ICNMD 2022

17TH INTERNATIONAL CONGRESS ON NEUROMUSCULAR DISEASES

5 - 9 July 2022 Brussels, Belgium

Final Program

www.icnmd.org

#ICNMD2022

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Welcome Letter

Dear Colleagues,

On behalf of the ICNMD 2022 Organising Committee, the World Federation of Neurology and the Local Organising & Program Committees, I would like to formally welcome you to Brussels and the 17th International Congress on Neuromuscular Diseases. It is very exciting to be hosting an in-person ICNMD Congress after so many years being apart. We as a Planning Committee have spent a lot of time in building this year's Congress and are thrilled to be able to finally share it with you all. The Scientific Program is top notch. The collection of speakers and presenters are diverse, incredibly experienced and are eager to share their knowledge and ideas with you.

We are thankful to all of our sponsors and exhibitors as without their support, it would be extremely difficult to host a Congress of this size and status so I encourage you to take the time to visit with them and see what they have to show you. In addition to the exemplary science and the industry representation, there will be plenty of opportunities to network and socialize with your colleagues and peers from around the world.

All of this with the beautiful city of Brussels and its rich history and culture, vast culinary choices and endless entertainment options as the backdrop, we are certain this year's ICNMD will be one to remember.

Once again, welcome to Brussels and we hope you enjoy your stay!

Sincerely,

Gauthier Remiche

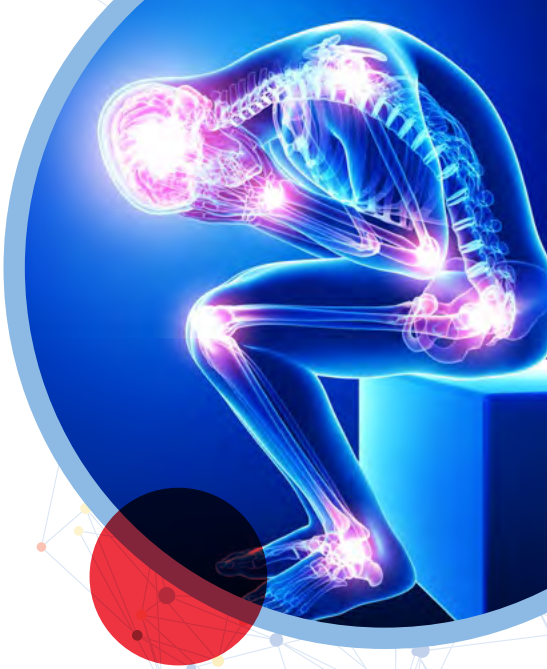
Gauthier Remiche (MD, PhD)
ICNMD 2022 Congress President
Hôpital Erasme, Université Libre de Bruxelles

We are excited to be hosting an in-person Congress in Brussels. For the health and safety and enjoyment for all delegates, ICNMD will at minimum be following the local recommendations and guidelines as outlined by the Belgian public health authority.

The use of face masks are encouraged and appreciated.

For the latest current measures, please visit
www.info-coronavirus.be/en

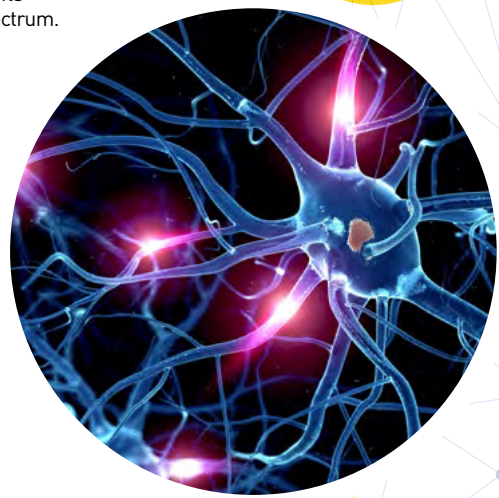
About ICNMD



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). Since 2014, the Congress has taken place in a two-year cycle.

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

Specialty Group on
Neuromuscular Diseases



James P. Dyck
ICNMD 2022 Chair
Mayo Clinic Adult Neurology and Neurosurgery
Rochester, Minnesota

ICNMD 2022 Committees

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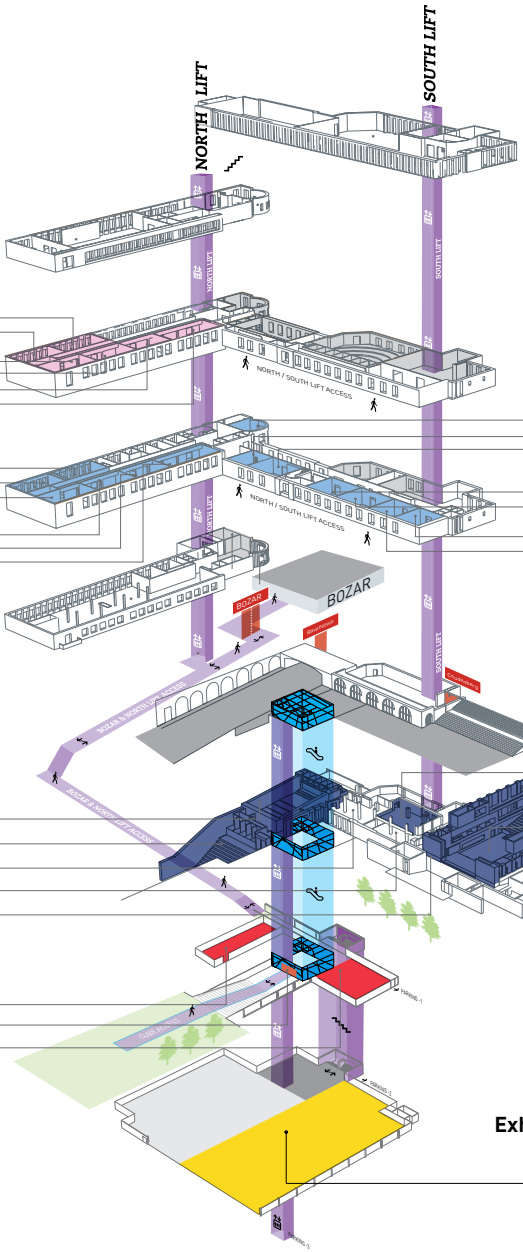
Michael Shy (Iowa City)
Andreas Steck (Basel)
Jung-Joon Sung (Seoul)
Bjarne Udd (Tamper)
Steven Vargas (CDMX)
Angela Vincent (Oxford)
Edmar Zanoteli (Sao Paulo)

