ICNMD 2026

19TH 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, physiatrists, 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,

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. Mauermann
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

Vincenzo Nigro – Co-Chair
University of Campania "Luigi Vanvitelli", Italy

Ichizo Nishino
National Center of Neurology and Psychiatry, Japan

John Vissing
Copenhagen University Hospital, Denmark

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

Marco Savarese
Folkhalsan Research Center, Finland

Massimiliano Filosto
University of Brescia, Italy

Mazen M. Dimachkie
University of Kansas Medical Center, USA

Peripheral Nerve

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

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

David Cornblath
Johns Hopkins University, USA

Eduardo Nobile-Orazio
University of Milan, Italy

Kleopas A. Kleopa
Institute of Neurology & Genetics, Cyprus

Marcelo Rugiero
Hospital Italiano de Buenos Aires, Argentina

Mary Reilly
University College London, UK

P. James B. Dyck
Mayo Clinic, USA

Satoshi Kubawara
Chiba University, Japan

Neuromuscular Junction

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

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

Andreas Meisel
Charité – Universitätsmedizin Berlin, Germany

Carlo Antozzi
IRCCS Istituto Neurologico Carlo Besta, Italy

Gil Wolfe
University at Buffalo, USA

Hanns Lochmuller
University of Ottawa / CHEO Research Institute, Canada

Henry Kaminski
George Washington University, USA

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

Nils Erik Gilhus
University of Bergen, Norway

Pushpa Narayanaswami
Beth Israel Deaconess Medical Center, USA

Motor Neuron

Adriano Chiò – Co-Chair
University of Turin, Italy

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

Jessica Mandrioli
University of Modena and Reggio Emilia, Italy

Jonathan Glass
Emory University, USA

Julian Großkreutz, University of Lübeck, Germany

Leonard van den Berg, UMC Utrecht, Netherlands

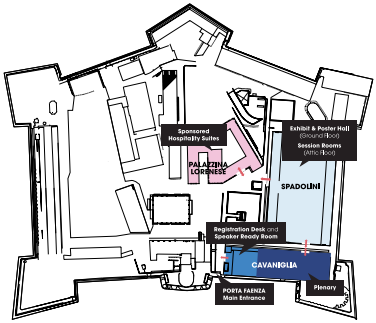
Merit Cudkowicz, Massachusetts General Hospital / Harvard Medical School, USA

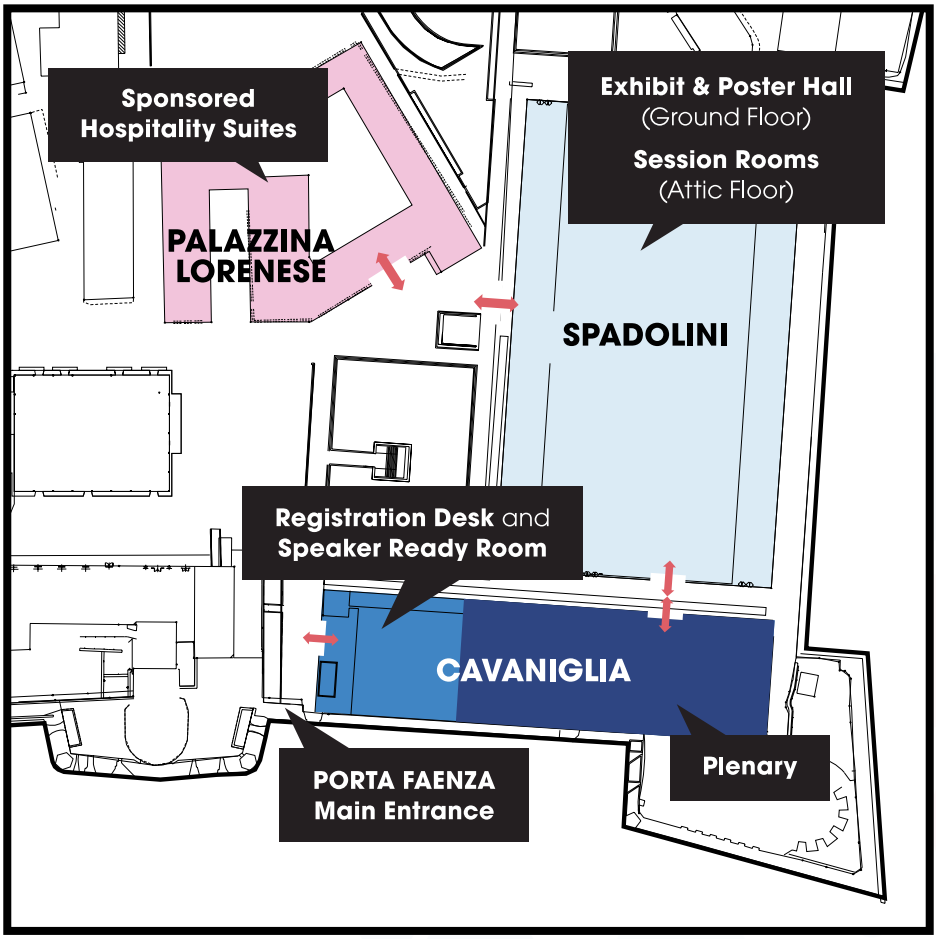
Nicola Ticozzi, University of Milan, IRCCS Istituto Auxologico Italiano, Italy

Venue Floorplan

Fortezza da Basso

FIRENZE FIERA

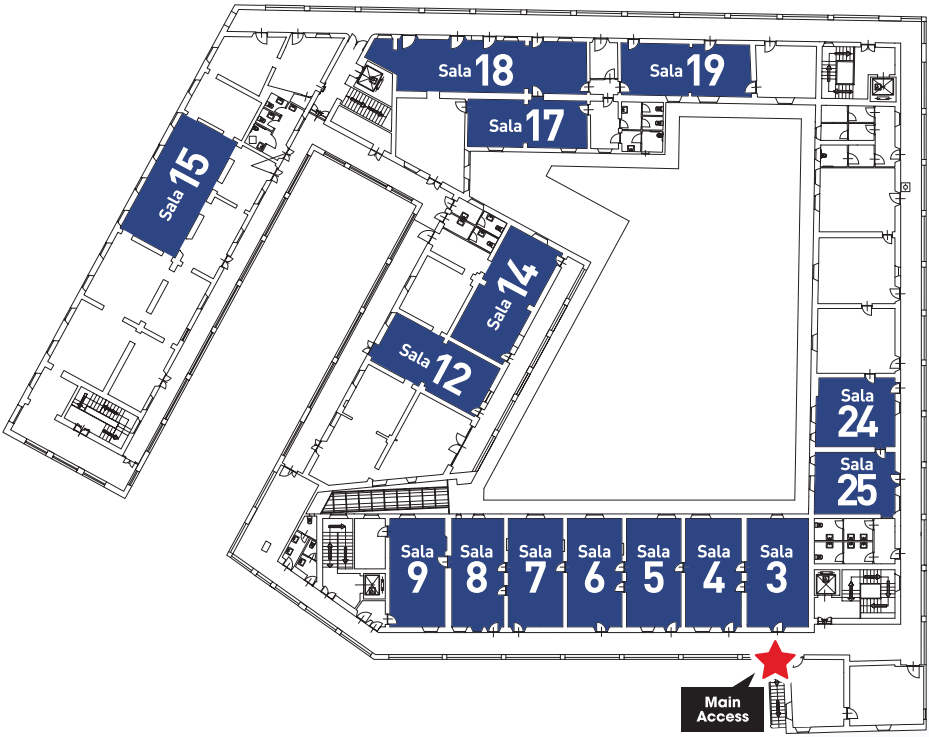




Venue Floorplan

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

Schedule at a Glance

Teaching Courses · Social Functions · Joint Sessions · Oral Abstracts · Ticketed Sessions
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Wednesday 8 July

