Vita-Salute San Raffaele University, Milan, Italy

**PP01.72 Real-World Experience With Gene Therapy in Duchenne Muscular Dystrophy: Experience From Qatar.**

Prof. Tawben Omran

Sidra Medicine, Doha, Qatar

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Assoc. Prof. Utkarsh Dang<sup>1</sup>

<sup>1</sup>Carleton University, Ottawa, Canada

**PP01.74 Open-Label Extension Analysis Suggests Givinstat Delays Age at Loss of Ambulation in Patients With DMD**

Prof. Valeria Sansone<sup>2</sup>

<sup>2</sup>The NeMO Clinical Center in Milan, Neurorehabilitation Unit, Milan, Italy

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Prof. Yoshihide Sunada<sup>1</sup>

<sup>1</sup>Kawasaki Medical School, Kurashiki, Japan

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Prof. Eren Gozke<sup>2</sup>

<sup>2</sup>University of Health Sciences FSM Training and Research Hospital, Istanbul, Turkey

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Prof. John Vissing<sup>1</sup>

<sup>1</sup>Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark

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Prof. John Vissing<sup>1</sup>

<sup>1</sup>Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark

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Dr. John Vissing<sup>6</sup>

<sup>6</sup>University of Copenhagen, Copenhagen, Denmark

## **PP01.81 Myasthenia Gravis Inebilizumab Trial (MINT): Efficacy and Pharmacodynamics in AChR+ Cohort (Week 52)**

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<sup>6</sup>University of Copenhagen, Copenhagen, Denmark

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Dr. John Vissing<sup>3</sup>

<sup>3</sup>Department of Neurology, Copenhagen Neuromuscular Center, Copenhagen, Denmark

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Prof. Lea Leonaridis<sup>1</sup>

<sup>1</sup>Institute of Clinical Neurophysiology, University Medical Centre Ljubljana, Ljubljana, Slovenia

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<sup>1</sup>Neuromuscular Unit, Department of Neurosciences "Rita Levi Montalcini", University of Turin, Turin, Italy

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Prof. Tsuyoshi Matsumura<sup>1</sup>

<sup>1</sup>NHO Osaka Toneyama Medical Center, Toyonaka, Japan

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<sup>1</sup>Sapienza University of Rome, Nesmos Departement, Rome, Italy

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<sup>1</sup>Internal Medicine Trainee 2, Royal Devon and Exeter Hospital, Royal Devon University Healthcare NHS Foundation Trust, Exeter, United Kingdom

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[Dr. Chafic Karam<sup>2</sup>](#)

<sup>2</sup>Perelman School of Medicine, Philadelphia, United States

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<sup>4</sup>University of Pennsylvania, Perelman School of Medicine, Pennsylvania, United States

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[Dr. Giorgia Riolo<sup>1,2</sup>](#)

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[Dr. Hyunjin Kim<sup>1</sup>](#)

<sup>1</sup>Department of Neurology, Asan Medical Center, University of Ulsan College of Medicine, Seoul, Korea, Republic of

**PP01.98 Anti-HMGCR Myopathy in Latvia: Epidemiology and National Prevalence Data**

Dr. Marija Roddate<sup>1,2</sup>

<sup>1</sup>Pauls Stradins Clinical University Hospital, Department of Neurology, Riga, Latvia. <sup>2</sup>Riga Stradins University, Riga, Latvia

**PP01.99 Evaluating ChatGPT's advice and recommendations regarding exercise for people with Inclusion Body Myositis**

Prof. Merrilee Needham<sup>2,3,4,6</sup>

<sup>2</sup>Notre Dame University, Fremantle, Australia. <sup>3</sup>Perron Institute, Nedlands, Australia. <sup>4</sup>Murdoch University, Murdoch, Australia. <sup>5</sup>Neuroscience Research Australia, Rendwick, Australia. <sup>6</sup>Fiona Stanley Hospital, Murdoch, Australia

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Dr. Michele Giovanni Croce<sup>3</sup>

<sup>3</sup>Department of Brain and Behavioural Sciences, University of Pavia, Pavia, Italy

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Dr. Nicole Rigler

Mayo Clinic Department of Neurology, Rochester, United States

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Dr. Nobuyuki Eura

Nara Medical University, Kashihara, Japan

**PP01.103 Multimodality Testing in Patients Suspected of Treatable Idiopathic Inflammatory Myopathy. A Prospective Diagnostic Accuracy Study (ADAPT-Study)**

Miss Pinar Özkaynar<sup>1</sup>

<sup>1</sup>Amsterdam UMC, department of neurology, Amsterdam, Netherlands

**PP01.104 A Novel Cohort of Italian Patients With Late-Onset Pompe Disease and Extended IVS1-32-13T>G Screening**

Prof. Stefania Corti<sup>1,3</sup>

<sup>1</sup>IRCCS Foundation Ca' Granda Ospedale Maggiore Policlinico, Neuromuscular and Rare Disease Unit, Milan, Italy. <sup>3</sup>Dino Ferrari Center, Department of Pathophysiology and Transplantation, University of Milan, Milan, Italy

**PP01.105 Anti-HMGCR Immune-Mediated Necrotizing Myopathy: Characterization of a Monocenter Cohort and Development of a Therapeutic Algorithm**

Prof. Stefania Corti<sup>4,5</sup>

<sup>4</sup>IRCCS Foundation Ca' Granda Ospedale Maggiore Policlinico, Neuromuscular and Rare Disease Unit, Milan, Italy. <sup>5</sup>Dino Ferrari Center, Department of Pathophysiology and Transplantation, University of Milan, Milan, Italy

**PP01.106 Solid Facial and Palpebral Edema: Amyopathic Dermatomyositis, Morbihan Disease?**

Dr. Zoltan Zsigmond Major<sup>1,2</sup>

<sup>1</sup>National Center for Spinal Disorders, Budapest, Hungary. <sup>2</sup>Municipal Clinical Hospital, Neurology Department, Cluj-Napoca, Romania