Venue Floorplan

MAP OF SQUARE



LEVEL 3

Hospitality Suites

313
315
316
314
312
311
213
215
216
214
212
211

Circle 210
207
206
204
202
201 A
201 B

LEVEL 2

Scientific Sessions & Hospitality Suites

LEVEL 0

Scientific Sessions

Silver Hall
Copper Hall
Silver Foyer
Magritte Foyer
Delvaux Foyer

Magritte Lounge
Speaker Ready Room
Gold Hall

Copper Foyer
Glass Entrance
Registration Hall

LEVEL -1

Entrance & Registration Hall

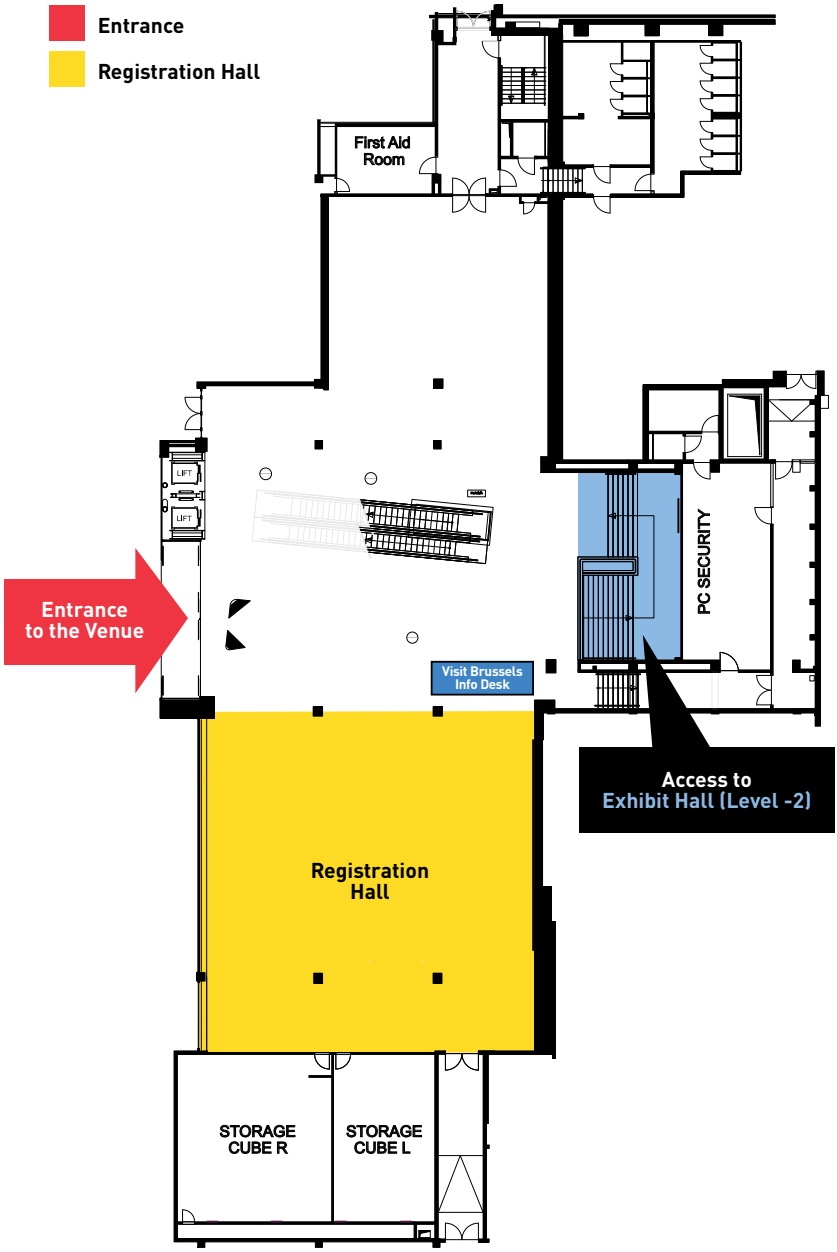
LEVEL -2

Exhibit Hall, ePoster Sessions and Refreshment Breaks

Grand Hall 2

Venue Floorplan

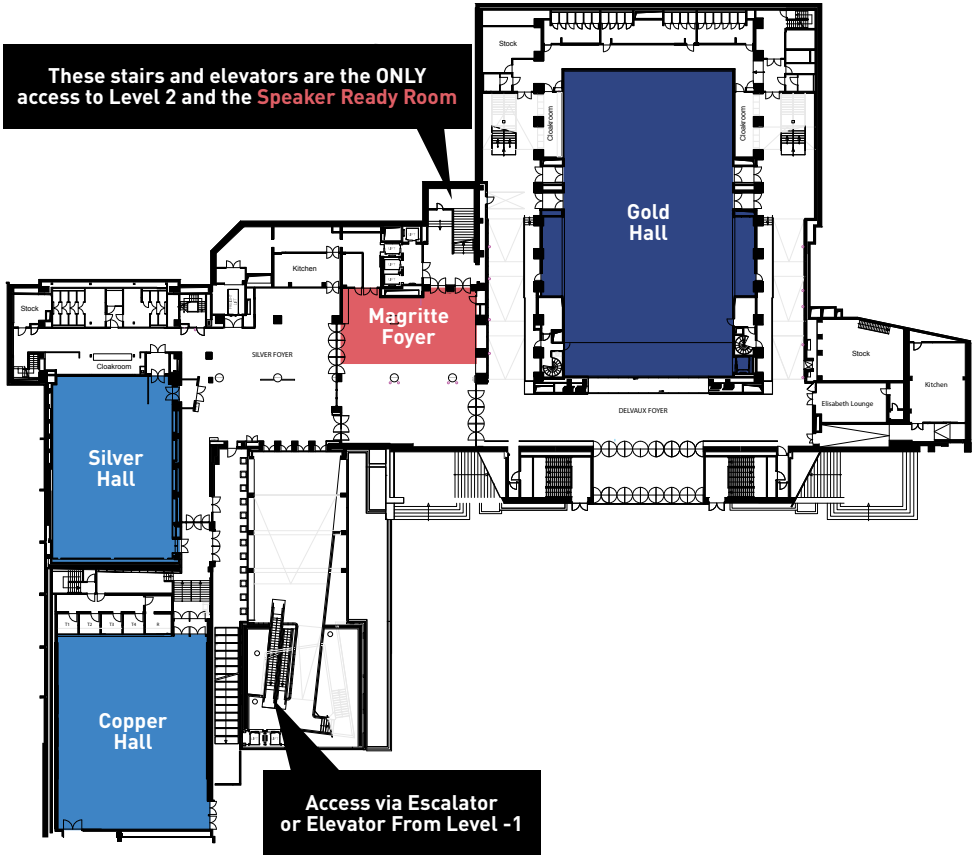
LEVEL -1



Venue Floorplan

LEVEL 0 - FOYER

- Plenary
- Speaker Ready Room
- Session Room

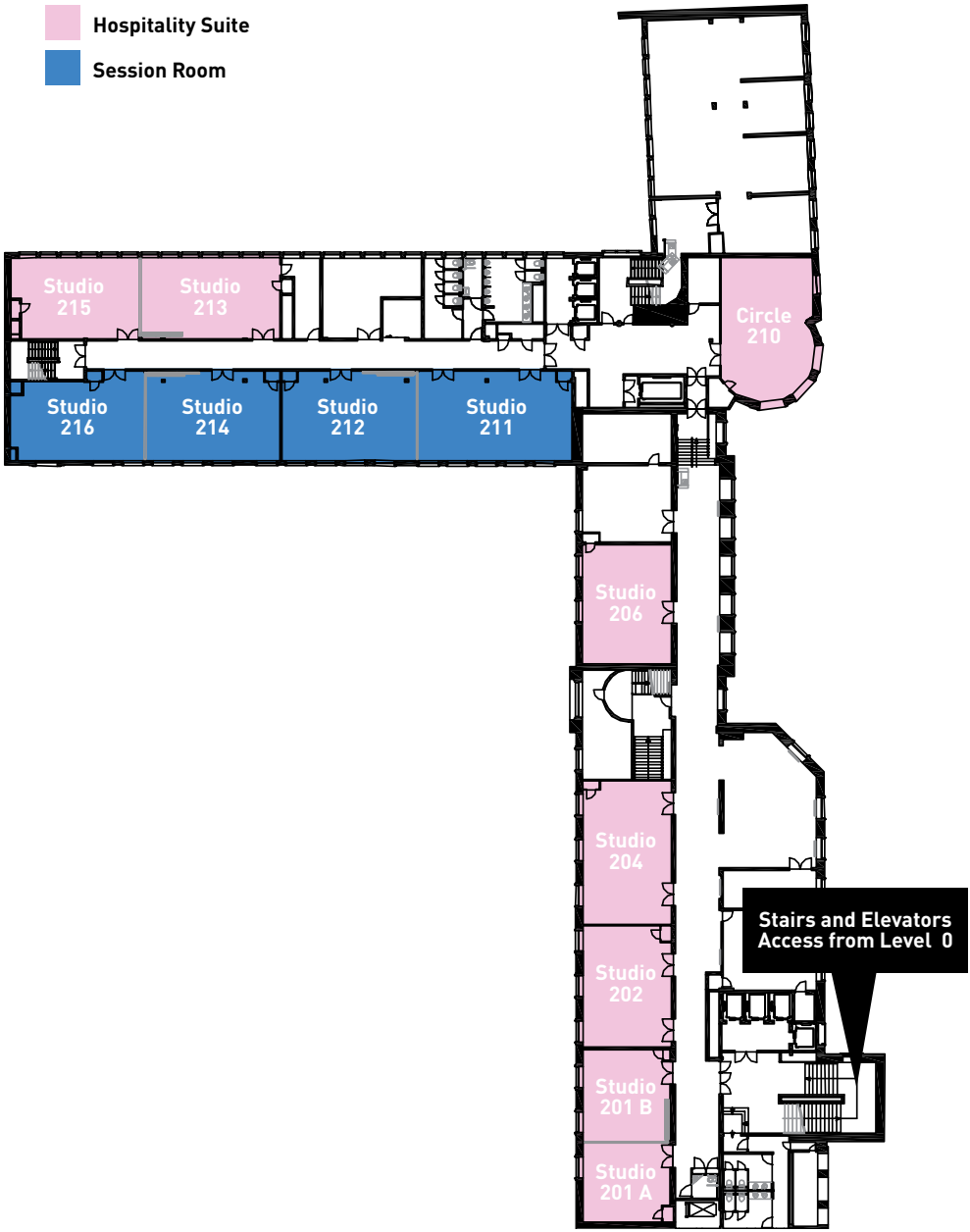


Venue Floorplan

LEVEL 2

Hospitality Suite

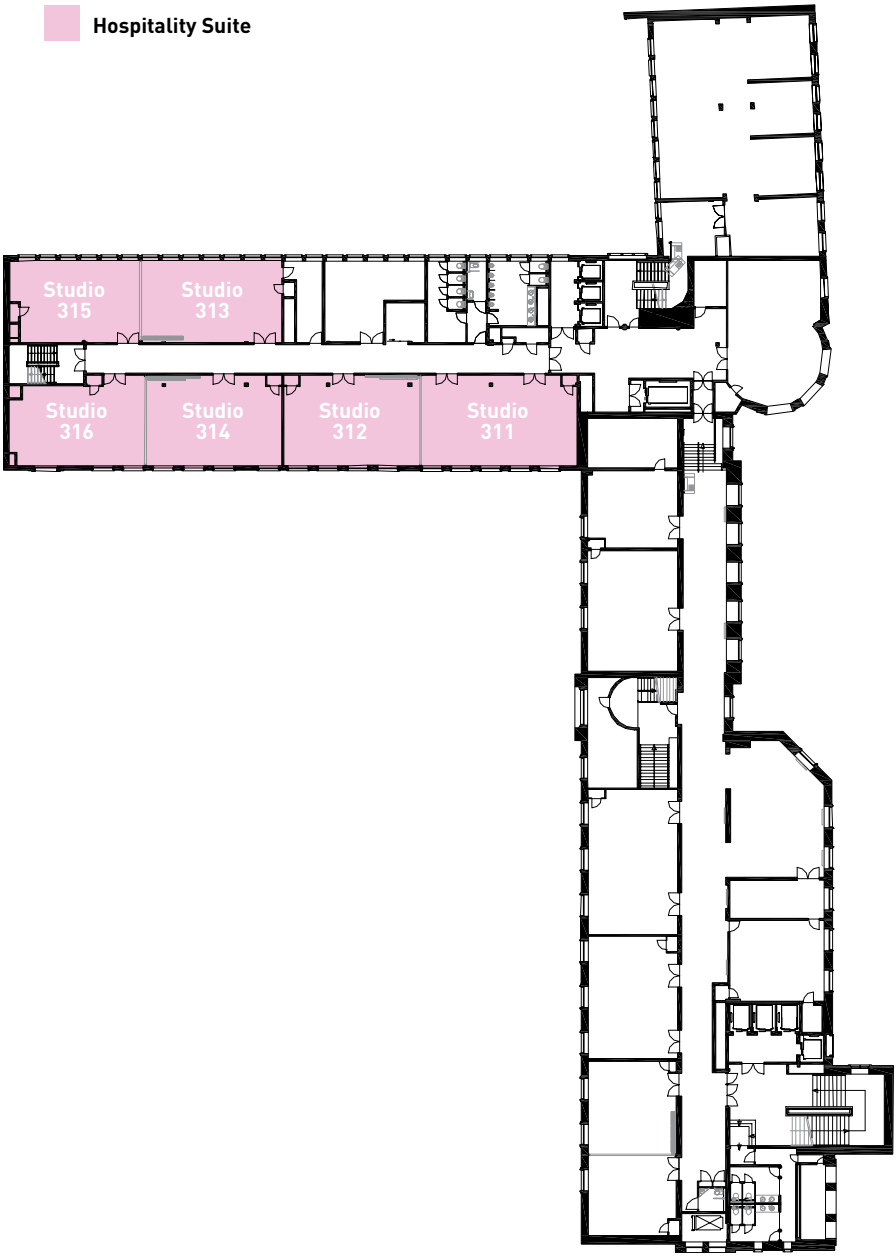
Session Room



Venue Floorplan

LEVEL 3

Hospitality Suite



Program at a Glance

Tuesday 5 July, 2022

The official time zone for ICNMD 2022 is in UTC +2:00.

Legend

- SS: Scientific Session
- TC: Teaching Course
- WS: Workshop
- HO: Hands On Course
- OS: Overarching Session
- RS: Regional Session
- PS: Poster Session - Oral Presentations

DAY 1

HO01 Meeting Studio 202 08:00 - 12:00	**TC01** Meeting Studio 201 AB 08:00 - 12:00	**TC02** Meeting Studio 204 08:00 - 12:00	**TC03** Meeting Studio 206 08:00 - 12:00	**TC04** Meeting Studio 211+212 08:00 - 12:00
Lunch Break Meeting Studio 205 Foyer 12:00 - 13:00				
HO02 Meeting Studio 202 13:00 - 17:00	**TC05** Meeting Studio 201 AB 13:00 - 17:00	**TC06** Meeting Studio 204 13:00 - 17:00	**TC07** Meeting Studio 206 13:00 - 17:00	**TC08** Meeting Studio 211+212 13:00 - 17:00
Opening Ceremony Gold Hall 18:00 - 19:00				
Welcome Reception Level 0 Foyer 19:00 - 22:00				

For more detailed Program Information, please visit **icnmd.org** to download the Digital Program or download the official ICNMD Mobile App.

Program at a Glance

Wednesday 6 July, 2022

The official time zone for ICNMD 2022 is in UTC +2:00.

Legend

- SS: Scientific Session
- TC: Teaching Course
- WS: Workshop
- HO: Hands On Course
- OS: Overarching Session
- RS: Regional Session
- PS: Poster Session - Oral Presentations

DAY 2

PL01
Gold Hall
08:30 - 10:30

AM Break Exhibit Hall (Level -2) 10:30 - 11:15

SS02 Gold Hall 11:15 - 12:45	**SS01** Meeting Studio 211+212 11:15 - 12:45	**WS01** Meeting Studio 214+216 11:15 - 12:45	**SS03** Copper Hall 11:15 - 12:45	**WS02** Silver Hall 11:15 - 12:45
Lunch Break & ePoster Session Exhibit Hall (Level -2) 12:45 - 14:15	**Industry Supported Symposia** Meeting Sudio 211+212 / Copper Hall / Silver Hall 13:00 - 14:00			
SS06 Gold Hall 14:15 - 15:45	**SS04** Meeting Studio 211+212 14:15 - 15:45	**WS03** Meeting Studio 214+216 14:15 - 15:45	**SS05** Copper Hall 14:15 - 15:45	**OS01** Silver Hall 14:15 - 15:45

PM Break Exhibit Hall (Level -2) 15:45 - 16:15

WS04 Gold Hall 16:15 - 17:45	**WS06** Meeting Studio 211+212 16:15 - 17:45	**WS07** Meeting Studio 214+216 16:15 - 17:45	**WS05** Coppper Hall 16:15 - 17:45	**SS07** Silver Hall 16:15 - 17:45

Industry Supported Symposium
Copper Hall
18:00 - 19:00

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The EVENT APP

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Program at a Glance

Thursday 7 July, 2022

The official time zone for ICNMD 2022 is in UTC +2:00.

Legend

- SS: Scientific Session
- TC: Teaching Course
- WS: Workshop
- HO: Hands On Course
- OS: Overarching Session
- RS: Regional Session
- PS: Poster Session - Oral Presentations

DAY 3

Industry Supported Symposia
Meeting Studio 214+216 / Copper Hall
07:00 - 08:00

PL02
Gold Hall 08:30 - 10:30

AM Break Exhibit Hall (Level -2) 10:30 - 11:15

SS08 Gold Hall 11:15 - 12:45	**OS02** Meeting Studio 211+212 11:15 - 12:45	**PS05** Meeting Studio 214+216 11:15 - 12:45	**SS09** Copper Hall 11:15 - 12:45	**OS03** Silver Hall 11:15 - 12:45

Lunch Break & ePoster Session
Exhibit Hall (Level -2)
12:45 - 14:15

Industry Supported Symposia
Gold Hall / Copper Hall / Silver Hall 13:00 - 14:00

SS10 Gold Hall 14:15 - 15:45	**OS05** Meeting Studio 211+212 14:15 - 15:45	**PS01** Meeting Studio 214+216 14:15 - 15:45	**SS11** Copper Hall 14:15 - 15:45	**OS04** Silver Hall 14:15 - 15:45

PM Break Exhibit Hall (Level -2) 15:45 - 16:15

SS12 Gold Hall 16:15 - 17:45	**PS03** Meeting Studio 211+212 16:15 - 17:45	**PS04** Meeting Studio 214+216 16:15 - 17:45	**WS08** Copper Hall 16:15 - 17:45	**PS02** Silver Hall 16:15 - 17:45

Industry Supported Symposia
Copper Hall / Silver Hall
18:00 - 19:00

For more detailed Program Information, please visit **icnmd.org** to download the Digital Program or download the official ICNMD Mobile App.

Program at a Glance

Friday 8 July, 2022

The official time zone for ICNMD 2022 is in UTC +2:00.