TIME	SESSION NAME	LOCATION
08:30 - 10:30	**PL01:** Plenary: Myology 2030	Cavaniglia - Plenary Room
10:30 - 11:15	Morning Break & Poster Tour 1	Exhibit & Poster Hall
11:15 - 12:45	**SS01:** Up-to-date on Muscle Inflammation	Hall 2
	SS02: Up-to-date on FSHD and OPMD	Hall 5
	SS03: Primary Lateral Sclerosis	Hall 3
	SS04: FcRn and Complement Inhibitors in MG Severe Deteriorations	Hall 4
	SS05: ICNMD & World Muscle 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	**SS06:** Up-to-date on Metabolic Muscle Disorders	Hall 2
	SS07: Biomarkers of Early Diagnosis in ALS	Hall 3
	SS08: Tools for the Next Generation Myologists	Hall 5
	SS09: Advances in TTR Amyloid	Hall 4
	SS17: ICNMD, AIM & ASNP Joint Session	Hall 1
16:00 - 16:45	Afternoon Break & Showcase Theatre	Exhibit & Poster Hall
16:45 - 18:15	**SS11:** Up-to-date on Limb Girdle Myopathies	Hall 1
	SS12: Early-phase Treatments in MG	Hall 2
	OS01: Oral Session 1	Hall 3
	OS02: Oral Session 2	Hall 4
	OS03: Oral Session 3	Hall 5

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Schedule at a Glance

Teaching Courses | Social Functions | Joint Sessions | Oral Abstracts | Ticketed Sessions
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Thursday 9 July

TIME	SESSION NAME	LOCATION
07:00 - 08:00	Industry-Supported Symposia	Spadolini – Attic
08:30 - 10:30	**PL02:** 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	**SS13:** Cancer Related Peripheral Neuropathies	Hall 2
	SS14: Challenges in MG Treatment	Hall 3
	SS15: Biomarkers of Disease Progression and Prognosis	Hall 5
	SS16: Up-to-date on Immune-medicated Neuropathies	Hall 4
	SS10: 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	**SS18:** Up-to-date on Myotonic Dystrophies	Hall 2
	SS19: Technologies Updates in Neuropathies (formally Updates in CMT)	Hall 3
	SS20: Tailoring MG Care for Women	Hall 5
	SS21: Hereditary Neuropathy Overlap Syndromes	Hall 4
	SS22: 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	**SS23:** Update on Diagnostic Tools in Neuropathy	Hall 1
	SS24: Patient Monitoring in NMJ Diseases	Hall 5
	OS04: Oral Session 4	Hall 2
	OS05: Oral Session 5	Hall 3
	OS06: Oral Session 6	Hall 4

Schedule at a Glance

Teaching Courses · Social Functions · Joint Sessions · Oral Abstracts · Ticketed Sessions
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Friday 10 July

TIME	SESSION NAME	LOCATION
07:00 - 08:00	Industry-Supported Symposia	Spadolini – Attic
08:30 - 10:30	**PL03:** 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	**SS25:** New Outcome Measures	Hall 1
	SS26: ALS Clinical Trials: Novel Approaches Toward an Effective Cure	Hall 2
	SS27: Diabetic Neuropathies	Hall 3
	SS28: From Duchenne to Becker Muscular Dystrophies	Hall 4
	SS29: 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	**SS30:** Up-to-date Supportive and Palliative Care in Muscle Disorders	Hall 2
	SS31: A Worldwide Overview of ALS	Hall 3
	SS32: Updates on ALS Clinical Trials	Hall 5
	SS33: MG in the Elderly	Hall 4
	SS34: 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	**SS35:** Cognition in ALS	Hall 1
	SS36: Advanced Topics in Peripheral Neuropathy	Hall 2
	OS07: Oral Session 7	Hall 3
	OS08: Oral Session 8	Hall 4
	OS09: Oral Session 9	Hall 5
19:30 - 22:30	Networking Dinner	Villa Vittoria

Schedule at a Glance

Teaching Courses · Social Functions · Joint Sessions · Oral Abstracts · Ticketed Sessions
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Saturday 11 July

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

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Teaching Courses | Social Functions | Joint Sessions | Oral Abstracts | Ticketed Sessions
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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

Teaching Courses | Social Functions | Joint Sessions | Oral Abstracts | Ticketed Sessions
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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

Teaching Courses | Social Functions | Joint Sessions | Oral Abstracts | Ticketed Sessions
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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

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

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
Nataliia 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 Mitochodrial 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

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

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

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

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 Nlsdm/nlsdi :(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

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

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

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 Paralyses 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

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

14:30 - 16:00 **SS22 ICNMD & German Nerve Club Joint Session - Nerve Surgery, Diagnostics and Rehabilitation**
Location: Spadolini Attic - Hall 1
Chair(s): Robert Schmidhmmer, 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

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

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

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

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

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

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

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

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 Lulè, Germany

16:50 - 17:10 **SS35.01** Correlates and Impact of Cognitive Impairment in ALS
Dorothee Lulè, 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

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

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 Doksani, 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 Rezania, 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

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

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-Puszczyk, 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

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

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

14:15 - 15:45 **SS46 Ageing and Senescense**
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

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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 Examinaton	Rocco Ligouri	Italy
Speaker	Nerve Conducton Studies of Proximal Nerves (RNS)	William Litchy	USA

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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 fascio-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 expanion 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

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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

Teaching Courses

Location: Spadolini – Attic
Access: Pre-Registration required
Cost: €200.00 per course

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 b 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 Schmidhmmer	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 Atamyo Therapeutics in 2021. She has published over 205 scientific publications.

Muscle Disorders · Motor Neuron Diseases · Peripheral Neuropathies · Neuromuscular Junction Disorders

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.

Muscle Disorders | Motor Neuron Diseases | Peripheral Neuropathies | Neuromuscular Junction 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 Heath 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

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

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:

sanofi

Speaker Ready Room Hours

Location: Cavaniglia

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

WiFi

Network: ICNMD Password: icnmd2026

WiFi Supported by:

Dyne THERAPEUTICS

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.

Amicus Therapeutics

SYMPOSIUM • FRIDAY, JULY 10, 2026 • 13:15-14:15 • SPADOLINI ATTIC - ROOM 2

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.

Com-PO-IN-26-00017-1 | April 2026

Johnson&Johnson
The Health&Care Company

CP-578328 | May 2026

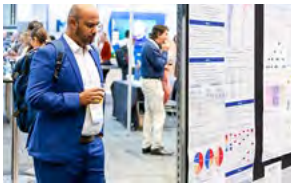
Social Events

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
Poster Tour 1: Muscle and Neuromuscular Junction	**Poster Tour 2:** Motor Neuron	**Poster Tour 3:** Peripheral Neuropathy

Poster Tours supported by:
santhera
THEIR FUTURE - OUR FOCUS

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**

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.

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.

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

Dyne THERAPEUTICS

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Date of preparation: April 2026. MED-01338

PNS
PERIPHERAL NERVE
SOCIETY
CHICAGO
8–11 MAY, 2027
SAVE THE DATE!
2027 ANNUAL MEETING
Hilton Chicago • Chicago, Illinois, USA

Showcase Theatre

Location: Spadolini – Exhibit & Poster Hall

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

Phase 3 FORTIFY Study: Scientific Update in LGMD2I/R9

Presenter: Douglas M. Sproule, 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 αDG 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

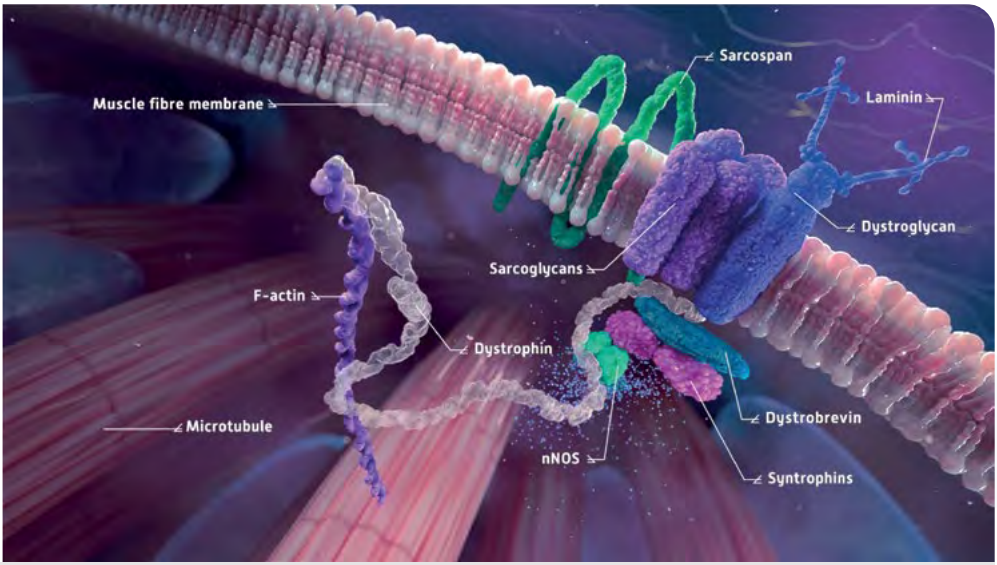
Channelopathies in childhood: the mosaic of myotonia

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 RARE DISEASES

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

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

ITALFARMACO

www.itfrarediseases.com is dedicated exclusively for healthcare professional who practise in Europe. This website requires registration for full access.
IT-DMD-26-00002 (May 2026)

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

Spadolini Attic: Room 4, Fortezza da Basso, Florence, Italy

Wednesday 8 July 2026, 13:15–14:15 CEST

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

LUNCH PROVIDED

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

Inspired by patients. Driven by science.