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Prof. Corrado Angelini<sup>1</sup>

<sup>1</sup>University of Padova, Padova, Italy

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Dr. Douglas Sproule<sup>5</sup>

<sup>5</sup>ML Bio Solutions Inc., a BridgeBio company, Palo Alto, United States

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<sup>1</sup>Department of Clinical Neurophysiology, AHEPA University Hospital Aristotle University of Thessaloniki, Thessaloniki, Greece

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<sup>1</sup>University of Ljubljana, Ljubljana, Slovenia

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Dr. Audrey Cambier<sup>1</sup>

<sup>1</sup>Centre de Référence des maladies Neuromusculaires Nord/Est/Ile-de-France, Institut de Myologie, Hôpital Pitié-Salpêtrière, APHP, Paris, France

## **PP01.114 Real-world Outcomes of Very Early-treated Infantile-onset Pompe Disease in Taiwan After Switching to Avalglucosidase Alfa**

Dr. Chia-Feng Yang<sup>1,2</sup>

<sup>1</sup>Department of Pediatrics, Taipei Veterans

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Dr. Diana Chitimus<sup>1,2,3</sup>

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Prof. Enzo Stinghel Pellacani<sup>8</sup>

<sup>8</sup>ABC Medical School, São Paulo, Brazil

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Prof. Giacomo Pietro Comi<sup>1,4</sup>

<sup>1</sup>Neurology Unit, IRCCS Fondazione Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy. <sup>4</sup>Dino Ferrari Center, Department of Pathophysiology and Transplantation, University of Milan, Milan, Italy

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Neurology and Neurophysiology Center, Vienna, Austria

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Prof. Josef Finsterer

Neurology and Neurophysiology Center, Vienna, Austria

**PP01.123 Danon Disease in Japan: An Updated Nationwide Clinicogenetic Analysis**

Prof. Kazuma Sugie<sup>1,2,3</sup>

<sup>1</sup>Department of Neurology, Nara Medical University, Nara, Japan. <sup>2</sup>Center for Autophagy and Anti-Aging Research, Nara Medical University, Nara, Japan. <sup>3</sup>Department of Neuromuscular Research, National Institute of Neurology, National Center of Neurology and Psychiatry, Tokyo, Japan.

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Dr. Leila Tamaoui<sup>2</sup>

<sup>2</sup>Clinical Neurophysiology Department Hôpital des Spécialités of Rabat / Ibn Sina University Hospital Center, Rabat, Morocco

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Asst. Prof. Leila Tamaoui<sup>1,2</sup>

<sup>1</sup>Clinical Neurophysiology Department, Hôpital des Spécialités of Rabat, Ibn Sina University Hospital, Rabat, Morocco. <sup>2</sup>Medical school of Mohammed V University, Rabat, Morocco

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Asst. Prof. Leila Tamaoui<sup>1,2</sup>

<sup>1</sup>Clinical Neurophysiology Department, Hôpital des Spécialités of Rabat, Ibn Sina University Hospital, Rabat, Morocco. <sup>2</sup>Medical school of Mohammed V University, Rabat, Morocco

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Prof. Olimpia Musumeci

University of Messina, Messina, Italy

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Dr. Sofie Sunebo<sup>1,2</sup>

<sup>1</sup>Department of Biomedical and Clinical Sciences, Linköping University, Linköping, Sweden. <sup>2</sup>Clinical Department of Neurology in Linköping, Region Östergötland, Linköping, Sweden

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Dr. Kiran Polavarapu<sup>1</sup>

<sup>1</sup>CHEO Research Institute, Ottawa, Canada

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Prof. Kristl Claeys<sup>1,2</sup>

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Dr. Mathias Maisenbacher

Department of Neurological Sciences, University of Nebraska Medical Center, Omaha, United States

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, Prof. Pascal Laforêt<sup>10</sup>

<sup>10</sup>Nord-Est/Ile-de-France Neuromuscular Reference Center, UMR 1179, Neurology Department, Raymond-Poincaré Hospital, Garches, FHU PHENIX, UVSQ Paris-Saclay University, Garches, France

**PP01.133 HARMONY360 Study for Comprehensive Monitoring of Generalized Myasthenia Gravis With Passive and Active Digital Biomarkers**

Prof. Pascal Laforêt<sup>11</sup>

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University Of North Carolina, Chapel Hill, United States

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Miss Caterina M. Wendel<sup>1</sup>

<sup>1</sup>Friedrich Baur Institute at the Department of Neurology, LMU University Hospital, Munich, Germany

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Prof. Elise Duchesne<sup>1,2,3,4</sup>

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BioMarin, San Rafael, CA, United States

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Prof. Nalini Atchayaram

National Institute of mental Health and Neurosciences, Bangalore, India

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Prof. Nalini Atchayaram<sup>1</sup>

<sup>1</sup>National Institute of Mental Health and Neurosciences, Bangalore, India

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Dr. Rui Shimazaki<sup>1,2</sup>

<sup>1</sup>Nara Medical University, Kashihara, Japan.  
<sup>2</sup>National Center of Neurology and Psychiatry, Kodaira, Japan

**PP01.141 Progression in Myotonic Dystrophy Type 1; Following Up of Patients With Myotonic Dystrophy Type 1**

Dr. Andrea Sipos<sup>1</sup>

<sup>1</sup>UPMS, Pécs, Hungary

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Dr. Douglas Kerr

Dyne Therapeutics, Waltham, United States

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Prof. Dusanka Savic-Pavicevic<sup>1</sup>

<sup>1</sup>University of Belgrade-Faculty of Biology, Center for Human Molecular Genetics, Belgrade, Serbia.