Legend

- SS: Scientific Session
- TC: Teaching Course
- WS: Workshop
- HO: Hands On Course
- OS: Overarching Session
- RS: Regional Session
- PS: Poster Session - Oral Presentations

DAY 4

Industry Supported Symposia
Meeting Studio 211+212 / Silver Hall
07:00 - 08:00

PL03
Gold Hall 08:30 - 10:30

AM Break Exhibit Hall (Level -2) 10:30 - 11:15

SS13 Gold Hall 11:15 - 12:45	**SS16** Meeting Studio 211+212 11:15 - 12:45	**WS09** Meeting Studio 214+216 11:15 - 12:45	**SS14** Copper Hall 11:15 - 12:45	**SS15** Silver Hall 11:15 - 12:45
Lunch Break & ePoster Session Exhibit Hall (Level -2) 12:45 - 14:15	**Industry Supported Symposia** Meeting Sudio 211+212 / Copper Hall / Silver Hall 13:00 - 14:00			
SS18 Gold Hall 14:15 - 15:45	**OS06** Meeting Studio 211+212 14:15 - 15:45	**WS10** Meeting Studio 214+216 14:15 - 15:45	**SS19** Copper Hall 14:15 - 15:45	**SS17** Silver Hall 14:15 - 15:45

PM Break Exhibit Hall (Level -2) 15:45 - 16:15

RS - WFN JOINT SESSION Gold Hall 16:15 - 17:45	**PS08** Meeting Studio 211+212 16:15 - 17:45	**PS09** Meeting Studio 214+216 16:15 - 17:45	**PS06** Copper Hall 16:15 - 17:45	**PS07** Silver Hall 16:15 - 17:45

Networking Dinner
Royal Museums of Fine Arts
20:00 - 23:00

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Enter "ICNMD" for Event Code

Program at a Glance

Saturday 9 July, 2022

The official time zone for ICNMD 2022 is in UTC +2:00.

Legend

- SS: Scientific Session
- TC: Teaching Course
- WS: Workshop
- HO: Hands On Course
- OS: Overarching Session
- RS: Regional Session
- PS: Poster Session - Oral Presentations

DAY 5

PL04
Gold Hall 08:30 - 10:30

AM Break Level 0 Foyer 10:30 - 11:15

SS22 Gold Hall 11:15 - 12:45	**SS20** Meeting Studio 211+212 11:15 - 12:45	**SS21** Meeting Studio 214+216 11:15 - 12:45	**OS08** Copper Hall 11:15 - 12:45	**OS09** Silver Hall 11:15 - 12:45

Lunch Break & ePoster Session
Level 0 Foyer
12:45 - 14:15

OS07 Gold Hall 14:15 - 15:45	**OS10** Meeting Studio 211+212 14:15 - 15:45	**OS11** Meeting Studio 214+216 14:15 - 15:45	**WS11** Copper Hall 14:15 - 15:45	**WS12** Silver Hall 14:15 - 15:45

PM Break Level 0 Foyer 15:45 - 16:15

Closing Ceremony
Gold Hall 16:15 - 17:45

For more detailed Program Information, please visit **icnmd.org** to download the Digital Program or download the official ICNMD Mobile App.

Plenary Speakers

Amelia Evoli

Associate Professor of Neurology
Università Cattolica

Amelia Evoli is Associate Professor of Neurology at the Neuroscience Department of the Catholic University in Rome and leads the Myasthenia Gravis Unit at the University Hospital Policlinico A. Gemelli in Rome, Italy.
Her clinical and research interests are mainly focused on Neuro-immunology:

- Myasthenia gravis: Pathogenesis (genetic susceptibility, immune-pathology), markers of disease activity, clinical studies
- Other disorders of neuromuscular transmission: Lambert-Eaton myasthenic syndrome, neuromyotonia, congenital myasthenic syndromes
- Paraneoplastic neurological diseases and autoimmune disorders of central nervous system.

She is actively involved in clinical and research studies, participates in guidelines for the management of these disorders and in the standardization of clinical and diagnostic assessments.

She is a current member of the Italian Society of Neurology and the Italian Myology Association. She has authored 191 peer-reviewed articles and nine book chapters.

Bart Jacobs

Professor in Neurology and Immunology
Erasmus MC

Bart Jacobs is a professor in Neurology and Immunology at the Erasmus MC, Rotterdam, The Netherlands. His main interest in research are epidemiology, pathogenesis, diagnoses and treatment of inflammatory neuropathies, in particular the Guillan-Barré syndrome and CIDP. He is the initiator and chair of the International GBS Outcome Study (IGOS).

Plenary Speakers

Matthew Kiernan

Co-Director Brain and Mind Centre
University Of Sydney

Professor Matthew Kiernan is the Bushell Chair of Neurology at the University of Sydney and Co-Director of the Brain and Mind Centre. He is Chair of the World Federation of Neurology ALS/MND Specialty Group and established the Pan-Asian Consortium for Treatment and Research in ALS (PACTALS). He is President of the Brain Foundation and Editor Emeritus of the Journal of Neurology, Neurosurgery & Psychiatry.

Volker Straub

Director, John Walton Muscular Dystrophy Research Centre

Professor of Neuromuscular Genetics
Harold Macmillan Professor of Medicine, Director, John Walton Muscular Dystrophy Research Centre, Co-director, MRC Centre for Neuromuscular Diseases Deputy Dean, Institute of Translational and Clinical Research, Newcastle University, Newcastle upon Tyne, UK

Volker was trained as a pediatric neurologist at the Universities in Germany. He wrote his PhD thesis on Duchenne muscular dystrophy (DMD) and worked as a postdoctoral research fellow at the University of Iowa, USA.

Volker has a long-standing interest in the pathogenesis of genetic muscle diseases. His current research involves animal models, the application of magnetic resonance imaging, next generation sequencing and other – omics technologies for the characterization of primary neuromuscular disorders.

One of Volker's main interests in muscle diseases is around translational research. He was the co-founder of the EU FP6 funded network of excellence for genetic neuromuscular diseases, TREAT-NMD (www.treat-nmd.eu). He is the CI/ PI for a number of natural history and interventional trials in DMD, LGMD, Pompe disease, spinal muscular atrophy and other NMDs. He is currently the president of the World Muscle Society and an author on >400 peer-reviewed publications.

Social Events

Opening Ceremony

Date: Tuesday 5 July, 2022
Time: 18:00 – 19:00
Location: Gold Hall
Access: Open to all Registered Delegates

The Opening Ceremony will set the tone for the Congress with official Welcome from the ICNMD Chair and entertainment from Jan Cayers the artistic director of Le Concert Olympique. Following the Opening Ceremony, guests are invited to the Welcome Reception to have the opportunity to mix and mingle with colleagues and friends while enjoying a drink and small snacks.

Welcome Reception

Date: Tuesday 5 July, 2022
Time: 19:00 – 22:00
Location: Level 0 Foyers
Access: Open to all Registered Delegates
– Additional tickets can be purchased at EUR 75.00 each.

Join your fellow peers on the first evening for a drink to kick off the 17th ICNMD!

Networking Dinner

Date: Friday 8 July, 2022
Time: 19:00 -23:00
Location: Royal Museums of Fine Arts (Rue de la Régence 3, Brussles)
Access: Ticket required - EUR 125.00 each

For the final evening, join other delegates at the Royal Museums of Fine Arts and celebrate yet another fantastic and engaging ICNMD Congress. The networking event begins at 7:00 pm which includes (1) a visit to a part of the museum (2) reception (3) dinner (at 08:00) and (4) post-dinner "lounge bar".

Closing Ceremony

Date: Saturday 9 July, 2022
Time: 16:15 – 17:45
Location: Gold Hall
Access: Open to all Registered Delegates

The Closing Ceremony will celebrate the success of the Congress and mark the inauguration of the incoming ICNMD President. The next host city will be announced and launch their official invitation to the 18th ICNMD Congress in 2024.

Teaching & Hands-On Courses

***Pre-registration required**

8:00 - 12:00

HO01
Meeting Studio 202

Neuro-Imaging in Neuromuscular Diseases

Chair: Stephan Goedee

- Introduction of Neuromuscular Imaging
  Stefan Meng
- Neuromuscular Imaging in Myopathies
  Juerd Wijntjes
- Neuromuscular Imaging in Neuropathies
  Stephan Goedee
- Imaging of the Respiratory Muscles in Neuromuscular Diseases
  Jonne Doorduin

This hands-on session will teach delegates the basics of NM imaging with ultrasound and MRI including relevant practice examples of the techniques dedicated for myopathies and inflammatory and hereditary neuropathies. A topic will be related with imaging trial biomarkers for neuropathies. A topic about usefulness and new developments of imaging for anatomy and physiology of the respiratory muscles will also be developed.

TC01
Meeting Studio 201 AB

Update on Inflammatory Myopathies

Chair: Werner Stenzel

- New Classification of IIM
  Werner Stenzel
- Myopathology of IIM
  Ichizo Nishino
- Old and New Antibodies in IIM
  Lisa Christopher
- Treatment Options for IIM
  Olivier Benveniste

This workshop addresses in depth the novel aspects in idiopathic inflammatory myopathies that have emerged over the past years. Those include the way in which IIMs can be classified and the expanding spectrum of IIMs beyond PM, DM and IBM. This classification is based on morphological and immune aspects, which will be presented in detail. The IIMs have seen a spectrum of autoantibodies linked to certain disease entities and their association as well as pathophysiological background is discussed. Finally, the etiopathogenic underpinnings have led to development of novel treatment concepts that may be explored in the future.

TC02
Meeting Studio 204

Diagnosis and Treatment of GBS, CIDP and Autoimmune Nodopathies

Chair: Peter Van den Bergh

- 2022 EAN/PNS GBS Guideline
  Rob Hadden
- 2021 EAN/PNS CIDP Guideline
  Peter Van den Bergh
- Autoimmune Nodopathies
  Luis Querol
- Biomarkers and Outcome Measures in Immune-mediated Neuropathies
  Jeffrey Allen

The new EAN/PNS GBS and CIDP guidelines constructed by an international task force of disease experts according to modern GRADE methodology will be presented. The GBS guideline is the first ever and the CIDP guideline is a long awaited revised version of the 2010 guideline. The state-of-the-art

management recommendations for GBS and CIDP will be discussed. The autoimmune nodopathies, a novel group of neuropathies resembling CIDP but distinct from it, are caused by nodal/paranodal antibodies mainly of the IgG4 subclass. The special features associated with these will be discussed as will be the treatment recommendations. Biomarkers helpful for the diagnosis and follow-up of these autoimmune neuropathies are being developed. Together with an increasing number of validated outcome measures, they are helpful instruments to monitor the disease course and treatment response and to reshape treatment.

TC03

Meeting Studio 206

Towards Targeted Treatments in Myasthenia Gravis: Update on Pathogenetic Mechanism and New Drugs

Chair: Pushpa Narayanaswami

- Towards Targeted Therapies in Myasthenia Gravis- Pathogenic Mechanisms Translated to Treatments
  Pushpa Narayanaswami
- Complement Inhibitor Therapy for Myasthenia Gravis
  Srikanth Muppidi
- The role of FcRn Antagonists in MG Treatment
  Nils-Erik Gilhus
- Peripheral Nervous System Complications of Immune Checkpoint inhibitors
  Jan De Bleecker

There have been several exciting advances in the treatment of autoimmune myasthenia Gravis (MG) over the last few years. Treatments targeted to specific pathophysiological underpinnings of MG are being rapidly developed and approved for use. The aim of this course is to review these treatments. First, advances in the pathophysiological basis of MG will be discussed in depth, with emphasis on how the understanding of pathophysiology influences the development of targeted therapies. Two major therapeutic targets, complement inhibition and neonatal Fc receptor blockade will then be discussed with results of completed studies of these agents. Finally, Immune Checkpoint Inhibitors (ICIs) are used widely in oncology and MG or MG/myositis overlap is an important complication of these treatments. The last talk will address immune checkpoints, the mechanism of action ICIs and the neuromuscular complications of ICI therapy with emphasis on the neuromuscular complications.

TC04

Meeting Studio 211+212

Clinical Manifestations of Myopathies – Less Common Presentations

Chair: Zohar Argov

- Dropped Head Syndrome
  Antonio Toscano
- Asymptomatic Very High Creatine Kinase
  Benedikt Schoser
- Drug Induced Weakness
  Zohar Argov
- What Do We Need to Know When Our NMD Patients Get Pregnant?
  Sabine Rudnik Schoeneborn

Although weakness is the main feature of NMD presentation, there are presentation which are more rare but need to be recognized as they carry different differential diagnoses and some specific management modes. This course targets

Teaching & Hands-On Courses

***Pre-registration required**

the young as well as the more experienced NMD expert. In each of the lectures the speakers will present the unique clinical features and the dilemmas involved with these situations. It is part 2 of a more basic TC about clinical manifestations.

13:00 - 17:00

HO02
Meeting Studio 202

Morphological Aspects in Neuromuscular Diseases

Chair: Monika Hofer

- Muscle and Nerve Biopsy Pathology. Basic Histology and Morphology
  Monika Hofer
- What's in a Nerve? Neuropathology Analysis of Frozen Tissue and a New Treatable Neuropathy
  Alan Pestronk
- Myopathology in Congenital Myopathies
  Teresinha Evangelista
- Myopathology in Vacuolar and Protein Aggregate Myopathies
  Montse Olivé

Many new neuromuscular diseases and genes have been identified over the past few years and current knowledge about up-to-date structural and ultrastructural are presented are presented following a basic introduction to morphology. Key areas are selected for in-depth presentation with a focus on the clinical phenotype, the genotype and the corresponding morphological abnormalities.