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 needfor objective biomarkers	Prof. Anna Rostedt Punga
13:30 - 14:10	Targeted treatment selection: Facilitating patient choice	Dr. Vincenzo Di Stefano/ Prof. Suraj Muley
	MuleyDiscussions 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

LUNCH PROVIDED

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 zeleciment 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 zeleciment 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

NOVARTIS

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

NOVARTIS

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 Remai	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

BREAKFAST PROVIDED

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

LUNCH PROVIDED

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)
- **Spealer:** 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

LUNCH PROVIDED

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 Saccani (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 Saccani
	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

LUNCH PROVIDED

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

LUNCH PROVIDED

sanofi

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

BREAKFAST PROVIDED

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 5) 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

BREAKFAST PROVIDED

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 telitacicept, 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 telitacicept 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	Telitacicept 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

LUNCH PROVIDED

argenx

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

LUNCH PROVIDED

medthority

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

NOVARTIS

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

LUNCH PROVIDED

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 Shahar 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 Shahar 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

LUNCH PROVIDED

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

LUNCH PROVIDED

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

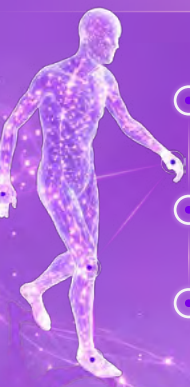
Exploring clinical gaps and what's next in CIDP management

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.

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sanofi

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

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C-ANPROM/INT/NON/0275 | June 2026.

Exhibit & Poster Hall Information

Location: Spadolini – Ground Floor

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

Poster Tours supported by: santhera THEIR FUTURE - OUR FOCUS

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Alexion, AstraZeneca Rare Disease **107**
Amgen **449**
Amicus Therapeutics **212**
argenx **401**
ATOM International **137**
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BridgeBio **240**
DEYMED Diagnostic **123**
Dyne Therapeutics **101**
Edgewise Therapeutics **117**
Entrada Therapeutics **135**
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EURO-DM-CRN **159**
European Neuromuscular Centre (ENMC) .. **254**
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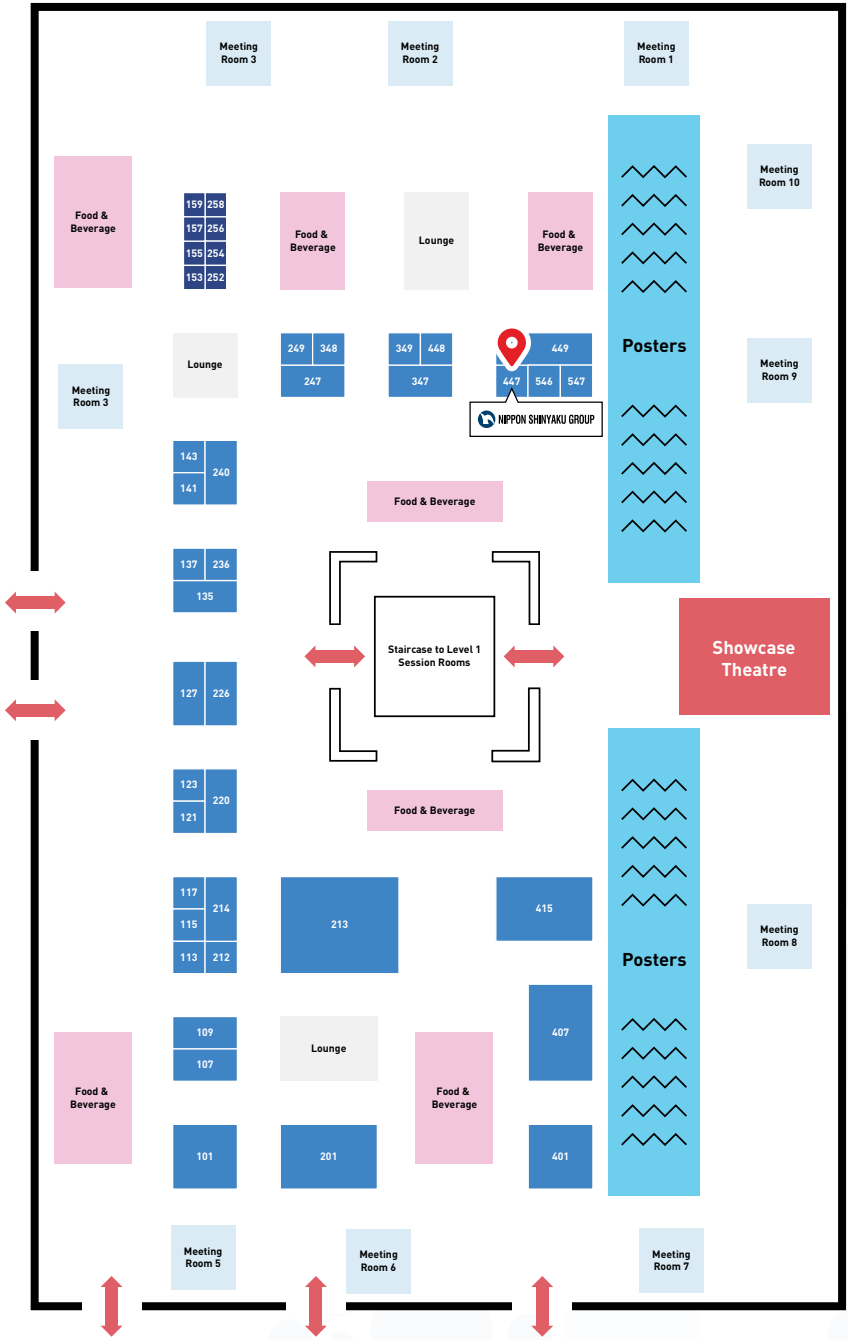
Vor Biopharma **214**
World Federation of Neurology (WFN) **349**

Exhibit & Poster Hall Floorplan

Spadolini



Exhibitor Information

Alexion, AstraZeneca Rare Disease

★ **Booth #107**

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

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

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.

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argenx

★ **Booth #401**

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

★ **Booth #415**

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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 (TGFβ) superfamily biology, the company is focused on advancing innovative treatments where protein growth factors are fundamental.

Takeda Pharmaceuticals

★ **Booth #121**

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

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

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

Vor Bio is a clinical-stage biotechnology company rapidly advancing telitacicept, 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

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

Dr. Sithara Ramdas[1,2]

[1]Department of Paediatric Neurology, John Radcliffe Hospital, Oxford, United Kingdom. [2]MDUK Oxford Neuromuscular Centre, Department of Paediatrics, University of Oxford, Oxford, United Kingdom

PP01.02 Specific Myostatin Inhibition Ameliorates Sarcopenia and Extends Lifespan in α-Klotho-Null Mice

Assoc. Prof. Yutaka Ohsawa[1]

[1]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 Risi[1,2]

[1]NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy, Brescia, Italy. [2]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 Risi[1,2]

[1]NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy. [2]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 Risi[1,2]

[1]NeMO-Brescia Clinical Center for Neuromuscular Diseases, Brescia, Italy. [2]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[1]

[1]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[1]

[1]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[1],

[1]Department of Laboratory Medicine, University of Gothenburg, Gothenburg, Sweden

PP01.09 Biallelic SNAPC4 Variants Associatedwith Lower Motor Neuron Disease

Dr. Chiara Fiorillo[1,2]

[1]Department of Neuroscience, University of, Genova, Italy. [2]Pediatric Neurology, Gaslini Children Hospital, Genova, Italy

PP01.10 Expanding the Phenotypic Spectrum of FKTN Mutations: A Case of Generalized Epilepsy, Neurodevelopmental Delay, andHyperCKemia

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[2,1]

[1]Department of Neuroscience, University of Genova, Genova, Italy. [2]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[1],

[1]Department of Neurology, Karolinska University Hospital, Stockholm, Sweden

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

Dr. Gordana Kovacevic[1,2]

[1]Neurology Department, Mother and Child Health Care Institute of Serbia "Dr Vukan Cupic", Belgrade, Serbia. [2]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[1],

[1]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[1]

[1]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[1]

[1]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[1,2]

[1]Neuromuscular Repair Unit, Institute of Experimental Neurology, Ospedale San Raffaele, Milan, Italy. [2]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[1]

[1]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[1]

[1]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[1],

[1]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[1]

[1]Department of Neurology, Gangnam Severance Hospital, Younsei 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[1]

[1]Department of Neurology, Gangnam Severance Hospital, Younsei University College of Medicine, Seoul, Korea, Republic of

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

Dr. Paola Laghetti[1]

[1]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[1]

[1]Jagiellonian University, Kraków, Poland

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

Dr. Ana Kosac[1,2]

[1]Clinic of neurology and psychiatry for children and youth, Belgrade, Serbia. [2]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[1]

[1]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[1]

[1]Department of Pharmacy – Drug Sciences, University of Bari "Aldo Moro", Bari, Italy

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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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

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

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

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

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

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

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

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

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

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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?