**PP01.144 Cognitive and Behavioral Impairment in Myotonic Dystrophy Type 2: Evidence From ECAS and Plasma Biomarkers**

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**PP01.145 Efficacy and Safety of Metformin in Myotonic Dystrophy Type 1: The MetMyd Study**

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**PP01.146 Neurofilament Light Chain as a Surrogate Marker for Motor Functions in Myotonic Dystrophy Type 1**

Assoc. Prof. Jin-Sung Park<sup>1</sup>

<sup>1</sup>Kyungpook National University Chilgok Hospital, Daegu, Korea, Republic of

**PP01.147 Gynecological Disorders in Women With Myotonic Dystrophy Type 1: A Retrospective Study**

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**PP01.148 Oculopharyngeal Muscular Dystrophy in a Korean Family with a Pathogenic PABPN1 Gene Duplication**

Prof. Hyunjin Ju<sup>1</sup>

<sup>1</sup>Department of Neurology, Soonchunhyang University Seoul Hospital, Soonchunhyang University College of Medicine, Seoul, Korea, Republic of

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<sup>1</sup>Myongji Hospital, Goyang, Korea, Republic of

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Dr. Dipti Baskar<sup>1</sup>

<sup>1</sup>National Institute of Mental Health and Neuro Sciences (NIMHANS), Bengaluru, India

**PP01.151 Occult and Overt Myopathy in Adult Cystinosis: Clinical Characterization and Longitudinal Assessment**

Dr. Edouard Berling<sup>1</sup>

<sup>1</sup>Service de Neurologie, AP-HP, Hôpital Raymond Poincaré, Centre de référence Nord-Est-Ile-de-France, Garches, France

**PP01.152 Fbxo30/MUSA1 is a Novel Critical Regulatory Element for Z-Line Homeostasis and Skeletal Muscle Function**

Miss Martina Esposito<sup>1,2</sup>

<sup>1</sup>Università degli Studi di Padova, Department of Biomedical Sciences, Padova, Italy. <sup>2</sup>Veneto Institute of Molecular Medicine, Padova, Italy

**PP01.153 Amyloid Myopathy: An Underrecognized but Potentially Treatable Condition**

Dr. Carolina Azcona

Hospital Italiano, Ciudad Autonoma de Buenos Aires, Argentina

**PP01.154 Peripheral Nerve Involvement in Biallelic SCA27B: A Multicenter Study**

Dr. Sara Nagy<sup>1,2</sup>

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**PP01.155 Zilucoplan as Rescue Therapy in Refractory Myasthenia Gravis Exacerbations**

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**PP01.156 Phase 1b Study of Safety and Efficacy of Adimanebart (ARGX-119) in DOK7 Congenital Myasthenic Syndromes**

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**PP01.157 Association Between Measured Variables and Family History of Neuromuscular Disease in Adults Using Macedonian INQoL**

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**PP01.159 Safety and Efficacy of Claseprubart, an Active C1s Inhibitor, in Patients with Generalized Myasthenia Gravis**

Dr. Shahar Shelly<sup>1</sup>

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Dr. Shahar Shelly<sup>1</sup>

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Prof. Lore Mariën<sup>9</sup>

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**PP01.167 The First Korean Case of Congenital Myasthenic Syndrome 10 Responsive to Ephedrine Therapy**

Mr. Sangwon Joe<sup>1</sup>,

<sup>1</sup>Department of Neurology, Gangnam Severance Hospital, Yonsei University College of Medicine, Seoul, Korea, Republic of

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Mr. Syed Raza<sup>1</sup>

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Dr. Ali A. Habib<sup>4</sup>

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Assoc. Prof. Ali Habib<sup>1</sup>

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Prof. Anneke Van der Walt<sup>1,2</sup>

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## **PP01.175 Long-Term Safety and Efficacy of Nipocalimab: Approximately 2 Years Follow-Up Results From Vivacity-MG3 Open-Label Extension**

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## **PP01.176 Long-Term Safety and Efficacy of Subcutaneous Efgartigimod PH20 for Generalized Myasthenia Gravis: ADAPT-SC+ Final Results**

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## **PP01.180 Morphological and Quantitative Analysis of Neuromuscular Junction Pathology in AChR Antibody-Positive Myasthenia Gravis**

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<sup>1</sup>Neurologia Śląska Centrum Medyczne, Katowice, Poland

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## **PP01.208 Assessing Relationship Between Immunoglobulin-G Level and Nipocalimab Efficacy using Myasthenia Gravis-Activities of Daily Living Scale**

Dr. Marie Fitzgibbon<sup>5</sup>

<sup>5</sup>Johnson & Johnson, Raritan, NJ, United States

## **PP01.209 Efficacy and Safety of Nipocalimab: Switch From 15-mg/kg Every-2-Week to 30-mg/kg Every-4-Week (VIVACITY-MG3 Open-Label Extension)**

Dr. Marie Fitzgibbon<sup>2</sup>

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## **PP01.210 Corticosteroid Usage in Patients with Generalized Myasthenia Gravis Receiving Nipocalimab: Phase 3 Vivacity-MG3 Open-Label Extension**

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Dr. Mazen Dimachkie<sup>1</sup>

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**PP01.214 Characterisation of Patients With Generalised Myasthenia Gravis on Zilucoplan Treatment in European Managed Access Programmes**

Dr. Natasa Savic<sup>1</sup>

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Dr. Nicholas Silvestri<sup>2</sup>

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Dr. Niklas Huntemann<sup>1</sup>

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**PP01.218 Efgartigimod Provides Rapid Clinical Improvements and Reduces Corticosteroid Use in gMG: Initial Results From PREMIER**

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Dr. Rebecca Leung<sup>1,2,3</sup>

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Dr. Sabrina Guye<sup>1</sup>

<sup>1</sup>UCB, Zurich, Switzerland.