TC05
Meeting Studio 201 AB

Amyotrophic Lateral Sclerosis - Recent Advance, Pathogenesis, Diagnosis and Treatment

Chair: Vivian Drory

- What Can we Learn From Recent Advances in ALS Genetics?
  Philip Van Damme
- Biomarkers - Friend or Foe for ALS Drug Development?
  Albert Ludolph
- New and Experimental Treatments for ALS – from One Drug for All to Personalized Medicine
  Angela Genge
- PLS and ALS – Part of a Continuum or Two Different Diseases?
  Vivian Drory

The last decade has seen tremendous advances in our understanding of ALS mechanisms and diagnostics, as well as first seeds of pathogenesis-led treatments. This course targets the young as well as the more experienced NMD clinicians and health professionals who are less familiar with the last developments in the area of ALS and seek to update their knowledge and understanding of the disease toward personalized patient management.

TC06
Meeting Studio 204

Diagnosis of Inherited Neuropathies

Chair: Mary Reilly

- Clinical Approach to Diagnosis of CMT
  Davide Pareyson
- Genetic Diagnosis of Inherited Neuropathies
  Mary Reilly

Teaching & Hands-On Courses

***Pre-registration required**

- Diagnosis of TTR Amyloid Polyneuropathy for a Curable Disease
- David Adams

- Approach to Complex Neuropathies
- Alexander Rossor

This teaching course aims to cover in depth the diagnosis of inherited neuropathies including CMT, TTR amyloidosis and the more complex forms which overlap with HSP and ataxia. The aim is attendees would get an state of the art up to date diagnostic guidelines.

TC07

Meeting Studio 206

Congenital Myasthenic Syndromes

Chair: Duygu Selcen

- Morphological Aspects and Introduction
- Duygu Selcen

- Clinical aspects of Congenital Myasthenic Syndromes in Adulthood
- Bruno Eymard

- Genetic Aspects of Congenital Myasthenic Syndrome
- Hanns Lochmuller

- Treatment Aspects
- Ulrike Schara-Schmidt

CMS are a field that is important on all the above-mentioned levels and bears many new and relevant aspects both on the basic research side as well as on the clinical side.

TC08

Meeting Studio 211+212

Clinical Manifestations of Myopathies – Other than Limb Weakness

Chair: Marianne de Visser

- Cognitive Impairment
- Jens Reimann

- Swallowing Difficulty in Myopathies
- Marianne de Visser

- Skin and Connective Tissue Abnormalities
- Carsten G. Bönnemann

- Heart Involvement
- Wahbi Karim

Pattern recognition plays an important role in the diagnostic process of myopathies. Most myopathies manifest primarily with proximal and/or distal muscle weakness. However, also other features may be presenting symptoms and sometimes hamper a timely diagnosis, as may be the case if a patient with inclusion body myositis presents with swallowing difficulty and no signs of limb weakness. Syncope's may herald myotonic dystrophy type 2 and should not be missed since they can cause sudden death. Skin abnormalities are well-known in dermatomyositis, but there are other myopathies in which the skin is also involved. And cognitive impairment is a frequent symptom in various myopathies and has considerable impact on daily activities and quality of life. This teaching course addresses these manifestations in a systematic way in order to reinforce the skills and competencies to establish an accurate and timely diagnosis and to take appropriate measures for management.

Tuesday 5 July, 2022

18:00 - 19:00

Gold Hall

Opening Ceremony

19:00 - 22:00

Level 0 Foyer

Welcome Reception

Scientific Program

Wednesday 6 July, 2022

08:30 - 10:30

PL01
Gold Hall

Trial Readiness and Advances in Therapy in Hereditary and Inflammatory Myopathies
Chair: Volker Straub

- An Update on the Limb Girdle Muscular Dystrophies
  Volker Straub
- Advances in Congenital Myopathies
  Heinz Jungbluth
- Clinical Trial Readiness in Inflammatory Myopathies
  Rohit Aggarwal

The diseases which will be addressed in this symposium are rare diseases in which diagnostics and pathomechanistic insight have made considerable progress. However, a curative therapy is currently lacking for the hereditary disorders and often insufficient for the group of idiopathic inflammatory myopathies (IIM). In hereditary myopathies management usually consists of timely recognition and symptomatic treatment of complications, aiming to improve life expectancy and quality of life. In IIM treatment leads to complete remission in a proportion of the patients and is often associated with severe side-effects.

After the preclinical phase of a drug developing programme has been successfully completed, there are numerous prerequisites which might be labelled as trial readiness, including deep knowledge of the natural history of the disease, clinically relevant outcome measures, and biomarkers to monitor the drug response.

This symposium will address trial readiness and promising developments in some limb girdle muscular dystrophies, congenital myopathies and inflammatory myopathies.

10:30 - 11:15

Exhibit Hall (Grand Hall 2)
AM Break

11:15 - 12:45

SS01
Meeting Studio 211+212

Muscle Channelopathies: From Bench to Bedside
Chair: Bertrand Fontaine

- Personalized Therapy in Skeletal Muscle Channelopathies
  Jean-François Desaphy
- Pathophysiology of Periodic Paralysis
  Stephen Cannon
- Treatments of Channelpathies
  Bertrand Fontaine

Recently, the understanding of muscle channelopathies has led to better treatment and management of patients. The aim of the scientific section is to present comprehensive and recent advances form basic science to patient treatment.

SS02
Gold Hall

Spotlight on FSHD: the Road to Treatments
Chair: Giorgio Tasca

- Genetics, Epigenetics and Downstream Consequences
  Peter Zammit
- Muscle MRI,Echo or EIM: Which is Best for FSHD?
  Giorgio Tasca
- Trials, Trial Readiness and the Requirements
  Nicol Voermans

This most frequent muscular dystrophy is best characterized by its enormous variable severity and rate of progression and by consequence its variable phenotype which in its early manifestation is often difficult to diagnose. Its genetic regulation is still not fully understood; the various pathways implicated as a consequence of DUX4 expression are the focus of many researchers. At the same time therapeutic windows are being explored and have revealed a trove of new information on the disease and raised many important questions for future explorations. All these aspects will be discussed for the neuromuscular community that seeks up to date information on where we stand in this rapidly evolving field of FSHD.

SS03
Copper Hall

Actininopathies – A New Category of Genetic Muscle Disease

Chair: Bjarne Udd

- Adult Dominant Distal Actininopathy
  Bjarne Udd
- Congenital Core Myopathy
  Jocelyn Laporte
- FSHD-like Dominant Cctininopathy
  Marco Savarese

Presentation of a new group of genetic muscle diseases by defect Z-disc protein alpha-actinin2 with variable phenotypes.

WS01
Meeting Studio 214+216

Muscle, Nerve and Brain in LAMA2 Disease: Pathogenesis, Natural History, and Potential Therapies

Chair: Stefano C. Previtali

- Peripheral Neuropathy and Brain Abnormalities in the Pathogenesis of LAMA2 Disease
  Stefano C. Previtali
- Muscular Dystrophy and Clinical Trial Readiness in LAMA2 Disease
  Anna Sarkozy
- Towards a New Potential Therapy of LAMA2-Related Muscular Dystrophy
  Markus A. Ruegg

LAMA2 disease (or Congenital muscular dystrophy with deficiency of merosin) is an autosomal recessive disorder due to mutations in the LAMA2 gene, encoding for the alpha2 chain of laminin-211. As a consequence, laminin heterotrimers containing the α2 chain are not assembled or are expressed at very low levels, causing progressive degeneration of skeletal muscles, nerves, and the brain. Thus, this defect results in a severe multi-organ disorder characterized by the progressive wasting of skeletal muscle, demyelinating peripheral neuropathy, and brain abnormalities causing death in the first decade or serious disability.

A number of mouse models reproducing clinical and pathological features of human LAMA2-CMD are available and recent advances in molecular genetics identified some of the molecular mechanisms that are deregulated downstream of the primary defect. The scientific community has been using these findings to better characterize the pathogenesis, to identify useful biomarkers, and to generate therapeutic strategies to ameliorate the disease.

The aim of this Workshop is to cover different aspects of the LAMA2-CMD disease, including molecular pathomechanisms, main clinical findings, lessons from animal models, development of potential treatments on the basis of mechanistic understanding, and the identification of potential biomarkers of the disease.

Scientific Program

WS02
Silver Hall

Metabolic Myopathies in Adults: Diagnosis and Treatments

Chair: Pascal Laforêt

- Overview of Metabolic Myopathies
  John Vissing
- Diagnostic Tools and Strategy for Diagnosis of Metabolic Myopathies
  Pascal Laforêt
- Current Treatments of Metabolic Myopathies
  Ans van der Ploeg

Metabolic myopathies (MM) are an important group of potentially treatable inherited muscle disorders affecting children and adults.

Major advances have been achieved in the diagnosis of these disorders over the past years thanks to improvements in biochemical and molecular techniques.

Better understanding of the clinical course of various metabolic myopathies has led to improved approach to care and follow-up and better outcomes. Disease modifying enzyme replacement therapy is available for one of the main muscle glycogenoses, Pompe disease, and novel drugs are in the pipeline for other conditions.

The aims of this teaching course are:

- To describe the pathophysiology and clinical features of main metabolic myopathies (glycogenoses, fatty acid oxidation disorders, and mitochondrial myopathies)
- To familiarize participants with the main diagnostic tools: exercise testing, muscle biopsy, biochemical and molecular genetic analysis
- To provide guidance for diagnosis and management of rhabdomyolysis
- To describe current treatments and follow-up of metabolic myopathies

Wednesday 6 July, 2022

12:45 - 14:15

Exhibit Hall (Grand Hall 2)

Lunch Break & ePoster Session

13:00 - 14:00

Meeting Sudio 211+212 / Copper Hall / Silver Hall

Industry Supported Symposia

14:15 - 15:45

SS04
Meeting Studio 211+212

Recent Developments in Therapies for Centronuclear Myopathies

Chair: Marc Bitoun

- Pathophysiological Mechanisms in the Dominant Centronuclear Myopathy Due to Dynamin 2 Mutations
  Marc Bitoun
- Allele specific Therapy for the Dynamin 2-linked Dominant Centronuclear Myopathy
  Delphine Trochet
- Dynamin 2 Therapy for Different Forms of Centronuclear Myopathies
  Jocelyn Laporte

The goal is to report advances in the field of the centronuclear myopathies with a particular focus on the Dynamin 2-linked form of the disease and the use of Dynamin 2 as therapeutic target.

SS05
Copper Hall

Treatable Limb Girdle Muscular Dystrophy-like Diseases

Chair: Antonio Toscano

- Anti-HMGCR Necrotizing Myopathy
  Carsten G. Bönnemann
- Congenital Myasthenic Syndrome
  Duygu Selcen
- Metabolic Myopathies
  Antonio Toscano

SS06
Gold Hall

Drug Repurposing to Accelerate Therapeutic Development in Neuromuscular Disorders

Chair: Peter Bram 't Hoen

- Drug Repurposing and Biomarker Candidates Discovered Through Molecular Profiling of Myotonic Dystrophy Type 1 Patients on Cognitive Behavioural Therapy
  Peter-Bram 't Hoen
- Metformin: a Repurposed Drug for Myotonic Dystrophy
  Guillaume Bassez
- Drug Repurposing Strategies for Congenital Myasthenic Syndromes
  Sally Spendiff

Most neuromuscular diseases can not be cured.

The drug development process is costly and lengthy.

We here discuss the drug repurposing strategy based on the reverse engineering of a heterogenous response to an intervention in myotonic dystrophy type 1, which may set the scene for future drug development trajectories for rare diseases. The drug repurposing strategy would considerably shorten the clinical development trajectory and costs associated with bringing the drug to the clinics.

In addition we show the value of drug repurposing by two recent examples: metformin for myotonic dystrophy type 1, and mexiletine for non dystrophic myotonia.

WS03
Meeting Studio 214+216

CNS Involvement in Myopathies: Stroke-like Episodes, Autism, and Behavioral Changes. How to Treat?

Chair: Corrado Angelini

- CNS Dysfunction in DMD and Muscle Glycosylation/Collagen Disorders
  Luca Bello
- Brain in DM1 Subtypes and DM2 has Differential Involvement
  Corrado Angelini
- CNS Involvement in Mitochondrial Disorders
  Thomas Klopstock

Dystrophinopathies and glyycosyilation disorders giving rise to Congenital muscle dystrophy present a spectrum of disorders ranging from autism to mental retardation and might present sometime epilepsy,and autism due to brain alteration.