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

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

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PP01.66 X-Inactivation Patterns in Blood and Muscle of symptomatic Duchenne Carriers: Cross-Sectional Study of 30 Cases

Dr. Miriam Hiebeler[1],

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PP01.67 Interim Safety Results From an Open-Label Extension Study of Givinostat Treatment For Duchenne Muscular Dystrophy

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

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

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

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

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

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PP01.73 Pharmacodynamic Response of Circulating Proteins to Different Corticosteroid Types and Regimens in Duchenne Muscular Dystrophy

Assoc. Prof. Utkarsh Dang[1]

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

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PP01.75 LT-001 a Long-Term Follow-Up Study for Participants That Completed the SAT-3247-CL-101 Study: 6-Month Update

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PP01.76 A Cyclic Peptide Targeting the Myostatin-Inhibitory Core Region Alleviates Dystrophic Pathology in D2-*mdx* Mice

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PP01.77 Fascioscapulohumeral Dystrophy and Noonan Syndrome

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PP01.78 Efficacy and Safety of Iptacopan in Patients With Generalized Myasthenia Gravis: APPRAISE Study Design

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PP01.79 Minimal Symptom Expression in Myasthenia Gravis: A Post Hoc Analysis of Phase 3 Rozanolixizumab Studies

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PP01.80 Myasthenia Gravis Inebilizumab Trial (MINT): Adverse Events of Special Interest During the Randomized Controlled Period

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

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PP01.82 Trial Design for the Phase 2 FORGE Study Evaluating Apitegromab in Adults With FSHD

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PP01.83 Phenotypic Diversity and Upper Limb Function in Slovenian Patients With Facioscapulohumeral Muscular Dystrophy

Prof. Lea Leonardis[1]

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PP01.84 Long-Term Respiratory Follow-Up of Patients with FSHD1: An Italian Single-Center Study

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PP01.85 Clinical Characteristics and Longitudinal Changes in the Japanese FSHD Registry: Insights From 206 Patients

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PP01.86 Myasthenia Gravis-Myositis Overlap: A Comparative Study of Idiopathic and Immune Checkpoint Inhibitor-Related Forms

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PP01.87 A Rare Autoimmune Myopathy in Later Life: Statin-Associated Anti-HMGCR IMNM Presenting with Troponin Elevation

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PP01.88 Clinical Characteristics and Therapeutic Response in Patients With Autoimmune Nodopathies: A Multi-Center US-Based Study

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PP01.89 Exercise and Functional Decline in Inclusion Body Myositis: A Study From the Yale IBM Registry

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PP01.90 Safety and Efficacy of Rese-cel in Severe, Refractory Idiopathic Inflammatory Myopathy

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PP01.91 Tiny Molecules, Mighty Impacts: MicroRNAs in Neuromuscular Diseases

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PP01.92 When Genes Fall Silent: Muscle Biopsy Guiding Lipid Storage Myopathy Management in Resource-Limited Settings

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PP01.93 Unveiling a Novel Link: Myofibrillar Myopathy Phenotype Associated with TRIM32 Gene Mutation.

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PP01.94 MxA Positivity Beyond Dermatomyositis: New Insights from a case of Lupus Myositis

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PP01.95 Novel TTN Variants Causing Emery-Dreifuss-Like Phenotype: Expanding the Genotypic Spectrum

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PP01.96 Eotaxin/CCR3-Mediated Inflammation in Inclusion Body Myositis: A Permissive Environment for Viral Entry and Muscle Degeneration

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PP01.97 Distinct Thigh Muscle MRI Patterns Across Idiopathic Inflammatory Myopathy Subtypes: A Single-Center Cohort Analysis

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

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

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PP01.100 IBM Presenting as Early-Onset Facial Diplegia and Axial Weakness: Two Italian Cases and Literature Review

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PP01.101 Systemic Anti-Neutrophil Cytoplasmic Antibody (ANCA) Vasculitis Presenting With Isolated Myositis Without Neuropathy

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PP01.102 Treatment Outcomes of Inflammatory Myopathies Stratified by Pathology and Autoantibodies: A Study of 127 Patients

Dr. Nobuyuki Eura

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

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

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

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

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PP01.107 Interplay Infection, Locking Syndrome in Lgmd Tnpo3-Related: Sporadic Slovakian Patient With Novel Mutation

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PP01.108 Interim Analysis from Ongoing Phase 3 FORTIFY Study of BBP-418 for LGMD2I/R9 Meets Efficacy Endpoints

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PP01.109 Childhood Limb-Girdle Muscular Dystrophies: Phenotype-to-Genotype Diagnostic Journey: Single-Center Experience

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PP01.110 Severe De Novo Desminopathy iwith Multisystem Involvement: A Case Report and Review of the Literature

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PP01.111 Differential Expression of Dysferlin in Rat Fast and Slow Skeletal Muscle

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PP01.112 Reclassifying ISPD VUS in Siblings: The Power of Deep Phenotyping in Neuromuscular Diagnosis

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PP01.113 MICU1-related Mitochondrial Myopathy Mimicking Limb-Girdle Muscular Dystrophy

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

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PP01.115 Characteristics of Patients with Late-Onset Pompe Disease: Insights from the Romanian Cohort

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PP01.116 Bridging the Diagnostic Gap in POLG Disorders: Phenotypic Variability and Clinical Challenges in Predominantly Brazilian-Sample

Prof. Enzo Stinghel Pellacani[8]

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PP01.117 Brain Physiological Transcriptomic Architecture Underpinnings of Regional Vulnerability to Stroke-Like Lesions in MELAS

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PP01.118 Late-Onset TK2 Deficiency in Adults: Long-Term Clinical Outcomes of Deoxynucleoside Therapy

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PP01.119 Quantitative Gait Analysis Using a Wereable Sensor Device (Baiobit) in Patients With Metabolic Myopathies

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PP01.120 Functional Performance, Patient-Reported Outcomes and Biomarkers in Primary Mitochondrial Myopathies During Clinical Follow-Up

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PP01.121 Slow Channel Congenital Myasthenic Syndrome Due To c.1396G>A In CHRNA1 Favourably Responding To 3,4-DAP

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PP01.122 Leigh Syndrome Due to the Compound Heterozygous Variants c.1162A > C and c.1138G > C in NDUFV1

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PP01.123 Danon Disease in Japan: An Updated Nationwide Clinicogenetic Analysis

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PP01.124 Small Fiber Neuropathies: The Role of Sudoscan in Clinical Practice

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PP01.125 Somatosensory Evoked Potentials (SSEP) in Phenotyping Chronic Inflammatory Demyelinating Polyradiculoneuropathy (CIDP) Variants

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PP01.126 Glycogen Storage Diseases in Morocco: A Clinical Series Highlighting Phenotypic Heterogeneity and Diagnostic Challenges

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PP01.127 Switching From Alglucosidase Alfa to Avalglucosidase: Real-World Data From a Single-Center Experience

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PP01.128 Sertraline-Associated Multiple Acyl-Coenzyme a Dehydrogenase Deficiency (MADD) in Sweden – a Nationwide Population-Based Study

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PP01.129 A Recurrent ATP2A2 p.R528Q Variant Causes Dominant Rhabdomyolysis Due to Impaired Muscle Ca^2 SERCA2 Pump

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PP01.130 Design of ACHILLES Phase 3 Randomised Study: Mexiletine PR vs. Mexiletine IR in Non-Dystrophic Myotonia

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PP01.131 Andersen-Tawil Syndrome without Cardiac Conduction Defect: A Genetically Confirmed Case