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Prof. Stephen Reddel<sup>1</sup>

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Dr. Thaïs Tarancón<sup>1</sup>

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Dr. Valentina Vera<sup>1</sup>

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Assoc. Prof. Andreas Roos<sup>1</sup>

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Dr. Francesco Guosso<sup>1,2,3</sup>

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Dr. Mariangela Goglia<sup>1</sup>

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Dr. Otto Hernandez Fustes<sup>1,2</sup>

<sup>1</sup>Federal University of Paraná, Curitiba, Brazil. <sup>2</sup>Clinics Complex Hospital at Federal University of Paraná, Curitiba, Brazil

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<sup>2</sup>Universidade Federal do Paraná, Curitiba, Brazil. <sup>4</sup>Hospital de Reabilitação, Curitiba, Brazil

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## **PP01.255 Late-Onset Hereditary Transthyretin Amyloidosis (Attr) with Polyneuropathy in an Elderly Person**

Dr. Otto Hernandez Fustes<sup>1,2</sup>

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Department of Neurological Sciences, University of Nebraska Medical Center, Omaha, NE, United States

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Prof. Paulo José Lorenzoni

Universidade Federal do Paraná, Curitiba, Brazil

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Prof. Sandra Milena Castellar-Leones<sup>1,2,3</sup>

<sup>1</sup>Universidad Nacional de Colombia, Bogota, Colombia. <sup>2</sup>CIFEL, Bogota, Colombia. <sup>3</sup>Hospital Universitario Nacional de Colombia, Bogota, Colombia

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Dr. Tarin Europa<sup>1</sup>

<sup>1</sup>University of Cape Town, Cape Town, South Africa

## **PP01.260 Focal CIDP Variants with Brachial Plexus Involvement Compared to Neuralgic Amyotrophy – Similarities and Differences**

Assoc. Prof. Alexander Grimm<sup>1</sup>

<sup>1</sup>University hospital of Neurology, Tübingen, Germany

## **PP01.261 Riliprubart in CIDP: Time and Magnitude of Response Analysis from a Phase 2 Trial**

Dr. Annie Dionne<sup>1</sup>

<sup>1</sup>CHU de Quebec Universite Laval, Quebec, Canada

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Dr. Anthony A. Amato<sup>2</sup>,

<sup>2</sup>Brigham and Women's Hospital Harvard Medical School, Boston, United States

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Sunshine Coast Health, Sunshine Coast, Australia. Griffith University, Sunshine Coast, Australia

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Assoc. Prof. Antony Winkel<sup>1,2</sup>

<sup>1</sup>Sunshine Coast Health, Sunshine Coast, Australia. <sup>2</sup>Griffith University, Sunshine Coast, Australia

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Dr. Asif Paker<sup>1</sup>

<sup>1</sup>Sanofi R&D, Neurology Development, Cambridge, MA, United States

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Dr. Claudia Sommer<sup>2</sup>

<sup>2</sup>Neurologische Klinik und Poliklinik, Universitätsklinikum Würzburg, Würzburg, Germany

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Prof. Eduardo Nobile-Orazio<sup>8</sup>

<sup>8</sup>Università degli Studi di Milano, Milan, Italy

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<sup>4</sup>University of Milan, Milan, Italy

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Dr. Elke Schipani

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**PP01.271 Immune-mediated Sensory Predominant Polyradiculoneuropathy Initially Mimicking Myelopathy**

Dr. Eunbyol Hwang<sup>1</sup>

<sup>1</sup>Department of Neurology, Incheon St. Mary's Hospital, College of Medicine, The Catholic University of Korea, Seoul, Korea, Republic of

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Prof. Gwendal Le Masson<sup>3</sup>

<sup>3</sup>Department of Neurology (Nerve-Muscle Unit), AOC National Reference Center for Neuromuscular Disorders, ALS Center, University Hospital of Bordeaux (CHU Bordeaux), Bordeaux, France

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Prof. Ha Young Shin<sup>2</sup>

<sup>2</sup>Department of Neurology, Yonsei University College of Medicine, Seoul, Korea, Republic of

**PP01.274 CASE REPORT: Chronic Inflammatory Demyelinating Polyneuropathy Initially Misdiagnosed as Subacute Paraneoplastic Polyneuropathy**

Dr. Ieva Glāzere<sup>1,2,3</sup>

<sup>1</sup>Pauls Stradins Clinical University Hospital, Department of Neurology, Centre of Rare Neurological Disorders, Riga, Latvia. <sup>2</sup>Riga Stradins University, Department of Biology and Microbiology, Riga, Latvia. <sup>3</sup>Pauls Stradins Clinical University Hospital, Department of Neurology, Laboratory of Neurophysiology, Riga, Latvia

**PP01.275 The Spectrum of Polyneuropathies and Polyradiculoneuropathies Requiring Recurrent Inpatient Admissions Over a 9-Year Period**

Dr. Inga Suna<sup>1,2</sup>

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Prof. Jee-Eun Kim

Soonchunhyang University Bucheon Hospital, Seoul, Korea, Republic of

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Prof. Joong-Yang Cho

Department of Neurology, Inje University College of Medicine, Ilsan Paik Hospital, Goyang, Korea, Republic of

**PP01.278 Intravenous Immunoglobulin to Subcutaneous Efgartigimod PH20 Transition in CIDP: A Phase 4 Study in Progress**

Dr. Katerina Anokhina<sup>3</sup>

<sup>3</sup>Argenx, Ghent, Belgium

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Dr. Kristl G. Claeys<sup>1,2</sup>

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Cork University Hospital, Cork, Ireland

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Dr. Maria Kovalchuk

Dmitry Rogachev National Medical Research Center Of Pediatric Hematology, Oncology and Immunology., Moscow, Russia

**PP01.282 Long-Term Safety and Efficacy Data of Empasiprubarb in Multifocal Motor Neuropathy: Phase 2 ARDA+ Study**

Assoc. Prof. Pietro E. Doneddu<sup>6</sup>

<sup>6</sup>IRCCS Humanitas Research Hospital, University of Milan, Milan, Italy

**PP01.283 Telemedicine-Based INCAT Assessment in CIDP: Reliability and Agreement Between Nurse and Neuromuscular Expert Virtual Evaluations**

Dr. Pietro Emiliano Doneddu<sup>1,2</sup>

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**PP01.284 Empasiprubarb Versus Placebo (EMNERGIZE) or Immunoglobulin (EMVIGORATE) in Chronic Inflammatory Demyelinating Polyradiculoneuropathy: Study Designs**