DM1 is a myulti-systemic disorder characterized by brain and heart involvement, the changes found in brain MRI can be due to developmental delay or abnomal gyrification or, a progressive connectivity dysfunction, where CBT treatment seems useful. DM2 brain changes are less prominent. Mitochondrial disorders are characterized by stroke-like episodes.,epilepsy or ocular myopathy,

their clinical treatment is difficult.Brain dysfunction is progressive both in MELAS and MERRF syndromes, adPEO has to differentiated from OPMD, also with mental retardation, sometime.

This workshop will include advanced clinical, diagnostic and therapeutic discoveries of the very last few years in highly specific neuromuscular disorders. The topics are very attractive and up-to-date, and may offer a wide panorama about very specific neuromuscular conditions.

This workshop implies a higher level of clinical awareness of attendees and will supply new clinical relevance of molecular diagnostic, especially for the specific treatment of CNS cognitive problems in neuromuscular diseases.

OS01
Silver Hall

Newly Evolving Phenotypes in Neuromuscular Diseases Due to Novel Treatments

Chair: Liesbeth De Waele

- The Emerging Phenotype in Classic-infantile Pompe Disease: Challenges for the Future
  Nadine van der Beek
- The Changing Phenotype of DMD Patients
  Liesbeth De Waele
- Spinal Muscular Atrophy : The Paradigm of a Disease with Changing Phenotypes
  Laurent Servais

More and more new disease-modifying treatments for neuromuscular diseases are becoming available. Due to these treatments the phenotypic borders of diseases have changed and new important challenges are emerging. In these lectures we will tackle these very relevant questions and provide practice-based experiences.

15:45 - 16:15

Exhibit Hall (Grand Hall 2)

PM Break

16:15 - 17:45

WS04
Gold Hall

Treatable Myopathies

Chair: Jan De Bleeker

- Polymyositis, Dermatomyositis and Immune Necrotising Myopathy
  Jan De Bleeker
- Myasthenic Syndromes
  Sabrina Sacconi
- Intensive Care Unit Weakness
  Max Damian

Polymyositis ,Dermatomyosiitis and immune mediated myopathies affect people of any age and sex, but are more common in women than men,polymyositis is found more frequently in adults than children, and immune mediated necrotizing myopathy is a relatively steroid responsive disease.In the field of inflammatory myopathies there are several new treat ments.

Myasthenic syndrome can be treated with various drugs and IVIg,might be paraneoplastic syndrome prevention is important.

Intensive Care Unit Weakness is a major problem for neurology residents and intensive care personnel.

The electrodiagnostic and rehablitative features are both critical issues.

WS05
Copper Hall

Treatment of Late Onset Pompe Disease in 2022: Past, Present and Future

Chair: Pascal Laforêt

- Benefits and Limits of ERT in LOPD
  Gauthier Remiche
- Next-generation Clinical Outcomes and Biomarkers in LOPD
  Pascal Laforêt
- The Future of Treatments for Pompe Disease
  Nadine van der Beek

Enzyme replacement therapy (ERT) with recombinant enzyme produced on hamster ovarian cells (alglucosidase alfa, Myozyme) has been registered for the treatment of all forms of Pompe disease since 2006. The prognosis of classical infantile Pompe disease has been considerably modified by ERT, and recently published long-term analysis of Myozyme treatement in adults mainly show an improvement of motor and respiratory assessments in the 3 to 5 years following treatment initiation, with an important interindividual variability of response. Two next-generation ERTs have been recently compared to Myozyme in international multicentric randomized clinical trials, and phase 1 gene therapy trials are ongoing.

The goals of this symposium are: 1) to present a state of the art of the benefit and limits of alglucosidase alfa in Late onset Pompe disease, 2) to discuss the limits of current outcome measures and lack of biomarkers, and suggest new avenues to monitor clinical symptoms, and 3) to present the alternative therapeutic strategies and future treatments.

SS07
Silver Hall

Is There Still a Role for Biopsies in the Era of Next-Generation Sequencing?

Chair: Kristl Claeys

- Muscle Biopsies in the Era of NGS
  Kristl Claeys
- Nerve Biopsies in the Era of NGS
  Joachim Weis
- Skin Biopsies in the Era of NGS
  TBC

The diagnostic procedure in neuromuscular disorders has changed since the introduction of the novel genetic technologies, so-called Next-Generation Sequencing (NGS). In this Scientific Session we want to highlight the still important roles of muscle, nerve and skin biopsies in this newly evolving field.

Scientific Program

Wednesday 6 July, 2022

WS06
Meeting Studio 211+212

New Techniques in Myology. What Can One Expect from Using 'Modern' / 'Cutting Edge' Techniques in Routine and Research?

Chair: Andreas Roos

- Muscle Proteomics - New Avenues in Diagnostics and Research of Neuromuscular Diseases
  Andreas Roos
- Utilising Novel Technologies to Improve the Diagnostic Yield of Whole Exome Sequencing in Patients with Neurogenetic Disease
  Rita Horvath
- Large-scale Electron Microscopy; Perspectives for Imaging Neuromuscular Diseases
  Carsten Dittmayer

This Workshop should give guidance and information on how to use cutting edge techniques in mycological research and what to expect from.

WS07
Meeting Studio 214+216

Pattern Recognition Approach to Patients with a Suspected Myopathy *

Chair: Benedikt Schoser

- The Gestalt Approach to Neuromuscular Disorders of Adulthood - a General Introduction
  Benedikt Schoser
- Scapular Winging
  Antonio Toscano
- Gait Patterns in Neuromuscular Diseases
  John Vissing

Workshop encompassing a basic approach to neuromuscular diseases. Key features of neuromuscular diseases will be presented with movies and picture. Common presentations like scapula alta, and neuromuscular gait pattern will be presented by state-of the-art lectures.

18:00 - 19:00

Copper Hall

Industry Supported Symposium

Scientific Program

Thursday 7 July, 2022

07:00 - 08:00

Meeting Studio 214+216 / Copper Hall

Industry Supported Symposia

08:30 - 10:30

PL02

Gold Hall

The ALS-FTD Overlap Syndrome

Chair: Matthew Kiernan

- Amyotrophic Lateral Sclerosis and Frontotemporal Dementia: Overlap Syndromes
- Matthew Kiernan

- Cognitive Features in ALS
- Patricia Lillo

- ALS/FTD Genetic Landscape and Therapies
- Eva Feldman

The ALS-FTD overlap is a very hot topic clinically and scientifically.

A spectrum of non-motor manifestations in amyotrophic lateral sclerosis (ALS) patients has been increasingly recognized, with cognitive and behavioral impairments the most prominent. Evidence suggests that ALS overlaps on a pathological, genetic, and clinical level with frontotemporal dementia (FTD), thereby suggesting a frontotemporal spectrum disorder (ALS-FTSD). Cognitive impairment has been reported in up to 75% of ALS patients, whilst the rate of behavioral dysfunction ranges up to 50%. Similarly, there is a growing literature about motor manifestations in FTD.

The genetics underlying the clinical spectrum are now well understood and the advent of genetic therapy is a timely development that underpins this symposium – the launch of antisense oligonucleotide therapies.

10:30 - 11:15

Exhibit Hall (Level -2)

AM Break

11:15 - 12:45

SS08

Gold Hall

Non 5q SMA: A Growing Spectrum of Disorders

Chair: Francesco Muntoni

- Clinical and Genetic Diversity of SMN1-negative Proximal Spinal Muscular Atrophies
- Francesco Muntoni

- The Distal Hereditary Motor Neuropathies.
- Alexander Rossor

- The Role of Sphingolipid Synthesis Regulation in Human Motorneuron Diseases
- Payam Mohassel

Common objective shared by both lectures:
How clinical features of the different subtypes of non 5qSMA help focus genetic testing for the practicing clinician?
Review the neuroscience that underpins our current understanding of how these mutations lead to a motor neuron disease (and eventually highlight potential therapeutic strategies).
Discuss the presenting features of conditions with neurogenic arthrogryposis and conditions related to developmental defects of axonal path finding (for example ECEL1, COL25A1, ZC4H2).

SS09
Copper Hall

New Horizons in ALS in 2022

Chair: Eva L. Feldman

- The Immune System: Friend or Foe in ALS
  Eva Feldman
- The Promise of Biomarkers in ALS becomes a Reality
  Philip Van Damme
- Presymptomatic ALS: an Opportunity for Disease Intervention
  Joke de Vecht

OS02
Meeting Studio 211+212

Digital Outcome Measures in Neuromuscular Disorders

Chair: Gabriele Siciliano

- Facial and Vocal Recognition as a Decision Support Tool for Neuromuscular Diseases: The FACE-NMD Project
  Sabrina Sacconi
- Why and How to Digitalize Bulbar Motor Dysfunction Patterns in NMD
  Gabriele Siciliano
- Body Posture Recognition in NMDs
  Tiziana Enrica Mongini
- The Role of Digital PROMs in the Data Collection for Real World Evidence Evaluation
  Peter Balicza

The development of a roadmap for the implementation of outcome measures is essential to optimize diagnostic and follow-up strategies. Obtaining reliable information about everyday functioning from individuals with NMD, alsoin natural environments, is critical for clinical care and research, therefore advancesin mobile health technologies which allow to mix digital outcomemeasurespatient-centered with clinical relevance will be fundamental. The aim of this HANDS On COURSE is purposed to make aware neurologists of the usefulness of assistive technology application to clinical practice and to increase skills in that for disease follow up and trial readiness in NMD.

OS03
Silver Hall

Transition of Neuromuscular Patients into Adult Care

Chair: Ros Quinlivan

- General Principles of Transition into Adult Care
  Karsten Vanden Wyngaert
- Transition to Adult Services for Young People with Neuromuscular Disease: A Neurologist's Perspective
  Ros Quinlivan
- Transition to Adult Neuromuscular Care: the Pneumologist's Perspective
  Alessandro Onofri

Transition from paediatric to adult healthcare system is an undervalued and challenging issue for children with chronic conditions such as neuromuscular disorders. An additional challenge are patients on non-invasive or invasive mechanical ventilation. In this session we discuss challenges and difficulties of transition in adult care and provide evidence-based and practice-based data for an optimal management of this patient group.

Scientific Program

Thursday 7 July, 2022

PS05
Meeting Studio 214+216

Selected Abstracts for Oral Presentation

Chair: TBC

- Rozanolixizumab in Generalized Myasthenia Gravis: Responder Analyses From the Phase 3 MycarinG Study
  Vera Brill
- Combining Clinical Trial and Real-world Data to Model the Benefit of Efgartigimod on Productivity Losses
  Francesco Saccà
- Humoral Responses Following SARS-CoV-2 Vaccination in Patients with Commonly Used Immunosuppressants in Neuromuscular Disorders
  Pieter Jacob van Dam
- Disease Activity after SARS-CoV-2 Vaccination and Infection in Patients with Immune-Mediated Neuromuscular Diseases
  Eileen Stalman
- Immunosuppressive Therapy as Risk Factor for Severe SARS-CoV-2 Infection in Myasthenia Gravis
  Frauke Stascheit
- Pathogenic Effects of IgG1-MuSK Antibodies on the Agrin-induced AChR Clustering Pathway in C2C12 Myotubes
  Angela Vincent

12:45 - 14:15

Exhibit Hall (Grand Hall 2)

Lunch Break & ePoster Session

13:00 - 14:00

Gold Hall / Copper Hall / Silver Hall

Industry Supported Symposia

14:15 - 15:45

SS10
Gold Hall

Spinal Muscular Atrophy: The Buzz of New Treatment but still Many Open Questions

Chair: Nicolas Deconinck

- What do we Finally Know About SMN Biology?
  Charlotte Sumner
- Challenges in Optimizing SMN Restoration in SMA
  Richard Finkel
- SMA and New Treatments: Making Sure We Capture All Disease Aspects
  Nicolas Deconinck

Multiple and complex roles of SMN protein during development and postnatally, in motor neurons but also in other cell types, implication for timing of SMN replacement, and differential CNS/ peripheral rescue of different therapies.

Clinical trials are providing data on window of optimal response and implication of duration of disease before treatment, with important learning for clinical practice.

IN only a few years, the cohort of treated SMA1 patients who would have otherwise succumbed to the disease is expanding very rapidly, providing also insight on which aspects of the condition respond better or less well to therapeutic intervention, and consideration for novel emerging phenotypes.

SS11
Copper Hall

Biomarkers in Spinal Muscular Atrophy and other Neurodegenerative Diseases

Chair: Piera Smeriglio

- Imaging Biomarkers in SMA and ALS
  Giorgia Querin
- NatHis for Identification of Biomarkers in Spinal Muscular Atrophy
  Shahram Attarian
- Neurofilaments and Other Molecular Biomarkers in Treated SMA Patients
  Piera Smeriglio

The goal of this session is to report on the latest advancements on the identification of biomarkers for patient classification and treatment follow-up. A particular focus will be given to spinal muscular atrophy and the quest for biomarkers to help for a better stratification of the patients and to predict their response to available treatments, notably nusinersen – the most widely administered therapy for SMA disease.