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PP01.132 Monitoring Generalized Myasthenia Gravis Symptoms With ME&MG™ Digital Biomarkers: DOMYA, a Validation Study

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PP01.133 HARMONY360 Study for Comprehensive Monitoring of Generalized Myasthenia Gravis With Passive and Active Digital Biomarkers

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PP01.134 When One Diagnosis Was Not Enough: A Rare Neuromuscular Case Evolving Into Triple Trouble

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PP01.135 Precision Nutrition in Neuromuscular Diseases: Influencing Factors and Feasibility of a Protein-Optimized Intervention

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PP01.136 CPET Validity and Aerobic Training Feasibility in Oculopharyngeal Muscular Dystrophy

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PP01.137 A Transcriptomics-Based Biomarker for Muscle Quality Measures BMN 351 Treatment Response in DMD Patient Biopsies

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PP01.138 Personalised Neuromuscular Models Driving New CMS Therapeutics

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PP01.139 Recurrent Homozygous SGCA Splice-Site Variant c.37+6T>C in Indian Patients with Limb-Girdle Muscular Dystrophy Type 3

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PP01.140 Chronological and Spatial Patterns of Muscle Fat Replacement in FHL1-Related Myopathies

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PP01.141 Progression in Myotonic Dystrophy Type 1; Following Up of Patients With Myotonic Dystrophy Type 1

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PP01.142 HARMONIA: A Global Phase 3 Trial Assessing the Efficacy and Safety of Z-basivarsen in DM1

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PP01.143 Somatic Repeat Expansion Rate Is Higher in DM1 Patients With a Faster Muscle Weakness Progression

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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

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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

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PP01.149 Mitochondrial Dysfunction in Phosphaturic Mesenchymal Tumor-Induced Myopathy and Various Clinical Presentations of Phosphaturic Mesenchymal Tumors

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PP01.150 Clinical and Genetic Profiling of Multisystemic Proteinopathies: Insights From Indian Cohort

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PP01.151 Occult and Overt Myopathy in Adult Cystinosis: Clinical Characterization and Longitudinal Assessment

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PP01.152 Fbxo30/MUSA1 is a Novel Critical Regulatory Element for Z-Line Homeostasis and Skeletal Muscle Function

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PP01.153 Amyloid Myopathy: An Underrecognized but Potentially Treatable Condition

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PP01.154 Peripheral Nerve Involvement in Biallelic SCA27B: A Multicenter Study

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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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PP01.158 Adimanebart (ARGX-119): A First-in-Class, Humanized, Agonistic Monoclonal Antibody With Therapeutic Potential in Neuromuscular Junction Disorders

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PP01.159 Safety and Efficacy of Claseprubart, an Active C1s Inhibitor, in Patients with Generalized Myasthenia Gravis

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PP01.160 Potential Mechanistic Advantages of aC1s Targeting in Myasthenia Gravis: Upstream Versus Downstream Complement Blockade

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PP01.161 Value of Plasma Neurofilament Light Chain for Monitoring Efficacy in Presymptomatic Spinal Muscular Atrophy Children

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PP01.162 Telitacicept Use in Children With Ocular Myasthenia Gravis: A Case Series Report

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PP01.163 Janus Faces of Plectin: A Unified Pleiotropic Neuromuscular Disorder

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PP01.164 Challenging Occam's Razor in Neuromuscular Genetics: Coincident Molecular Diagnoses in Two Patients

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PP01.165 Late-Onset Limb-Girdle Phenotype Caused by a Homozygous COLQ Mutation: A Case Report

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PP01.166 In Vivo Effects of a MuSK Agonist Antibody Against Mutation-Specific Congenital Myasthenic Syndromes

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PP01.167 The First Korean Case of Congenital Myasthenic Syndrome 10 Responsive to Ephedrine Therapy

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PP01.168 The Epidemiology of Congenital Myasthenic Syndromes: A Comparison of Diagnosed and Genetic Prevalence

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PP01.169 Sustained Clinical Efficacy and Long-Term Safety of Intravenous Efgartigimod for gMG: ADAPT NXT Part B

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PP01.170 Impact of Time Since Diagnosis on Inebilizumab Efficacy: Post-Hoc Phase 3 MINT Trial Data Analysis

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PP01.171 Safety and Efficacy of rese-cel, an Autologous CD19-CAR T Cell Therapy, in Generalized Myasthenia Gravis

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PP01.172 Gender Differences in Myasthenia Gravis in a Neuromuscular Reference Center

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PP01.173 Exploring Alternative Operational Definitions for Refractory Myasthenia Gravis Using Real-World Data From MGBase Registry

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PP01.174 Prevalence and Risk Factors of Perioperative Myasthenia Gravis Exacerbation: A Single-Center Study in Thailand

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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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PP01.177 Assessing Relationship Between IgG-Levels and Infection Rate in Nipocalimab-Treated Patients with Generalized Myasthenia Gravis (Vivacity-MG3)

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PP01.178 Efficacy and Safety of Subcutaneous Efgartigimod PH20 in Adults With oMG: ADAPT OCULUS Interim Results

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PP01.179 Individual Item-Level Analyses of Myasthenia Gravis-Specific Outcomes During Zilucoplan Treatment in RAISE and RAISE-XT

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PP01.180 Morphological and Quantitative Analysis of Neuromuscular Junction Pathology in AChR Antibody-Positive Myasthenia Gravis

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PP01.181 Thiol–disulfide Homeostasis in Patients with Myasthenia Gravis: Association With Disease Severity and Clinical Parameters

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PP01.182 Treatment of Newly Diagnosed AChR - Myasthenia Gravis with Rituximab

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PP01.183 Management of Severe Paediatric Guillain-Barré Syndrome Due to Post-Traumatic Stress Disorder: Case Presentation

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PP01.184 Management of Paediatric Guillain-Barré Syndrome: Case Presentations

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PP01.185 Favorable Outcome in a 2-Year-Old Girl With Anti-MuSK Myasthenia Gravis. a Rare Paediatric Case Presentation

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PP01.186 Effect of Rozanolixizumab on Ocular Symptoms in Generalised Myasthenia Gravis: Analysis of Phase 3 Studies

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PP01.187 Neisseria Mucosa Sepsis in a MG Patient Treated With Ravulizumab: Case Report and Literature Review

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PP01.188 Timing of Thymic and Extrathymic Malignancies Relative to Myasthenia Gravis Onset: Oxford Myasthenia Service Cohort

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PP01.189 Effect of Nipocalimab on Sustained Myasthenia Gravis Control During Infections: Post-hoc Analysis of Vivacity-MG3 Study

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PP01.190 Follow Up and Management of Patients With Myastheni a Gravis

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PP01.191 Real-world Oral Glucocorticoid Dosage and Healthcare Resource Use in gMG Patients Starting Efgartigimod in Japan

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PP01.192 Exploring Associations Between Patient Factors and Diagnostic Delay in Myasthenia Gravis at a Tertiary Centre

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PP01.193 Efgartigimod in Acetylcholine Receptor Antibody Seronegative Generalized Myasthenia Gravis: Initial Results of ADAPT SERON

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PP01.194 Real-World Cardiovascular Burden Among Myasthenia Gravis Patients Across the United States, France, and Japan

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PP01.195 Quality of Life in Myasthenia Gravis: *Post Hoc* Pooled Analysis of Phase 3 Rozanolixizumab Studies

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PP01.196 Clinical Factors Associated With Quality of Life in Patients With Myasthenia Gravis

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PP01.197 Thymic Hyperplasia in Myasthenia Gravis: Clinical and Lifestyle Determinants and Long-Term Outcomes in Nordic Countries

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PP01.198 Single Center Real-World Experience With Ravulizumab for Patients With Generalized Myasthenia Gravis in Japan

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PP01.199 Preclinical Characterization of MGAC-007, a First-in-Class Antigen Drug Conjugate for AChR Myasthenia Gravis

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PP01.200 Clinical Utility of Therapeutic Drug Monitoring of Mycophenolic Acid in Patients with Myasthenia Gravis

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PP01.201 Effect of Rozanolixizumab on MG-ADL and QMG Items: A Post-Hoc Phase 3 Final Pooled Analysis

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PP01.202 Real-World Evidence on Utilization and Clinical Outcomes of Efgartigimod in Patients With Myasthenia Gravis

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PP01.203 Novel Therapies in Generalized Myasthenia Gravis: Real-World Experience of Zilucoplan in Spain