Dr. Pietro Emiliano Doneddu<sup>3,4</sup>

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**PP01.285 ORBIT-CIDP: A Real-World CIDP Study Leveraging Digital and AI-Powered Patient-Centric Innovation**

Dr. Pietro Emiliano Doneddu<sup>5,6</sup>

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Asst. Prof. Sophie Teng<sup>1</sup>

<sup>1</sup>University of California, Sacramento, United States

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Dr. Stephan Goedee<sup>1</sup>

<sup>1</sup>Brain Center Rudolph Magnus, University Medical Center Utrecht, Utrecht, Netherlands

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Dr. Daryl Yin Keong Lo<sup>1</sup>

<sup>1</sup>Department of Neurology, National Neuroscience Institute (Singapore General Hospital Campus), Singapore, Singapore

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<sup>3</sup>Scientific and Practical Psychoneurological Center named after Z. P. Solovyov, Moscow, Russia

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Dr. Benedetta Tierro<sup>1</sup>

<sup>1</sup>Neurology Unit, Department of Neurosciences, University of Padova, Padova, Italy

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Mrs Liubov Pikus<sup>1</sup>

<sup>1</sup>Moscow Multidisciplinary Clinical Center «Kommunarka», Moscow, Russia

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Ulsan University Hospital, Ulsan, Korea, Republic of

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Dr. Tanish Modi<sup>1</sup>

<sup>1</sup>Mayo Clinic, Rochester, United States

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<sup>1</sup>St Vincent's Hospital, Melbourne, Australia.

<sup>2</sup>The University of Melbourne, Melbourne, Australia

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Prof. Hans Katzberg<sup>1</sup>

<sup>1</sup>University of Toronto, Toronto, Canada

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<sup>1</sup>Neurology Department Universitas Indonesia Hospital, Depok, Indonesia. <sup>2</sup>Neurology Department Faculty of Medicine Universitas Indonesia, Depok, Indonesia

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Dr. Sang Beom Kim<sup>1</sup>

<sup>1</sup>Department of Neurology, Kyung Hee University Hospital at Gangdong, Seoul, Korea, Republic of.

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Dr. Hsueh-Wen Hsueh<sup>1,2,3</sup>

<sup>1</sup>Department of Neurology, National Taiwan University Hospital, Taipei, Taiwan.

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Miss Reka Hejas<sup>1</sup>

<sup>1</sup>University of Pecs, Department of Neurology, Pécs, Hungary

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Mrs Emily Farrugia<sup>1,2</sup>

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<sup>1</sup>Northern Health, Melbourne, Australia. <sup>2</sup>La Trobe University, Melbourne, Australia

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Mr. Lok Man WAN

Occupational Therapy Department, North District Hospital, Hong Kong

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Dr. Magda Quagliotto<sup>1</sup>

<sup>1</sup>Neurology Unit, Department of Medical, Surgical and Health Sciences, University of Trieste, Trieste, Italy

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Dr. Marco Guglielmi<sup>1</sup>

<sup>1</sup>University of Bari "Aldo Moro", Bari, Italy

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Dr. Giovanni Vietri<sup>1,2,3</sup>

<sup>1</sup>Department of Systems Medicine, University of Rome Tor Vergata, Rome, Italy. <sup>2</sup>Neuromuscular Diseases Unit, Tor Vergata University Hospital, Rome, Italy. <sup>3</sup>Unit of Neurology, IRCCS Neuromed, Pozzilli (IS), Italy

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Dr. Jhee LEE

Department of Neurology, Seoul National University Hospital, Seoul, Korea, Republic of

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Prof. Makito Hirano<sup>1</sup>

<sup>1</sup>Department of Neurology, Kindai University, Faculty of Medicine, Sakai, Japan

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Ms. CHIHARU ISONO<sup>1</sup>

<sup>1</sup>Division of Rehabilitation Medicine, Kindai University Hospital, OSAKA, Japan

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Dr. Ester Franchi<sup>1</sup>

<sup>1</sup>NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy

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Assoc. Prof. eun bin cho<sup>1,2</sup>

<sup>1</sup>Department of Neurology, College of Medicine, Gyeongsang National University, Jinju, Korea, Republic of. <sup>2</sup>Gyeongsang National University Changwon Hospital, Changwon, Korea, Republic of

**PP01.325 5q-Associated Spinal Muscular Atrophy in Austria: Epidemiology and Comorbidities**

Dr. Omar Keritam<sup>1,2</sup>

<sup>1</sup>Department of Neurology, Medical University of Vienna, Vienna, Austria. <sup>2</sup>Comprehensive Center for Clinical Neurosciences and Mental Health, Medical University of Vienna, Vienna, Austria

**PP01.326 Increasing Prevalence of Amyotrophic Lateral Sclerosis in Austria Correlates With Reduced Mortality**

Dr. Omar Keritam<sup>1,2</sup>

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**PP01.327 Incidence and Prevalence of Amyotrophic Lateral Sclerosis in Curitiba, Brazil**

Dr. Otto Jesus Hernandez Fustes<sup>1</sup>

<sup>1</sup>Universidade Federal do Paraná, Curitiba, Brazil

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Assoc. Prof. Aysha Alshareef

King Abdulaziz University Hospital, Jeddah, Saudi Arabia

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Dr. Laura Ferraiuolo<sup>1</sup>

<sup>1</sup>Insmad Gene Therapy LLC, San Diego, CA, United States

**PP01.330 Quantitative Assessment of Motor Band Sign as a Neuroimaging Biomarker in Amyotrophic Lateral Sclerosis**

Dr. Eunhee Sohn<sup>1</sup>

<sup>1</sup>Chungnam National University Hospital, Daejeon, Korea, Republic of

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Ms. Jin-Ah Kim<sup>1,2</sup>

<sup>1</sup>Seoul National University Hospital, Seoul, Korea, Republic of. <sup>2</sup>Genomic medicine Institute, Seoul, Korea, Republic of

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Assoc. Prof. Jinwoo Park<sup>1,2</sup>