OS04
Silver Hall

Palliative Care in Neuromuscular Diseases - A Neglecte Area

Chair: David Oliver

- Overview on Palliative Care in Neuromuscular Disorders
  Marianne de Visser
- The Role of the Neurologist and Palliative Care Specialist in ALS
  David Oliver
- Approach to Palliative Care in Pediatric NMD'
  Maja Von der Hagen

Palliative care aims to improve the quality of life of patients affected by life-threatening illness and their families - with palliative care provided from neurology teams, collaborating withs specialist palliative care teams. Most neuromuscular disorders are chronic and progressive and associated with a shortened life expectancy. However, palliative care is underutilized in most neuromuscular disorders despite the positive experiences in amyotrophic lateral sclerosis. Unawareness of the benefits of palliative care and perceived barriers are the main reasons for the limited use of palliative care.

Challenges in the palliative care of patients with chronic progressive life-limiting neuromuscular diseases include the broad spectrum of the rate of symptom progression, a lack of reliable and valid condition-specific outcome measures of quality of life, the existential distress that may be seen for patients, families and professionals, and a scarcity of evidence for efficacy of symptomatic treatments.

Optimal palliation requires various skills, provided by a multidisciplinary team of health-care professionals. Education of these health care professionals, directed towards improving communication strategies, is crucial in this respect.

Scientific Program

Thursday 7 July, 2022

OS05
Meeting Studio 211+212

Preconception Carrier and Newborn (DMD and SMA) Screening in Neuromuscular Diseases

Chair: Nigel Laing

- The Changing Scope of Newborn Screening: Wilson and Jungner, Duchenne Muscular Dystrophy and Beyond
  Angus Clarke

- Newborn Screening of Spinal Muscular Atrophy. What Have We Learned?
  Laurent Servais

- The Role of Preconception and Early Pregnancy Carrier Screening in Neuromuscular Disorders
  Nigel Laing

Screening programs for recessive diseases are a public health measure to allow couples to have information about their carrier status for severe recessive diseases. Couple may then use that information to avoid having children affected with those diseases, if this accords with their personal values. There are now multiple commercial suppliers of carrier screening operating in many countries. At the same time, many governments are researching population-wide carrier screening.

Newborn screening for spinal muscular atrophy in particular, but also for Duchenne muscular dystrophy are being increasingly implemented or researched. These newborn screening programs for neuromuscular disorders have the twin aims of allowing couples to avoid having second affected children and to allow as early as possible intervention with available therapies. Implementation of therapies as early as possible for these diseases give the best outcomes for the affected children.

PS01
Meeting Studio 214+216

Selected Abstracts for Oral Presentation

Chair: Eric Hoffmann

- Analysis of the Longitudinal CINRG Becker Natural History Study Dataset
  Utkarsh Dang

- Results of a Double-Blind Cross-over Trial of Vamorolone in DMD: A Safer Alternative to Corticosteroids
  Eric Hoffman

- Early Effect of Steroids on Functional Outcomes in Young Boys with Duchenne Muscular Dystrophy
  Marianela Schiava

- Retrospective, Longitudinal Clinical Analysis in a Large UK Cohort of Patients with Nemaline Myopathies
  Luke Perry

- GNE Myopathy – Phenotype, Genotype Characteristics and Disease Progression in Large Cohort of Indian Patients
  Dipti Baskar

- Multimodal Assessment of Dysphagia in Patients with Inclusion Body Myositis and Oculopharyngeal Muscular Dystrophy
  Rachel Zeng

15:45 - 16:15

Exhibit Hall (Grand Hall 2)

PM Break

Scientific Program

16:15 - 17:45

SS12
Gold Hall

Newborn Screening of SMA: Aspects to Consider When Launching a New Program

Chair: Laurent Servais

- Why Newborn Screening and Early Treatment are Game Changing
  Laurent Servais
- Health Economic Consideration of Newborn Screening of SMA
  Tamara Dangouloff
- Organisational, Ethical, and Regulatory Considerations When Setting up an NBS Program
  Corinne Betts

PS02
Silver Hall

Selected Abstracts for Oral Presentation

Chair: Giorgia Querin

- The Thymus in the Pathogenesis/ Pathophysiology of Amyotrophic Lateral Sclerosis
  Julia P. Lemos
- Spinal Cord MRI for Tracking of Early Degeneration in C9orf72 Asymptomatic Carriers: A Longitudinal Study
  Giorgia Querin
- Analysis of Muscle Resonance Imaging of Cohort of Chronic Motor Neuropathy/ Neuronopathy Patients Reveals Characteristic Features
  Jordi Diaz-manera
- Deep Learning-Powered Hybrid Optoacoustic Imaging for Characterization of Pediatric Spinal Muscular Atrophy
  Ferdinand Knieling
- Description of a Spanish cohort with Cerebellar Ataxia with Neuropathy and Vestibular Areflexia Syndrome (CANVAS)
  Daniel Santirso
- Contribution of Magnetic Resonance Spectroscopy in the study of Hereditary Spastic Paraplegia.
  Andrea Martinuzzi

PS03
Meeting Studio 211+212

Selected Abstracts for Oral Presentation

Chair: Jeffrey Chamberlain

- Phase 1/2a Trial of Delandistrogene Moxeparvovec in Patients with DMD: 4-year Update
  Stefanie Mason
- IGNITE DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for DMD: 2-Year Outcomes Update
  Perry Shieh
- Minimizing Immune Responses Against Micro-Dystrophin
  Jeffrey Chamberlain
- Safety, ß-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAVrh74.MHCK7. hSGCB in LGMD2E/R4
  Andre Müller-York
- AAV Vector-mediated RNAi of Mutant LDB3 Expression as a Therapeutic Strategy for Myofibrillar Myopathy
  Pankaj Pathak

- ASPIRO Gene Replacement Therapy (Resamirigene Bilparvovec) Trial in XLMTM: Pathologic Findings in Four Deceased Participants
- Michael W. Lawlor

PS04
Meeting Studio 214+216

Selected Abstracts for Oral Presentation

Chair: Eva Feldman

- Useful and Cost-effective Workup in Chronic Polyneuropathy (the EXPRESS Study)
- Madde Wiersma

- The Prevalence of and Risk Factors for Distal Symmetric Polyneuropathy in a Low-income, U.S. Population
- Eva Feldman

- Assessment Timing and Choice of Outcome Measure in Determining Treatment Response in CIDP: Post-hoc PRISM
- Rabye Ouaja

- Peripheral Neuropathies Associated with Systemic Autoimmune Disorders: A Single-Centre Retrospective Study
- João Moura

- Autoantibody Screening in Idiopathic Small-Fiber Neuropathy
- Elba Pascual-Goñi

- Diagnostic Value of Standardized Nerve Ultrasound of the Plexus Brachialis in Chronic Inflammatory Neuropathies
- Nicolas Dubuisson

WS08
Copper Hall

Epigenetic Regulation in Neuromuscular Diseases

Chair: Piera Smeriglio

Epigenetic regulation in neuromuscular diseases (NMD). The goal of this session is to highlight the latest discoveries about the role of epigenetic regulation in the onset and progression of neuromuscular disorders. Both intra-generational and intra-tissues differences are due to the influence of epigenetic modifications which are contributing to modify the disease severity and have a key role in the onset and progression of NMD.

- Understanding Myogenic Transcription Factors Through Quantitative Biology
- TBC

- Epigenetic Changes in Spinal Muscular Atrophy
- Piera Smeriglio

- Genomic Rearrangements in FSHD
- Silvère van der Maarel

18:00 - 19:00

Copper Hall / Silver Hall

Industry Supported Symposia

Scientific Program

07:00 - 08:00

Meeting Studio 211+212 / Silver Hall

Industry Supported Symposia

08:30 - 10:30

PL03
Gold Hall

Guillain-Barré Syndrome: State of the Art and Challenges

Chair: Bart C. Jacobs

- GBS and COVID-19 Infections and Vaccinations
  Michael Lunn
- Predicting the Clinical Course of GBS
  Bart C. Jacobs
- Treatment of GBS
  Christa Walgaard

10:30 - 11:15

Exhibit Hall (Grand Hall 2)

AM Break

11:15 - 12:45

SS13
Gold Hall

Not Only CIDP: Critical Issues in Other Chronic Immune-Mediated Neuropathies

Chair: Eduardo Nobile-Orazio

- Multifocal Motor Neuropathy: Diagnosis Beyond Conduction Block and Anti-GM1 Antibodies
  Leonard H. van den Berg
- Anti-MAG Neuropathy: Diagnosis Beyond Antibodies
  Eduardo Nobile-Orazio
- CISP and CISP-plus: a CIDP Variant or Separate Diseases?
  Peter J B Dyck

There are immune neuropathies that closely resemble CIDP but appear to be separate diseases. The diagnostic criteria for these neuropathies are far from being fully established, as is the response to therapy in some of them. Specific antibodies are closely related to these neuropathies even if their specificity is quite debated. The diagnosis is often made on the presence of these antibodies but it is not so infrequent to find these antibodies in other neuropathies often leading to an inappropriate therapeutic approach.

SS14
Copper Hall

Therapeutic Targets in Diabetic Neuropathy

Chair: James W. Russell

- Genomics and DNA Methylation in Diabetic Neuropathy
  Eva Feldman
- Lipid Regulation and the Ketogenic Diet in Diabetic Neuropathy
  Douglas Wright
- Mitochondrial Pathways in Diabetic Neuropathy
  James W. Russell

The scientific content would provide information about the pathogenesis of diabetic neuropathy and how specific molecular targets or pathways may be used to treat or ameliorate diabetic neuropathy. The session will present recent research in the field and outline progress in diabetic neuropathy. The research will be used to outline an approach to improve treatment of diabetic neuropathy.

SS15
Silver Hall

Differential Diagnosis of Neuropathies: Inherited or Acquired

Chair: Davide Pareyson

- Demyelinating Neuropathies: Inherited or Acquired?
  Davide Pareyson
- How to Avoid Misdiagnosis of Hereditary Amyloid Neuropathy (ATTRv)
  Chiara Briani
- Acute Recurrent Focal or Generalized Neuropathy: Inherited or Acquired?
  Stojan Peric

The differential diagnosis between hereditary and acquired neuropathies is sometimes a challenge and misdiagnoses may result in improper treatments and delay in administration of adequate effective treatment. A rationale clinical approach to avoid diagnostic pitfalls will be provided together with insights into the interplay between the immune system and the hereditary neuropathies.

SS16
Meeting Studio 211+212

Chemotherapy and Neuropathy

Chair: Michael Lunn

- Update on Chemotherapy Neuropathy
  Nathan Staff
- POEMS and Lymphomatous Neuropathy – Novel Treatments
  Michael Lunn
- Checkpoint Inhibitors and Neuropathy
  Divyanshu Dubey

The differential diagnosis between hereditary and acquired neuropathies is sometimes a challenge and misdiagnoses may result in improper treatments and delay in administration of adequate effective treatment. A rationale clinical approach to avoid diagnostic pitfalls will be provided together with insights into the interplay between the immune system and the hereditary neuropathies.

WS09
Meeting Studio 214+216

Advances in Treatment of Antibody-Mediated Neuropathies

Chair: Andreas Steck

- Paraneoplastic Neuropathies: Diagnosis and Treatment
  Divyanshu Dubey
- Immunological Markers in CIDP: New Clues to Treatment
  Elba Pascual Goni
- Advances in Treatment of MAG Neuropathy
  Andreas Steck

The field of antibody mediated neuropathies has undergone major advances with the identification of autoantibodies directed at paranodal antigens in the case of CIDP and at the myelin associated glycoprotein in MAG neuropathy. The concept of paraneoplastic neuropathies will also be discussed.

12:45 - 14:15

Exhibit Hall (Grand Hall 2)

Lunch Break & ePoster Session

13:00 - 14:00

Meeting Studio 211+212 / Copper Hall / Silver Hall

Industry Supported Symposia

14:15 - 15:45

SS17
Silver Hall

TTR Amyloidosis

Chair: Violaine Plante Bordeneuve

- Early Diagnosis of TTR Amyloidosis
  Violaine Plante Bordeneuve
- Update on Therapies for TTR Amyloidosis
  Teresa Coelho
- Retinal and CNS TTR Amyloidosis – an Emerging Problem
  Luisa Sousa

Comprehensive review of diagnosis and novel therapies for TTR amyloidosis to include early diagnosis, gene silencing and editing therapies and the emerging problem of CNS TTR amyloidosis.

SS18
Gold Hall

Developing Novel Therapies for Inherited Neuropathies

Chair: Michael Sereda

- Classical Pharmacological Therapy
  Michael Sereda
- Development of a Targeted Therapy by siRNA for Charcot-Marie-Tooth 1A Neuropathy
  Liliane Massad-Massade
- Gene Therapy and Gene Editing
  Kleopas Kleopa

Despite huge progresses in the genetic diagnosis of these heterogeneous disorders and the elucidation of pathogenetic mechanisms underlying some of these forms, no therapies are still available for Charcot-Marie-Tooth neuropathies.

This might be due to the huge genetic heterogeneity; to the fact that animal models not always reproduce human phenotypes, particularly for axonal CMT2; to the limited numbers of natural history studies, and finally to the lack of informative and robust outcome measures for many of the CMT subtypes.