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PP01.204 Exploring the Role of Motor Unit Number Index (MUNIX) in Myasthenia Gravis

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PP01.205 Environmental Risk Factors for Thymoma-Associated Myasthenia Gravis

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PP01.206 Minimal Symptom Expression With Claseprubart, an Active C1S Inhibitor, in Patients With Generalized Myasthenia Gravis

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PP01.207 Successful Off-Label Treatment of Aggressive Juvenile Seronegative Generalized Myasthenia Gravis With Efgartigimod Alfa

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PP01.208 Assessing Relationship Between Immunoglobulin-G Level and Nipocalimab Efficacy using Myasthenia Gravis-Activities of Daily Living Scale

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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)

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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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PP01.211 Multiple Responder Analyses With Claseprubart, an Active C1s Inhibitor, in Patients With Generalized Myasthenia Gravis

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PP01.212 Clinical and Immunological Features of Thymoma Patients With and Without Myasthenia Gravis: A Pilot Study

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PP01.213 A Case of Bulbar-Onset Motor Neuron Disease Emerging in AChR-Positive Myasthenia Gravis

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PP01.214 Characterisation of Patients With Generalised Myasthenia Gravis on Zilucoplan Treatment in European Managed Access Programmes

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PP01.215 Steroids Tapering Strategies in Patients With Generalized Myasthenia Gravis Initiating Efgartigimod: STRIVE-gMG Interim Results

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PP01.216 Early FcRn Blockade with Efgartigimod in Generalized Myasthenia Gravis: A German Multicenter Real-World Study

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PP01.217 Myasthenia Gravis in Armenia: Real-World Diagnostic Pathways, Treatment Patterns, and System-Level Gaps

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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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PP01.219 Efgartigimod Improves Ability to Work and Patient Satisfaction in gMG: Initial Results From PREMIER

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PP01.220 Predictor of Conversion of Myasthenic Crisis from Impending Myasthenic Crisis- a Prospective Study

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PP01.221 Complement Inhibitor Use in an Adolescent With Refractory Juvenile-Onset, Thymomatous, Antibody-Positive Myasthenia Gravis

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PP01.222 Myasthenia Gravis During Pregnancy: A Descriptive Real-World Study Based on US Claims Data

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PP01.223 Design of Phase 2a Study Evaluating Empasiprubart Add-On Therapy to Efgartigimod in Adults With gMG

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PP01.224 Hereditary Spastic Paraplegia Associated With *SPTAN1* Mutation

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PP01.225 Myasthenia Gravis Mimicking Miller Fisher Syndrome

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PP01.226 Bruton's Tyrosine Kinase Expression in Peripheral Blood and Thymus of Patients with Myasthenia Gravis

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PP01.227 A Pilot Machine Learning Study on Muscle-Specific Diagnostic Contribution of Repetitive Nerve Stimulation in MG

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PP01.228 RELIEVE: A Phase 3 Study Evaluating Efficacy and Safety of Remibrutinib in Generalized Myasthenia Gravis

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PP01.229 Usability of Zilucoplan Auto-Injector in Generalised Myasthenia Gravis: A Human Factors Validation Study

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PP01.230 Hospitalisation, Crisis and ICU Trends in Myasthenia Gravis: Nationwide Study, Spain 2016–2022

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PP01.231 Characterisation of Patients With Generalised Myasthenia Gravis on Rozanolixizumab Treatment in European Managed Access Programmes

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PP01.232 Do We Really Need Non-Steroidal Immunosuppressants in Generalized Myasthenia Gravis Treated With New Targeted Therapies?

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PP01.233 Prevalence and Predictors of Sleep Disturbances in anItalian Cohort of Patients With Myasthenia Gravis

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PP01.234 Impact of Published Single-Fiber EMG Reference Values on Diagnostic Classification in Myasthenia Gravis

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PP01.235 Efficacy of Nipocalimab in Patients with Myasthenia Gravis and Lower Baseline Activity of Daily Living

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PP01.236 Efficacy of Nipocalimab in Patients Early in Their Disease Course of Generalized Myasthenia Gravis

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PP01.237 Treatment and Outcomes in a Real-World Australian Myasthenia Gravis Cohort: Baseline Findings from VALUE-MG

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PP01.238 Rituximab in Myasthenia Gravis: A Real-World Study Using Inverse Probability of Treatment Weighting

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PP01.239 A New Clinical Entity: Retrospective Case Study of Immune Checkpoint Inhibitor Induced Myasthenia Gravis

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PP01.240 Clinical Features in Patients With Neuroimmunological Disorders Associated With Antibodies to Voltage-Gated Calcium Channel

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PP01.241 Genetic Landscape and Innovative Therapeutic Approaches Concerning a Large Algerian Series of Congenital Myasthenic Syndromes

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PP01.242 Application of Gait Training Robots According to the Functional Level of Patients With Guillain-Barré Syndrome

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PP01.243 Beyond the Nerve: Uncovering Skeletal Muscle Involvement in *SORD* Neuropathy

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PP01.244 Novel *SIGMAR1* Missense Variant: A Case Report and Literature Review

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PP01.245 Acute Brachial Plexopathy as a Presenting Feature for Hereditary Neuropathy With Liability to Pressure Palsies

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PP01.246 Infra-Clinical Small-Fiber Impairment in Hereditary Transthyretin Amyloidosis with Polyneuropathy: A Latin American Phenotypic Study

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PP01.247 Toward Standardized Multidisciplinary Assessment of Charcot-Marie-Tooth Disease: Integrating MUNIX, Clinical Scales and Plasma Biomarkers

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PP01.248 Nerve Ultrasound, Clinical and Electrophysiological Characterization of Pediatric Neuralgic Amyotrophy, HNPP and Mononeuropathies

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PP01.249 Hereditary Neuropathy With Liability To Pressure Palsy (HNPP) Presenting As Bilateral Long Thoracic Nerve Injuries

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PP01.250 Plasma Phosphorylated Tau181 in Amyotrophic Lateral Sclerosis: Relationships With Clinical Measures and Disease Progression

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PP01.251 A Longitudinal Evaluation of Cardiovascular Autonomic Reflex Testing in Hereditary Transthyretin Amyloidosis

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PP01.252 Investigating Sympathetic Small Fibers Using the Skin Wrinkle Test in Patients with Amyotrophic Lateral Sclerosis

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PP01.253 Nocturnal Oximetry in Neuromuscular Diseases

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PP01.254 Historical Notes on Spinocerebellar Ataxia Type 2. Contributions from Cuban Physicians

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PP01.255 Late-Onset Hereditary Transthyretin Amyloidosis (Attr) with Polyneuropathy in an Elderly Person

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PP01.256 CMT-1C c.334G>A LITAF Mutation: A Case Report of Clinical Features and Electrodiagnostic Findings

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PP01.257 Single-Centre Experience on Genotypic and Phenotypic Features of Southern Brazilian Patients With Hereditary Neuropathies

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PP01.258 Multidimensional Analysis of hATTR Polyneuropathy Burden: A Latin American Cohort Study

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PP01.259 The Genetic Profile of Hereditary Neuropathies in Southern Africa

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PP01.260 Focal CIDP Variants with Brachial Plexus Involvement Compared to Neuralgic Amyotrophy – Similarities and Differences

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PP01.261 Riliprubart in CIDP: Time and Magnitude of Response Analysis from a Phase 2 Trial

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PP01.262 Efgartigimod in Adults With Active Idiopathic Inflammatory Myopathy: The ALKIVIA+ Phase 3 Open-Label Extension Trial

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PP01.263 Lateral Dorsal Cutaneous Sural Entrapment - an Uncommon Neuralgic Syndrome of the Foot

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PP01.264 High Dose Intravenous Immunoglobulin for Multifocal Motor Neuropathy Resulting in Hyperviscosity and Digital Ischaemia

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PP01.265 Phase 2 Trial of Riliprubart in CIDP: Patient-reported Quality-of-life, Pain and Fatigue Outcomes at Week-76

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PP01.266 Clinical and Metagenomic Characterization of Gut Microbiome in Guillain-Barré Syndrome

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PP01.267 Riliprubart Phase 2 Trial Results: Efficacy Analysis Using Immune Neuropathy-No Evidence of Disease Activity Framework

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PP01.268 CAPTIVATE Trial Design: A Pivotal Phase 3 Study of Claseprubart in Chronic Inflammatory Demyelinating Polyneuropathy

Prof. Eduardo Nobile-Orazio[8]