<sup>1</sup>Korea University, Seoul, Korea, Republic of. <sup>2</sup>Vanderbilt University, Nashville, United States

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Assoc. Prof. Seol-Hee Baek<sup>1</sup>

<sup>1</sup>Department of Neurology, Korea University Ansan Hospital, Korea University College of Medicine, Ansan, Korea, Republic of

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Dr. Akiko Hachisuka<sup>1</sup>

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Miss Elena Karkkainen

Newcastle University - John Walton Muscular Dystrophy Research Centre, Newcastle Upon Tyne, United Kingdom

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Miss Elena Karkkainen

Newcastle University - John Walton Muscular Dystrophy Research Centre, Newcastle Upon Tyne, United Kingdom

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Asst. Prof. HALLAL SIHAM<sup>1</sup>

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Dr. Leslie Nelson<sup>1</sup>

<sup>1</sup>Department of Physical Therapy, University of Texas Southwestern Medical Center, Dallas, United States

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Dr. Mónica Povedano<sup>3</sup>

<sup>3</sup>Department of Neurology, Hospital Universitari de Bellvitge, Institut d'Investigació Biomèdica de Bellvitge-IDIBELL, Barcelona, Spain

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Dr. Paz Castro

<sup>1</sup>Medical Department, Roche Farma, Madrid, Spain.

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Dr. Evgeniya Melnik<sup>1</sup>

<sup>1</sup>Research Centre for Medical Genetics, Moscow, Russia.

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Prof. Enrico Bertini<sup>1</sup>

<sup>1</sup>Research Unit of Neuromuscular and Neurodegenerative Disorders, Bambino Gesù Children's Research Hospital IRCCS, Rome, Italy

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Dr. Marcondes Cavalcante França Jr<sup>1</sup>

<sup>1</sup>UNICAMP, Campinas, Brazil

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Ms. Mei Wun Cheung<sup>1</sup>

<sup>1</sup>Allied Health Department (Physiotherapy) of Hong Kong Children's Hospital, Hong Kong SAR, China

**PP01.346 Indirect Treatment Comparison of OAV101 IT vs Risdiplam and Nusinersen in SMA DMT-Experienced Patients**

Mr. Nicholas Riley<sup>1</sup>

<sup>1</sup>Novartis, Basel, Switzerland

**PP01.347 Indirect Treatment Comparisons of OAV101 IT vs Risdiplam and Nusinersen in SMA DMT-Naïve Patients**

Mr. Nicholas Riley<sup>1</sup>

<sup>1</sup>Novartis, Basel, Switzerland

**PP01.348 Neuron-Specific Lrp4 Overexpression Rescues Impaired Neuromuscular Junction Formation via BMP Signaling in Drosophila SMA Model**

Dr. Rei Takada<sup>1</sup>

<sup>1</sup>Department of Pediatrics, Graduate School of Medical Science, Kyoto Prefectural University of Medicine, Kyoto, Japan

**PP01.349 Histopathology in Mitochondrial Cardiomyopathies**

Prof. Anders Oldfors<sup>1</sup>

<sup>1</sup>Department of Clinical Pathology, Sahlgrenska University Hospital, Gothenburg, Sweden

**PP01.350 Blink Reflex Recording as a Diagnostic Tool for Facial Involuntary Movements**

Prof. Bum Chun Suh<sup>1</sup>

<sup>1</sup>Department of Neurology, Kangbuk Samsung Hospital, Sungkyunkwan University School of Medicine, Seoul, Korea, Republic of

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Dr. Clément Guémy

Hopital Raymond Poincaré, Garches, France

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Dr. Dario Zoppi<sup>2</sup>

<sup>2</sup>Department of Neurosciences, Reproductive Sciences, and Odontostomatology, University of Naples "Federico II", Naples, Italy

**PP01.353 Electrophysiological Characteristics of Subclinical Diabetic Polyneuropathy in Adolescents with Type 1 Diabetes Mellitus**

Prof. Hyung-Soo Lee<sup>1</sup>

<sup>1</sup>National Medical Center, Seoul, Korea, Republic of

**PP01.354 Clinical and Electrophysiological Aspects About Two Cases of Parsonage Turner Syndrom**

Prof. Lala Bouna Seck<sup>1,2</sup>

<sup>1</sup>Gaston Berger University, Saint Louis, Senegal. <sup>2</sup>Fann National University Hospital Centre, Dakar, Senegal

**PP01.355 Longitudinal Electrophysiological Changes Predict Clinical Outcome in Chronic Inflammatory Demyelinating Polyradiculoneuropathy**

Dr. Sadıka Özdemir

Bakirkoy Prof. Dr. Mazhar Osman Training and Research Hospital for Mental Health and Neurological Diseases, University of Health Sciences, Istanbul, Turkey, istanbul, Turkey

**PP01.356 Systemic Botulism Mimicking Generalized Myasthenia Gravis After Cosmetic Botulinum Neurotoxin Injection: A Case Report**

Dr. Tatsuya Abe<sup>1</sup>

<sup>1</sup>Department of Neurology, National Hospital Organization Hakone Hospital, Odawara, Japan

**PP01.357 A Spectrum of Rapid-Onset Motor-Bulbar Disability and Tocilizumab Treatment Outcomes in *RANBP2-ANE1***

Dr. Ljelja Muaremoska Kanzoska<sup>1</sup>

<sup>1</sup>University clinic for pediatric diseases, Skopje, North Macedonia

**PP01.358 Personalized Medicine From Birth: Nationwide Newborn Screening for Spinal Muscular Atrophy in Serbia**

Asst. Prof. Milos Brkusanin

University of Belgrade - Faculty of Biology, Centre for Human Molecular Genetics, Belgrade, Serbia

**PP01.359 Advancing Clinical Trials in Myotonic Dystrophy Type 1: Refining Radiological, Clinical and Patient-Reported Outcome Measures**