Nevertheless, in the past years proof-of-concept of efficacy has been provided at the preclinical level for several therapeutical strategies. Some of them are being translated at the clinical level, such as the PTX1003-based pharmacological approach (phase III) or the ASO-based gene dosage reduction of PMP22 for CMT1A.

In this session, we will describe the potential of the most prosing and interesting approaches ranging from classical pharmacology to gene therapy and gene editing.

SS19
Copper Hall

Update, Controversies and Treatment in Diabetic Neuropathies

Chair: James B. Dyck

- Advances, Controversies, and Treatment in Diabetic Polyneuropathy
  James Russell
- Advances, Controversies, and Care of Treatment Induced Diabetic Neuropathy
  Roy Freeman
- Advances, Controversies, and Treatment in Diabetic Lumbosacral Radiculoplexus Neuropathy
  P. James Dyck

To explore the advances in understanding of the pathophysiology and treatment of diabetic polyneuropathy, of treatment induced diabetic neuropathy and of diabetic lumbosacral radiculoplexus neuropathy.

Specifically to explore controversies in the treatment of these neuropathies in knowing how best to care for them. For example, whether treatment induced diabetic neuropathy and diabetic lumbosacral radiculoplexus neuropathy are immune mediated and should be treated with immunotherapy.

WS10
Meeting Studio 214+216

Pathophysiology of Guillain-Barré Syndrome

Chair: Peter Van den Bergh

- Pathology of GBS Focused on its Early Clinical Stage
  José Berciano
- Insights in GBS Pathophysiology Gained by Electrodiagnostic Studies
  Peter Van den Bergh
- Understanding GBS Immune Pathophysiology as it Relates to GM1
  Hugh Willison

Pathological studies in GBS are rare. Pathological events in the early stage of severe fatal GBS include endoneurial oedema leading to ischemic nerve injury. Implications for diagnosis and treatment will be discussed. Electrodiagnostic abnormalities are often found even in the early stages and major efforts have gone into classifying GBS as axonal or demyelinating. Prospective studies have tried to correlate electrodiagnostic abnormalities and criteria sets with ganglioside antibodies. The results indicate that subtyping GBS based on nerve conduction studies and antibodies is not straightforward. Ganglioside GM1 antibodies are relatively common in GBS. New insights into the distribution of GM1 in the nodal-paranodal area into why GM1 antibodies specifically affect the peripheral nerve when GM1 is omnipresent in the nervous system will be presented and discussed.

OS06
Meeting Studio 211+212

Plasma and Tissue Biomarkers For Neuromuscular Diseases

Chair: Corrado Angelini

- Serum Biomarkers for Myotonic Dystrophy Type 1 (DM1)
  Hanns Lochmuller
- Metabolic and Mitochondrial Biomarkers
  Corrado Angelini
- Peripherin and Neurofilaments in ALS
  Markus Otto

To date, CK levels have been routinely used to monitor muscle wasting and disease progression, but in several neuromuscular disease CK is generally normal or mildly elevated. In the recent years, circulating muscle specific microRNAs (myomi -RNAs) have been shown to be released in the bloodstream by muscles in response to physiological or pathological processes, and they could act as cell signaling, mediating cell-to-cell communication, regarding muscle repair, regeneration and remodeling. It has been observed that myo-miRNAs dysregulation occurs first in muscle biopsy and later extends to 388 plasma, suggesting a spill-over mechanism that might be of interest considering a possible role as biomarkers Moreover, myo-miRNAs expression has been shown to vary in presence of muscle atrophy in DM1Mtochondrial biomarkers are useful in diagnosis and clinical trials.

The advent of peripherin and neurofilament measurement helps in drug treatment of motor neuron diseases.

15:45 - 16:15

Exhibit Hall (Grand Hall 2)

PM Break

Scientific Program

Friday 8 July, 2022

16:15 - 17:45

PS06
Copper Hall

Selected Abstracts for Oral Presentation
Chair: Nicolas Deconinck

- SUNFISH: 3-year Efficacy and Safety of Risdiplam in Types 2 and 3 Spinal Muscular Atrophy
  Nicolas Deconinck

- Impact of Nusinersen on Caregiver Experience and HRQoL in Presymptomatic SMA: NURTURE Study Results
  Thomas O Crawford

- Combination of antisense oligonucleotide therapy with BIO101 demonstrates synergistic beneficial effects in severe SMA-like mice
  Cynthia Bezier

- RAINBOWFISH: Preliminary Efficacy and Safety Data in Risdiplam-Treated Infants with Presymptomatic Spinal Muscular Atrophy
  Laurent Servais

- Risdiplam: Pharmacokinetic, Pharmacodynamic, Safety and Efficacy Exposure Response Analyses
  Heidemarie Kletzl

- Matching-adjusted Indirect Comparison of Risdiplam Versus Nusinersen in Type 1 Spinal Muscular Atrophy: 2-year Update
  Neil Hawkins

PS07
Silver Hall

Selected Abstracts for Oral Presentation
Chair: Mazen Dimachkie

- Dysregulation of ER import proteins in IMNM with particular differences between SRP54+ and HMGCR+ patients
  Corinna Preusse

- Autoantibodies Against TRIM72/MG53 in Dysferlinopathy Patients and Mouse Models Decrease Sarcolemmal Membrane Repair Capacity
  Noah Weisleder

- A Structural Variant of the C-Terminal Prion-like Domain of TDP-43 Causes Vacuolar Muscle Degeneration
  Pedro Ervilha Pereira

- A Randomized, Double-Blind, Placebo-Controlled Study of Arimoclomol in Patients with Inclusion Body Myositis
  Mazen Dimachkie

- Clinical and Magnetic Resonance Imaging of Muscles in anti-Mi2B Inflammatory Myositis
  Akshata Huddar

- Key features for morphological classification of idiopathic inflammatory myopathies in children
  Schänzer Anne

RS - WFN JOINT SESSION
Gold Hall

Regional Aspects of Neuromuscular Diseases - a joint WFN-ICNMD session
Chair: Wolfgang Grisold and Riadh Gouider

- Neuromuscular Diseases in Latin America
  Renato Verdugo

- Neuromuscular Disease in Africa
  Imen Kacem

- Neuromuscular Disease in Thailand
  Rawiphan Witoonpanich

PS08
Meeting Studio 211+212

Selected Abstracts for Oral Presentation

Chair: James Russell

- A Recurrent Missense Variant In ITPR3 Is Associated With Demyelinating CMT
  Danique Beijer

- SCREEN4PN; a Novel iPSC Testing Platform for Efficient Evaluation of Compounds for CMT Neuropathies
  Tamira Van Wermeskerken

- Biomarkers and Outcome Parameters: A Global Natural History Study on SORD Neuropathy
  Maike Dohrn

- Response Predictors to Patisiran Treatment in Non-endemic ATTRv Patients
  Laura Martínez-Vicente

- Muscle Ultrasound as a Potential Progression Marker in Hereditary Neuropathies
  Natalie Winter

- Administration of AICAR, an AMPK Activator, Prevents and Reverses Diabetic Polyneuropathy (DPN) by Regulating Mitophagy
  James Russell

PS09
Meeting Studio 214+216

Selected Abstracts for Oral Presentation

Chair: Michel Toussaint

- Contractile Skeletal Muscle Organoids for Modelling Duchenne Muscular Dystrophy and Evaluating Potential Therapies
  Ainoa Tejedera-Villafranca

- Brody Disease: A Novel Potential Therapeutic Approach for This Rare Human Disease
  Eylem Emek Akyurek

- In Vivo Validation of the Efficacy of the CFTR Corrector C17 in Sarcoglycanopathy
  Martina Scano

- REN001, PPARδ Agonist, Preserves Muscle Strength and Promotes Recovery of Muscle Atrophy After Leg Immobilization
  Madhu Davies

- Impact of Mechanical Stretch on Nuclear Shape and Chromatin Organization in Skeletal Muscle
  Saline Jabre

- Pilot project of Home Mechanical Ventilation in Ukrainian patients with Duchenne Muscular Dystrophy
  Michel Toussaint

Scientific Program

Saturday 9 July, 2022

08:30 - 10:30

PL04
Copper Hall

New Developments in Diagnosis and Treatment of Myasthenia Gravis and Congenital Myasthenic Syndrome

Chair: Amelia Evoli

- Mechanism of Disease and Therapeutic Rescue of DOK7 Congenital Myasthenia and MuSK-Myasthenia Gravis
- Julien Oury

- Genetic Causes of Congenital Myasthenic Syndromes
- David Beeson

- The Emerging Treatment Landscape for Myasthenia Gravis
- Amelia Evoli

This session will highlight new developments in diagnosis and treatment of autoimmune and genetic neuromuscular transmission disorders.

10:30 - 11:15

Level 0 Foyer

AM Break

11:15 - 12:45

SS20
Meeting Studio 211+212

Clinical Research Update - Neuromuscular Transmission Disorders

Chair: Anna Kostera

- Epidemiology of Myasthenia Gravis
- Anna Kostera-Pruszczyk

- Correlation Between Muscle MRI and Genotype in Myasthenic Syndromes
- Atchayaram Nalini

- Efficacy of Efgartigimod in Generalized Myasthenia Gravis: Myasthenia Gravis Composite Score Analysis From ADAPT
- Jan de Bleecker

This session will entertain the latest clinical and scientific developments in autoimmune mediated myasthenia gravis and congenital myasthenia related to epidemiology, pathophysiology and imaging.

SS21
Meeting Studio 214+216

Inherited Disorders of Neuromuscular Transmission

Chair: Rita Horvath

- The Interface of Neuromuscular Transmission and Mitochondrial Diseases
- Rita Horvath

- New Synaptic and Presynaptic Defects of the Neuromuscular Junction
- Pedro M. Rodriguez Cruz

- Genetic Epidemiology of Congenital Myasthenic Syndromes
- Hanns Lochmuller

This session will cover the latest genetic and clinical discoveries for congenital myasthenic syndromes.

SS22
Gold Hall

Myasthenia Gravis Therapies

Chair: Angela Vincent

- Clinical Trial Update for Myasthenia Gravis
  Vera Bril
- Pregnancy and Treatment Considerations in Myasthenia Gravis and Congenital Myasthenic Syndrome
  Jacqueline Palace
- Role of Autoantibodies in Diagnosis, Treatment Choice and Monitoring of Myasthenia Gravis
  Angela Vincent

This session will entertain the latest clinical and scientific developments in autoimmune mediated myasthenia gravis.

OS08
Copper Hall

Emergencies in Neuromuscular Diseases

Chair: Zohar Argov

- Rhabdomyolysis and Acute Myopathies
  Zohar Argov
- Myasthenic Crisis
  Marianne de Visser
- ICU in NMD
  Maxwell Damian

This clinically oriented course targets the physicians who face patients presenting with acute weakness. At times this maybe a team work with a major role of the NMD expert. The main symptoms and signs of each condition will be discussed and clues to each diagnosis will be presented. General and specific treatments will be reviewed.

While this field has always been a very important topic in the basic and advanced training of young physicians, in current times with COVID19 pandemic this became a frequent problem for all practicing physicians.

OS09
Silver Hall

A Postpandemic Roadmap for New Technologies Including Remote Assessment in Neuromuscular Diseases

Chair: Gabriele Siciliano

- Use of Telemedicine and Home Infusion
  Gabriele Siciliano
- Machine Learning, Robotics and Assisting Technologhies in Rehabilitation
  Tina Duong
- Quick Clinical Outcome Assessments
  Corrado Angelini

The Postpandemic roadmap has required developing methods to assess individuals through telehealth and enuring robotic assistance in intensive care Units. Isolation requirements may impact on treatment regimens requiring hospital procedures (i.e. spinraza, myozyme, IVIg and rituximab infusions or treatments related to clinical trials).

These treatments should typically not be stopped, but when possible moved to a non-hospital setting (home-visiting or outreach nurses). IVIg can be changed to subcutaneous immunoglobulin.The limitations and beefits of such approache will be covered.Isolation requirements may impact on treatment regimens requiring hospital procedures (i.e. spinraza, myozyme, IVIg and rituximab infusions or treatments related to clinical trials). These treatments should typically not be stopped, but when possible moved to a non-hospital

setting (home-visiting or outreach nurses). IVIg can be changed to subcutaneous immunoglobulin.

Augmented Rehabilitation,machine learning and assistive technologies to promote inclusion in Labor and Decrease occuational stress were used and will be implemented with new clinical outcome assessments.

12:45 - 14:15

Level 0 Foyer

Lunch Break & ePoster Session

14:15 - 15:45

OS07
Gold Hall

Neuromuscular Complications of COVID-19 and Vaccination

Chair: Anthony Amato

- Overview of COVID-19 Infection and NM Disease Associated with COVID-19 Infection (e.g., GBS/neuritis, Myositis)
- Anthony Amato

- Management of COVID-19 Patients with Pre-exisiting Neuromuscular Conditions
- Maria Isabel Leite

- Neuromuscular Diseases Associated with COVID-19 Vaccines
- Pushpa Narayanaswami

Review how clinical care, teaching, and research had to be restructured during the pandemic, risks of COVID-19 in patients with neuromuscular disease (particularly those on immunotherapies) and neuromuscular complications of COVID-19 and vaccinations.