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PP01.269 Impact of Efgartigimod on Inflammatory Neuropathy Cause and Treatment Scores: ADHERE/ADHERE+ *Post Hoc* Analysis

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PP01.270 Multifocal Intraneural Perineurioma Mimicking Acquired Demyelinating Neuropathy

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PP01.271 Immune-mediated Sensory Predominant Polyradiculoneuropathy Initially Mimicking Myelopathy

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PP01.272 Chronic Inflammatory Demyelinating Polyradiculoneuropathy in France (REVEAL-CIDP): Healthcare Resource Use and Disease Management

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PP01.273 Cytokine Profiles Associated With IgG4 Class Switching in NF-155 Antibody–Positive Autoimmune Nodopathy

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PP01.274 CASE REPORT: Chronic Inflammatory Demyelinating Polyneuropathy Initially Misdiagnosed as Subacute Paraneoplastic Polyneuropathy

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PP01.275 The Spectrum of Polyneuropathies and Polyradiculoneuropathies Requiring Recurrent Inpatient Admissions Over a 9-Year Period

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PP01.276 Beyond Nonsystemic Vasculitic Neuropathy: Evidence for a PNS–CNS Vasculitis Spectrum

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PP01.277 A Case of Acute Bickerstaff Brainstem Encephalitis: Diagnostic Challenges and Therapeutic Response to Early Immunotherapy

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PP01.278 Intravenous Immunoglobulin to Subcutaneous Efgartigimod PH20 Transition in CIDP: A Phase 4 Study in Progress

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PP01.279 Understanding the Patient Journey in Multifocal Motor Neuropathy (MMN): Descriptive Insights From the iMMersioN Study

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PP01.280 Combined Cyclophosphamide and Rituximab as Rescue Therapy in Atypical CIDP Refractory to First-Line Immunotherapy

Dr. Laura Kennelly

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PP01.281 A Case of Acute Flacid Paralysis in a Child With Kaposiform lymphangiomatosis/ Kasabach–Merritt Phenomenon

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PP01.282 Long-Term Safety and Efficacy Data of Empasiprubart in Multifocal Motor Neuropathy: Phase 2 ARDA+ Study

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PP01.283 Telemedicine-Based INCAT Assessment in CIDP: Reliability and Agreement Between Nurse and Neuromuscular Expert Virtual Evaluations

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PP01.284 Empasiprubart Versus Placebo (EMNERGIZE) or Immunoglobulin (EMVIGORATE) in Chronic Inflammatory Demyelinating Polyradiculoneuropathy: Study Designs

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PP01.285 ORBIT-CIDP: A Real-World CIDP Study Leveraging Digital and AI-Powered Patient-Centric Innovation

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PP01.286 Long-Term Purity Profile of IQYMUNE®: A Direct Result of Quality by Design

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PP01.287 Double-Positive, Double Trouble: Anti–NF186 and Anti-FGFR3 Seropositivity in an Atypical Immune Neuropathy

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PP01.288 Easily Missed: A Case Series of Lewis-Summer Syndrome

Asst. Prof. Sophie Teng[1]

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PP01.289 Evaluation of the Effect of Empasiprubart on Nerve Morphology in Multifocal Motor Neuropathy: Study Design

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PP01.290 Giant Nerve Root Hypertrophy and Pseudofroin's Syndrome in Chronic Inflammatory Demyelinating Polyneuropathy

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PP01.291 Electrophysiological Evaluation of Oropharyngeal Dysphagia in Chronic Inflammatory Demyelinating Polyneuropathy

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PP01.292 Acute Ophthalmoplegia, Facial Weakness and Paraesthesia After Sea-Whelk Consumption – a Case of Paralytic Shellfish Poisoning

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PP01.293 Metabolic Variability and Inflammation-Derived Biomarkers for Diabetic Polyneuropathy Risk Prediction: A Systematic Review

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PP01.294 Multimodal Neurophysiological Correlates of Disease Severity in Diabetic Peripheral Neuropathy

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PP01.295 A Case of Ado-Trastuzumab Emtansine induced Nerve Vasculitis

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PP01.296 Sural-to-Radial Amplitude Ratio (SRAR) in Patients with Diabetic and Chemotherapy-Induced Polyneuropathy

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PP01.297 Chronic Ataxic Neuropathy With Disialosyl Antibodies Responsive to Zanubrutinib

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PP01.298 The Significance of Carpal Tunnel Syndrome for Diagnosing AL-Amyloidosis Associated With Multiple Myeloma

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PP01.299 Intravascular Large B-Cell Lymphoma Mimicking Guillain–Barré Syndrome

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PP01.300 Immunoglobulin Light-Chain Amyloid Neuropathy Mimicking a Lower Motor Neuron Disorder: A Case Report

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PP01.301 POEMS Syndrome: A Multisystem Presentation

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PP01.302 A Diagnostic Odyssey of Paraneoplastic Sensory Neuronopathy

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PP01.303 Prevalence and Phenotypic Correlates of Vestibulopathy in Peripheral Neuropathy and Ganglionopathy

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PP01.304 Ischemic Neuropathy Possibly as a Complication of Percutaneous Coronary Intervention: A Case Report

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PP01.305 The Muscle Cramp Impact Index: A Patient-Centered Scale for The Assessment of Muscle Cramps

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PP01.306 Ultrasonographic Localization of Ulnar Neuropathy With Normal or Isolated Sensory Nerve Conduction Studies

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PP01.307 Meralgia Paraesthetica Associated with Weight Loss

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PP01.308 Neuromuscular Involvement in Connective Tissue Disease

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PP01.309 Unveiling CTS: Ultrasound Detection of Median Neuropathy Despite Normal NCS - A Case Report

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PP01.310 Vessel Wall Characteristics in Compressive Trigeminal Neuralgia and Its Correlation With Vascular Risk Factors

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PP01.311 Machine Learning-Based Skin Nerve Morphometry for Diabetic Neuropathy: Diagnostic and Clinical Implications

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PP01.312 Small Fiber Neuropathy and Autonomic Dysfunction in Sjogren's Syndrome

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PP01.313 Virtual Emergency Care Use in Neuromuscular Disease: Foundations for Multidisciplinary Support

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PP01.314 Risks and Benefits of Gastrostomy in Non-Motor Neurone Disease Progressive Neurological Diseases: A Systematic Review

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PP01.315 Immersive Technology and AI Voice Synthesis: Advancing Equity in Motor Neuron Disease

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PP01.316 Evaluation of the Efficacy of Home-Based tDCS in ALS Patients: A Randomized, Placebo-Controlled Clinical Trial

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PP01.317 Spontaneous Resolution of Cognitive Symptoms in Morvan Syndrome: A Case Report and Literature Review

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PP01.318 Dermal Fibroblasts From Amyotrophic Lateral Sclerosis Patients as a Tool to Study Autophagy-Lysosome Pathway Alterations

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PP01.319 Young-Onset Amyotrophic Lateral Sclerosis at a Tertiary Center in Korea

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PP01.320 Dysphagia in Patients With Spinocerebellar Ataxia Type 8-Related Amyotrophic Lateral Sclerosis and in SCA8-Mouse Models

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PP01.321 Juvenil Amyotrophic Lateral Sclerosis in Two Algerian Sisters Expends the Phenotypic Spectrum of SYNE1-Related Disorders

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PP01.322 Swallowing Function in Patients With SOD1-Related Amyotrophic Lateral Sclerosis

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PP01.323 Analysis of Vocal Parameters in Als Patients: A Preliminary Study

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PP01.324 Ten-Year Trajectories of Pre-diagnostic Medical Conditions Preceding Amyotrophic Lateral Sclerosis: A Nationwide Korean Study

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PP01.325 5q-Associated Spinal Muscular Atrophy in Austria: Epidemiology and Co-Morbidities

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PP01.326 Increasing Prevalence of Amyotrophic Lateral Sclerosis in Austria Correlates With Reduced Mortality

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PP01.327 Incidence and Prevalence of Amyotrophic Lateral Sclerosis in Curitiba, Brazil

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PP01.328 Short-Term Outcome of Tofersen Treatment in Patients with SOD1-ALS at a Tertiary Academic Center

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PP01.329 Preclinical Safety and Efficacy of Intrathecal INS1202 AAV9-SOD1-shRNA in Mice and Nonhuman Primates

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PP01.330 Quantitative Assessment of Motor Band Sign as a Neuroimaging Biomarker in Amyotrophic Lateral Sclerosis

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PP01.331 Identification of Glial Biomarkers in ALS Using Single Nucleus Transcriptomic analysis