Miss Louise Iterbeke<sup>1</sup>

<sup>1</sup>Laboratory for Muscle Diseases and Neuropathies, Department of Neurosciences, KU Leuven, and Leuven Brain Institute (LBI) and Leuven Institute for Rare Diseases (Leuven.IRD), Leuven, Belgium

**PP01.360 Responsiveness to Change of Quantitative Whole-Body MRI in FSHD: Results From ReDUX4 OLE**

Dr. Per Widholm

AMRA Medical AB, Linköping, Sweden

**PP01.361 Expanding Spectrum of Molecular Landscape of Nematine Rod Myopathy**

Dr. MEHAR SHARMA

AIIMS, Delhi, India

**PP01.362 Increase of Number and Genic Type of eccDNA in Spinal Cord of ALS Murine Model**

Dr. Daniela Gerovska<sup>1</sup>

<sup>1</sup>Biogipuzkoa Health Research Institute, San Sebastian, Spain

**PP01.363 Exploring MSOT as a Non-Invasive Biomarker for Myotonic Dystrophy Type 2**

Ms. Jin Wang<sup>1,2</sup>

<sup>1</sup>University Medical Center Goettingen, Clinic for Neurology, Robert-Koch-Straße 40, 37075 Goettingen, Goettingen, Germany. <sup>2</sup>University Medical Center Goettingen, Heart & Brain Center Goettingen, Robert-Koch-Straße 42, 37075 Goettingen, Goettingen, Germany

**PP01.364 Orthogonal Methods Demonstrate Pharmacodynamic Responses in Duchenne Muscular Dystrophy Patients Treated with BMN 351**

Ms. Kristin Obrochta-Moss<sup>2</sup>

<sup>2</sup>BioMarin Pharmaceutical Inc., Novato, United States

**PP01.365 Muscle Mass and Strength Changes in Patients With Peripheral Neuropathy and Central Nervous System Disorders**

Dr. Sung-Ju Hsueh<sup>1</sup>

<sup>1</sup>National Taiwan University Hospital Bei-Hu Branch, Taipei, Taiwan

**PP01.366 STEP Study Design: Validating the Syde<sup>®</sup> Device to Measure Motor Function in Late-Onset Pompe Disease**

Dr. Andrew Oldham<sup>1</sup>

<sup>1</sup>Salford Royal NHS Foundation Trust, Salford, United Kingdom

**PP01.367 LEOPARD-DMD: Interval Results from a Duchenne Muscular Dystrophy Natural History Study**

Dr. Chad Heatwole<sup>1</sup>

<sup>1</sup>University of Rochester, Rochester, United States

**PP01.368 Smartphone-Based Assessment of Shoulder Motion in Patients With Facioscapulohumeral Muscular Dystrophy**

Dr. Claudia Mazza<sup>5</sup>

<sup>5</sup>Indivi AG, Basel, Switzerland

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## **PP01.369 Evaluation of Ambulation With a Wearable Sensor in Patients With CMT**

Mr. Damien Eggenspieler<sup>2</sup>

<sup>2</sup>Synnav, Vernon, France

## **PP01.370 Synthetic Data as a Research Enabler in CIDP: A Registry-Based Validation Study**

Dr. Erika Schirinzi<sup>2</sup>

<sup>2</sup>Department of Clinical and Experimental Medicine, Neurological Clinic, University of Pisa, Pisa, Italy

## **PP01.371 Non-Physician-Administered Neuropathy Symptom and Disability Scores for Screening of Diabetic Distal Symmetric Polyneuropathy**

Dr. Jakkrit Amornvit<sup>1,2</sup>

<sup>1</sup>Division of Neurology, Department of Medicine, King Chulalongkorn Memorial Hospital, Thai Red Cross Society, Bangkok, Thailand. <sup>2</sup>Faculty of Medicine, Chulalongkorn University, Bangkok, Thailand

## **PP01.372 Revolutionizing Gait Quality Monitoring in People with CIDP: An Instrumented Shoe Insole Solution**

Dr. Karen M. Lynch<sup>1</sup>

<sup>1</sup>Sanofi, Cambridge, MA, United States

## **PP01.373 Transitory Symptom Worsening in Facioscapulohumeral and Becker Muscular Dystrophies: Do They Exist and Matter?**

Dr. Nurit Birman Har-Noy<sup>1</sup>

<sup>1</sup>Neurorehabilitation Unit University of Milan The NeMO Clinical Center, Milan, Italy

## **PP01.374 Efficacy of Anti-Osteoporotic Drugs and Vitamin D/calcium Supplementation in NMD: A Systematic Review**

Dr. Sara Liguori<sup>1</sup>

<sup>1</sup>Department of Medical and Surgical

Specialties and Dentistry, University of Campania "Luigi Vanvitelli", Naples, Italy

## **PP01.375 Chronic Pain and Postural Instability in Individuals With Neuromuscular Diseases: A Cross-Sectional Study**

Dr. Sara Liguori<sup>1</sup>

<sup>1</sup>Department of Medical and Surgical Specialties and Dentistry, University of Campania "Luigi Vanvitelli", Naples, Italy

## **PP01.376 Musculoskeletal Impairment in Adults With Neurofibromatosis Type 1: An Observational Study**

Dr. Sara Liguori<sup>1</sup>

<sup>1</sup>Department of Medical and Surgical Specialties and Dentistry, University of Campania "Luigi Vanvitelli", Naples, Italy

## **PP01.377 The Role of Magnesium in Muscle Health and Neuromuscular Diseases**

Dr. Sara Liguori<sup>1</sup>

<sup>1</sup>Department of Medical and Surgical Specialties and Dentistry, University of Campania "Luigi Vanvitelli", Naples, Italy

## **PP01.378 Clinical and Functional Characterization of a Pediatric Cohort With Neurofibromatosis Type 1**

Dr. Sara Liguori<sup>1</sup>

<sup>1</sup>Department of Medical and Surgical Specialties and Dentistry, University of Campania "Luigi Vanvitelli", Naples, Italy

## **PP01.379 Multidimensional Evaluation of Functioning in a Pediatric Population With X-linked Hypophosphatemic Rickets: Observational Study**