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PP01.332 Autonomic Biomarkers Associated With Disease Severity and Duration in Amyotrophic Lateral Sclerosis

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PP01.333 Choroid Plexus Enlargement Is Associated With Disease Progression in Amyotrophic Lateral Sclerosis

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PP01.334 Electrophysiological Origin of Repeater F-waves and Their Correlation with Clinical Severity in Polio Survivors

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PP01.335 Adult SMA REACH: Safety and Efficacy Profiles of Nusinersen and Risdiplam in Adults With SMA

Miss Elena Karkkainen

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PP01.336 UK SMA Patient Registry: Characterise Disease Burden and Treatment Impact in Spinal Muscular Atrophy

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PP01.337 Adult SMA REACH: A Ready-Made Infrastructure to Support Research and Improvement Initiatives in SMA

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PP01.338 Experience of Algeria to Diagnosis and Management Adult Spinal Muscular Atrophy

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PP01.339 Understanding Neurologist Perspectives on the Clinical Meaningfulness of 'Any-Point Differences' on the HFMSE in SMA

Dr. Leslie Nelson[1]

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PP01.340 Design of a Non-interventional Study to Assess Patient Perspectives Inspinal Muscular Atrophy (SMA-Perceptions Study)

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PP01.341 Assessment and Management of Bulbar Function in Spinal Muscular Atrophy in Spain

Dr. Paz Castro

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PP01.342 DYNC1H1-Related Spinal Muscular Atrophy With Arthrogryposis and Congenital Fractures

Dr. Evgeniya Melnik[1]

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PP01.343 RAINBOWFISH: 3-Year Efficacy and Safety Data of Risdiplam in Infants With Presymptomatic Spinal Muscular Atrophy

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PP01.344 Clinical Outcomes of SMA Type 1 Patients Treated With Zolgensma in Brazil Public Health System

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PP01.345 SMA Type 1 Diagnosed by Newborn Screening Post Disease-Modifying Treatments in Hong Kong Chinese Children

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PP01.346 Indirect Treatment Comparison of OAV101 IT vs Risdiplam and Nusinersen in SMA DMT-Experienced Patients

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PP01.347 Indirect Treatment Comparisons of OAV101 IT vs Risdiplam and Nusinersen in SMA DMT-Naïve Patients

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PP01.348 Neuron-Specific Lrp4 Overexpression Rescues Impaired Neuromuscular Junction Formation via BMP Signaling in Drosophila SMA Model

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PP01.349 Histopathology in Mitochondrial Cardiomyopathies

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PP01.350 Blink Reflex Recording as a Diagnostic Tool for Facial Involuntary Movements

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PP01.351 Long Exercise Test: A Simple Diagnostic Tool for the Etiological Evaluation of Rhabdomyolysis

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PP01.352 Diagnostic Accuracy of Quantitative Electromyography in Idiopathic Inflammatory Myopathies: A Comparative Analysis with Muscle Biopsy

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PP01.353 Electrophysiological Characteristics of Subclinical Diabetic Polyneuropathy in Adolescents with Type 1 Diabetes Mellitus

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PP01.354 Clinical and Electrophysiological Aspects About Two Cases of Parsonage Turner Syndrom

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PP01.355 Longitudinal Electrophysiological Changes Predict Clinical Outcome in Chronic Inflammatory Demyelinating Polyradiculoneuropathy

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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

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PP01.357 A Spectrum of Rapid-Onset Motor–Bulbar Disability and Tocilizumab Treatment Outcomes in *RANBP2*-ANE1

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PP01.358 Personalized Medicine From Birth: Nationwide Newborn Screening for Spinal Muscular Atrophy in Serbia

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PP01.359 Advancing Clinical Trials in Myotonic Dystrophy Type 1: Refining Radiological, Clinical and Patient-Reported Outcome Measures

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PP01.360 Responsiveness to Change of Quantitative Whole-Body MRI in FSHD: Results From ReDUX4 OLE

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PP01.361 Expanding Spectrum of Molecular Landscape of Nemaline Rod Myopathy

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PP01.362 Increase of Number and Genic Type of eccDNA in Spinal Cord of ALS Murine Model

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PP01.363 Exploring MSOT as a Non-Invasive Biomarker for Myotonic Dystrophy Type 2

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PP01.364 Orthogonal Methods Demonstrate Pharmacodynamic Responses in Duchenne Muscular Dystrophy Patients Treated with BMN 351

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PP01.365 Muscle Mass and Strength Changes in Patients With Peripheral Neuropathy and Central Nervous System Disorders

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PP01.366 STEP Study Design: Validating the Syde® Device to Measure Motor Function in Late-Onset Pompe Disease

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PP01.367 LEOPARD-DMD: Interval Results from a Duchenne Muscular Dystrophy Natural History Study

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PP01.368 Smartphone-Based Assessment of Shoulder Motion in Patients With Facioscapulohumeral Muscular Dystrophy

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PP01.369 Evaluation of Ambulation With a Wearable Sensor in Patients With CMT

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PP01.370 Synthetic Data as a Research Enabler in CIDP: A Registry-Based Validation Study

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PP01.371 Non-Physician–Administered Neuropathy Symptom and Disability Scores for Screening of Diabetic Distal Symmetric Polyneuropathy

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PP01.372 Revolutionizing Gait Quality Monitoring in People with CIDP: An Instrumented Shoe Insole Solution

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PP01.373 Transitory Symptom Worsening in Facioscapulohumeral and Becker Muscular Dystrophies: Do They Exist and Matter?

Dr. Nurit Birman Har-Noy[1]

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PP01.374 Efficacy of Anti-Osteoporotic Drugs and Vitamin D/calcium Supplementation in NMD: A Systematic Review

Dr. Sara Liguori[1]

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PP01.375 Chronic Pain and Postural Instability in Individuals With Neuromuscular Diseases: A Cross-Sectional Study

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PP01.376 Musculoskeletal Impairment in Adults With Neurofibromatosis Type 1: An Observational Study

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PP01.377 The Role of Magnesium in Muscle Health and Neuromuscular Diseases

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PP01.378 Clinical and Functional Characterization of a Pediatric Cohort With Neurofibromatosis Type 1

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PP01.379 Multidimensional Evaluation of Functioning in a Pediatric Population With X-linked Hypophosphatemic Rickets: Observational Study

Dr. Sara Liguori[1]

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PP01.380 Investigation of Dysphagia Risk and Chewing Function in SMA Type II: A Cross-Sectional Descriptive Study

Miss Ceren Şevval KARATAŞ[3]

[3]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[1,2]

[1]Tel Aviv Sourasky Medical Center, Tel Aviv, Israel. [2]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[2]

[2]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 Activatingmicroglia 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[1]

[1]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[1,2]

[1]Graduate Institute of Clinical Medical Sciences, College of Medicine, Chang Gung University, Taoyuan City, Taiwan. [2]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

CYTOO 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[1]

[1]University of Deusto, Bilbao, Spain

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

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13:15–14:15

Room 1
Spadolini Attic

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- The pathophysiological mechanisms underlying TK2d, including the role of nucleoside metabolism and mitochondrial DNA maintenance
- 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 Domínguez-González

University Hospital 12 de Octubre
Madrid, Spain

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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.

TK2d, thymidine kinase 2 deficiency.

19th International Congress on Neuromuscular Diseases

INDUSTRY-SUPPORTED SYMPOSIUM INVITATION

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 Saccani
University of Parma
Parma, Italy

Prof. Sabrina Sacconi
Nice University Hospital
Nice, France

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A Novartis-sponsored symposium at the International Congress on Neuromuscular Diseases
7–11 July 2026

Shaping the journey ahead

The importance of starting early in SMA

Save the date:
Friday 10 July 2026, 13:15–14:15 CEST
Spadolini Attic: Room 4, Fortezza da Basso, Florence, Italy

Please join Prof. Giovanni Baranello, Prof. Liesbeth De Waele, and Dr. Teresa Moreno, in a discussion on emerging clinical data and real-world evidence for onasemnogene abeparvovec. They will review best practice in the holistic multidisciplinary care of patients with SMA in the era of disease-modifying therapies.

Doctor Teresa Moreno
Lisbon, Portugal
Session 1: Taking the first step: Early onset of action in SMA

Professor Liesbeth De Waele
Leuven, Belgium
Session 2: Staying on track: Durability of response in SMA

Professor Giovanni Baranello (Chair)
London, UK
Session 3: Finding a voice: Bulbar outcomes in SMA

SMA, spinal muscular atrophy.

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Thursday July 9th, 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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