Dr. Sara Liguori<sup>1</sup>

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## **PP01.380 Investigation of Dysphagia Risk and Chewing Function in SMA Type II: A Cross-Sectional Descriptive Study**

Miss Ceren Şeval KARATAS<sup>3</sup>

<sup>3</sup>University of Health Sciences Turkey, Gulhane Institute of Health Sciences, Ankara, Turkey

## **PP01.381 Quantitative Muscle Ultrasound as a Clinical Correlate in Fshd: Validation of a Rapid Protocol**

Assoc. Prof. Alon Abraham<sup>1,2</sup>

<sup>1</sup>Tel Aviv Sourasky Medical Center, Tel Aviv, Israel. <sup>2</sup>Tel-Aviv University, Tel-Aviv, Israel

## **PP01.382 Neurofilament Light Chain As A Biomarker In Chronic Inflammatory Demyelinating Polyradiculoneuropathy: Insights From ADHERE**

Dr. Bianca Balbino<sup>2</sup>

<sup>2</sup>Argenx, Ghent, Belgium

## **PP01.383 Treg Cells Attenuate Neuroinflammation and Protect Neurons in a Mouse Model of Parkinson's Disease**

Dr. Yan Huang

Nantong University, Nantong, China

## **PP01.384 Microglia-Derived Exosomal ciRS-7 Mediates IL-17A Effect of Promoting Neurodegeneration in an Experimental Parkinson's Disease**

Prof. Yi-Hua Qiu

Nantong University, Nantong, China

## **PP01.385 IL-17A Exacerbates Neuroinflammation and Neurodegeneration by Activating microglia in Rodent Models of Parkinson's Disease**

Dr. Zhan Liu

Nantong University, Nantong, China

## **PP01.386 Characterization of VCP-Interacting Proteins in VCP-iPS Cell-Derived Skeletal Muscle Cells**

Dr. Fumiaki Saito<sup>1</sup>

<sup>1</sup>Department of Neurology, School of Medicine, Teikyo University, Tokyo, Japan

## **PP01.387 Targeted Degradation of Polyglutamine-Expanded Androgen Receptor Improves Spinal and Bulbar Muscular Atrophy**

Prof. Hong-Yo Kang<sup>1,2</sup>

<sup>1</sup>Graduate Institute of Clinical Medical Sciences, College of Medicine, Chang Gung University, Taoyuan City, Taiwan. <sup>2</sup>Center for Hormone and Reproductive Medicine Research, Department of Obstetrics and Gynecology, Kaohsiung Chang Gung Memorial Hospital, Kaohsiung, Taiwan

## **PP01.388 MyoScreen, a Human Skeletal Muscle Platform, Reveals Novel Targets and Muscle-Preserving Small Molecules**

Dr. Louise Griveau

CYT00 SA, Grenoble, France

## **PP01.389 High-Throughput 3D Light-Sheet Imaging of Mouse Hindlimbs Enables Skeletal Muscle Morphometrics in Muscle Wasting Models**

Dr. Max Hahn

Gubra, Hørsholm, Denmark

## **PP01.390 Unmet Support Needs of ALS Patients Following Failed Clinical Trials**

Dr. Dalisha Dalisha

National Hospital Of Neurology and Neurosurgery, London, United Kingdom

## **PP01.391 Living With TNPO3-Related Limb-Girdle Muscular Dystrophy D2: A Quality of Life Analysis**

Dr. Irune García<sup>1</sup>

<sup>1</sup>University of Deusto, Bilbao, Spain

# Advancing TK2d management: From natural history to clinical evidence and real-world opportunities

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**July 10, 2026**  
13:15–14:15



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Spadolini Attic

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- Emerging evidence on patient characterization and treatment approaches in TK2d and their implications for clinical management
- Applying clinical insights from real-world cases to improve diagnosis, treatment initiation, and monitoring of patients with TK2d

## Faculty:



**Caterina  
Garone**

University of Bologna  
**Bologna, Italy**



**Yolanda  
Cámara**

Vall d'Hebron  
Research Institute  
**Barcelona, Spain**



**Cristina  
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University Hospital 12  
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# Complement Component 5 Inhibitor Therapies (C5ITs) in Generalised Myasthenia Gravis (gMG): From Established Evidence to Latest Clinical Data

13:15–14:15 • Thursday 9 July 2026 • Spadolini Attic - Room 2



**Prof. Elena Sacconi**  
University of Parma  
Parma, Italy



**Prof. Sabrina Sacconi**  
Nice University Hospital  
Nice, France

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This event is organised and funded by Alexion, AstraZeneca Rare Disease, and is intended for healthcare professionals only. Presentations will include data relating to Alexion products, with a focus on generalised myasthenia gravis in adult patients who are anti-acetylcholine receptor antibody-positive. Product registration conditions differ internationally, and prescribing information may vary depending on approval in each country. The views expressed in this industry-supported symposium are based upon the clinical experience of the presenters.

M/IT/ULT-g/0003 05/2026

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**Thursday July 9<sup>th</sup>, 2026 | 13:15-14:15 CEST**  
**Spadolini Attic - Room 1 (Level 2)**



**DMD Therapeutic Landscape and the Phase 3 EPIDYS  
Study**

**Chair: Dr. Erik Niks, M.D., Ph.D.**

*Department of Neurology, Leiden University Medical Center, Leiden  
(The Netherlands)*



**Long-Term Extension Data, Real-World Experience, and  
Practical Management Considerations**

**Prof. Dr. Eugenio Mercuri, M.D., Ph.D.**

*Department of women and children health sciences, Fondazione  
Policlinico Universitario Agostino Gemelli IRCCS, Roma  
Università Cattolica del Sacro Cuore, Roma (Italy)*



**US Clinical Practice Experience with Givinostat**

**Dr. Perry Shieh, M.D., Ph.D., FAAN**

*Departments of Neurology and Pediatrics, University of California,  
Los Angeles, California (USA)*



**Panel Discussion and Audience Q&A**

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