WS11
Copper Hall

Evolving Autoantibody and Treatment Landscape in Myasthenia Gravis

Chair: Gil I. Wolfe

- Novel Autoantibodies in MG: Do They Matter?
- Mamatha Pasnoor

- Emerging Therapies and Controversies in MG
- Mazen Dimachkie

- The 2020 Update of the International Consensus-based Treatment Recommendations for Myasthenia Gravis
- Gil I. Wolfe

Identify antibodies supportive of the diagnosis of autoimmune MG; Describe the clinical presentation of MG based on antibody status; Discuss the utility of newer serologic tests in diagnosing MG; Discuss promising therapies under development for autoimmune MG; Review cautionary medications that can compromise clinical status in MG; Review the 2020 updates international consensus recommendations for the treatment of MG; Discuss the implications of checkpoint inhibitor use in relation to MG;

WS12
Silver Hall

Muscle or Neuromuscular Junction or Both ? Disorders with Overlapping Muscle and Neuromuscular Junction Features

Chair: Markus Ruegg

- Muscle and Neuromuscular Junction Autoimmune Complications of Immune Checkpoint Inhibitor Cancer Immunotherapy
  Anastasia Zekeridou

- Association of Maternal Fetal-specific AChR Antibodies With Early-onset Neuromuscular Disorders
  Heinz Jungbluth

- Mechanistic Insights Into the Loss of Muscle Mass and Function at High Age
  Markus A. Ruegg

The workshop would be to explore some of the questions regarding involvement of muscle and NMJ in autoimmune disease and ageing.

OS10
Meeting Studio 211+212

Sleep Disorders in Neuromuscular Diseases: Treatable Conditions

Chair: Valeria Sansone

- Myotonic Dystrophy Type 1 and 2 Sleep Wake Pattern
  Valeria Sansone

- Sleep-related Breathing Disorders in Motor Neuron Diseases and Post-poliio Syndrome
  Lea Leonardis

- Sleep Apneas in Polyneuropathies and Late-onset Pompe
  Peter Young

Sleep is a major fronteer both in clinical science and for the treatment of neuromuscular disorders
In DM1 and DM2 the arousal system and brain are variably deranged in juvenile and adult DM1 and less in DM2, drugs and Cognitive Behavioral Treatment are used inDM1 after OPTIMISTIC trial.

In Motor Neuron Diseases (MND) respiratory muscle weakness is prominent in ALS and Post-polio syndrome, in fact severe manifestations of MND may be associated with sleep-disordered breathing (SDB), including obstructive sleep apnea (OSA) and nocturnal hypoventilation (NH), but prevalence data are scarce.

In polyneuropathy several patients suffer from respiratory disorders in cases of juvenile onset CMT that might be treated by BiPAP ,also it has been observed in late onset Pompe disease patients that are on ventilator ,when they are treated with ERT that there is a decreased hours in ventilator and sleep in adult Pompe patients can be improved.

Recent studies have shown that sleep treatment for SDB improve also QoL in neuromuscular patients and promote a better daily function.

Myotonic Dystrophy has prominent hypersomnia and sleep apnea are other well-recognized manifestations that appear later.Excessive daytime sleepiness is often caused by a central dysfunction of sleep dysregulation, but all of sleep disorders have been reported.

Scientific Program

OS11
Meeting Studio 214+216

Gender Issues in Neuromuscular Disorders

Chair: Nicol Voermans

- Gender Issues in Motor Neuron Diseases: has the Disease a Different Course in Men and Women?
  Giorgia Querin
- Symptomatology of Carriers of X-linked NMD: Duchenne and XL-MTM
  Nicol Voermans
- Myotonic Dystrophy in Man and Women has Variable Consequence
  Guillaume Bassez

Females recruit faster alpha motor neuron than males,showing a sexual dimorphism ,they have smaller muscle fiber diameters showing sexual different course in ALS that is more severe in women.

FSHD and Limb girdle muscular dystrophies (LGMD), a genetically and clinically heterogeneous group of neuromuscular disorders, show in their manifestation and clinical signs gender differences in the disease severity.a new entity is Bethlem myopathy that has characteristic clinical course also FSHD and RyR myopathy have gender differential expression.

Cognitive Behavior Therapy (CBT) is a possible disease-modifying therapy that can slow DM1,as well as in Alzheimer and other neurodegenerative disease progression .CBT is available for Myotonic Dystrophy,according to OPTIMISTIC trial patient management is largely mediated by symptomatic therapies, such as the use of muscle relaxants or mexiletine for hand myotonia and speech therapists prescribe condensers for dysphagia.A gender effect is seen in this multisystemic disorder.

Saturday 9 July, 2022

15:45 - 16:15

Level 0 Foyer
PM Break

16:15 - 17:45

Gold Hall
Closing Ceremony

Exhibitor Information

Exhibits & ePoster Hall - Hours

Location: Grand Hall 2

Wednesday 6 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 1)
Thursday 7 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 2)
Friday 8 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 3)
Saturday 9 July	13:00 – 14:00 (Poster Session 4 - Level 0 Foyer)

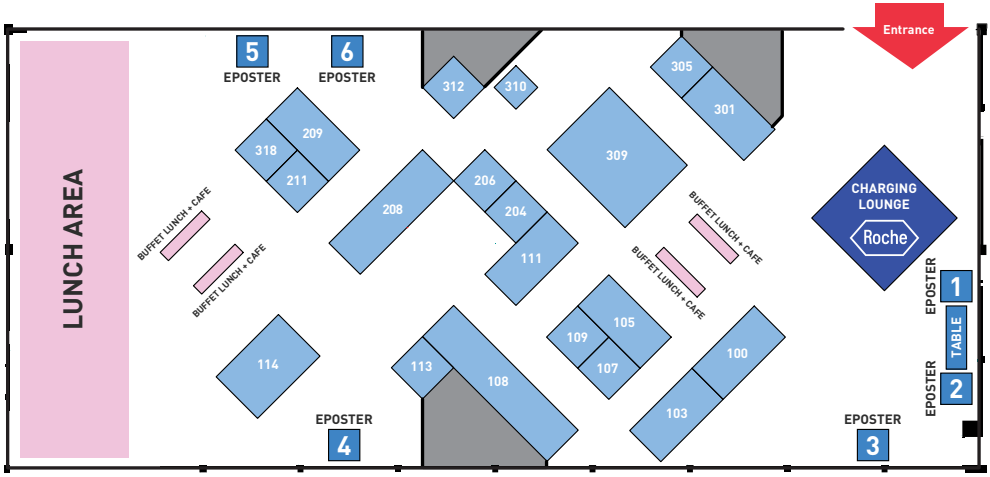
Poster Session Supported by

sanofi

Exhibitors — Booth

Novartis Gene Therapies	**100**
Janssen Research and Development	**103**
PTC - Duchenne muscular dystrophy (DMD)	**105**
Scholar Rock, Inc.	**107**
UCB	**108**
Grupo Ferrer	**109**
Sarepta Therapeutics	**111**
Alnylam Pharmaceuticals	**113**
Takeda International AG	**114**
Dyne Therapeutics	**204**
SERB Specialty Pharmaceuticals	**206**
Alexion Pharma GmbH	**208**
Astellas Gene Therapies	**209**
Lupin Neurosciences	**211**
Sanofi	**301**
iThera Medical	**305**
Roche	**309**
Journal of Neuromuscular Diseases	**310**
Amylyx Pharmaceuticals	**312**
ICNMD 2024 Perth Western Australia	**318**
European Neuromuscular Centre (ENMC)	**Level 0 Walkway**

Floorplan + Booth #s



Exhibitor Listing

Alexion Pharma GmbH

Booth #208 www.alexion.com

Alexion, AstraZeneca Rare Disease, is a leader in rare diseases for 30 years focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialisation of life-changing medicines. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries.

Alnylam Pharmaceuticals

Booth #113 www.alnylam.be

Founded in 2002, Alnylam has led the translation of RNA interference (RNAi) from Nobel Prize-winning discovery into an innovative, new class of medicines. Alnylam's vision is to harness the potential of RNAi therapeutics to transform the lives of people living with diseases for which treatment options are limited or inadequate.

Amylyx Pharmaceuticals

Booth #312 www.amylyx.com

Amylyx Pharmaceuticals is a clinical-stage biopharmaceutical company working on developing a novel therapeutic for amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases.

Astellas Gene Therapies

Booth #209 www.astellasgenetherapies.com

Astellas Gene Therapies is an Astellas Center of Excellence developing genetic medicines with the potential to deliver transformative value for patients. We are currently exploring three gene therapy modalities: gene replacement, exon skipping gene therapy, and vectorized RNA knockdown and will also advance additional Astellas gene therapy programs toward clinical investigation.

Exhibitor Listing

Dyne Therapeutics

Booth #204 www.dyne-tx.com

Dyne Therapeutics is building a leading muscle disease company dedicated to advancing innovative life-transforming therapeutics for people living with genetically driven diseases. With its proprietary FORCE™ platform, Dyne is developing modern oligonucleotide therapeutics that are designed to overcome limitations in delivery to muscle tissue seen with other approaches.

European Neuromuscular Centre (ENMC)

Level 0 Walkway www.enmc.org

The mission of the European Neuromuscular Centre (ENMC) is to encourage and facilitate communication and collaboration in the field of neuromuscular research with the aim of improving diagnosis and prognosis, finding effective treatments and optimizing standards of care to improve the quality of life of people affected by neuromuscular disorders.

Grupo Ferrer

Booth #109 www.ferrer.com

At Ferrer we want to make a positive impact in society and we do so by reinvesting a significant part of our profits in initiatives with social and environmental impact, as well as in our people. In order to fulfill our purpose, we offer transformative therapeutic solutions, with an increasing focus on pulmonary vascular and interstitial lung diseases and neurological disorders.

ICNMD 2024 Perth Western Australia

Booth #318 www.icnmd.org

Come to stand 318 to learn about Perth, Western Australia and ICNMD 2024.

Exhibitor Listing

iThera Medical

Booth #305 www.ithera-medical.com

iThera Medical develops and markets optoacoustic imaging (OAI) systems for preclinical and clinical research. OAI utilizes the photoacoustic effect to visualize and quantify optical contrast in deep tissue, at high spatiotemporal resolution. In the area of neuromuscular diseases, OAI has shown the potential to assess changes in tissue composition related to inflammation and fibrosis.

Janssen Research and Development

Booth #103 www.janssen.com

At Janssen, we're creating a future where disease is a thing of the past. We're the Pharmaceutical Companies of Johnson&Johnson, working tirelessly to make that future a reality for patients everywhere by fighting sickness with science, improving access with ingenuity, and healing hopelessness with heart.

Journal of Neuromuscular Diseases

Table #310 www.iospress.com/catalog/journals/journal-of-neuromuscular-diseases

The Journal of Neuromuscular Diseases facilitates progress in understanding the molecular genetics, pathogenesis, diagnosis, and treatment of neuromuscular diseases. Guided by Editors-in-Chief Carsten G. Bönnemann and Hanns Lochmüller, and published by IOS Press, JND is a vehicle for research that will improve understanding and lead to effective treatments of neuromuscular diseases.

Lupin Neurosciences

Booth #211 www.lupin.com

Lupin Neurosciences, a division of Lupin Atlantis Holdings SA, is focusing on neuromuscular disorders following the launch of the company's first Orphan Drug, NaMuscla® (mexiletine), indicated for the treatment of myotonia symptoms in adult patients with non-dystrophic myotonia. NaMuscla® is currently commercially available in Germany, UK, France, Spain and Norway. Please consult summary of product characteristics, available **here** EU-LUP-2201-00005.

Exhibitor Listing

Novartis Gene Therapies

Booth #100 www.novartis.com

Novartis Gene Therapies is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are working to turn promising gene therapies into proven treatments. We are powered by an extensive manufacturing footprint enabling us to bring gene therapy to patients around the world.

TELL ME MORE

PTC – Duchenne Muscular Dystrophy (DMD)

Booth #105 www.ptcbio.com

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development, and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. To learn more about PTC, please visit our website and Social Media pages.

TELL ME MORE

Roche

Booth #309 www.roche.com

Neuroscience is a cornerstone of our future. We will continue to push the boundaries of scientific understanding, together with our partners, to achieve clinical advancements and solve some of the greatest challenges in neuroscience today. Our hope is to create a tomorrow where nervous system disorders no longer limit human potential – to preserve what makes us who we are.

Sanofi

Booth #301 www.sanofi.com

Sanofi is an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives. Our teams across the world strive to transform the practice of medicine, turning the impossible into the possible for patients.

TELL ME MORE

Exhibitor Listing

Sarepta Therapeutics

Booth #111 www.sarepta.com

At Sarepta, we are working with urgency to develop breakthrough therapies to treat genetic diseases. Currently, we have more than 40 investigational therapies in various stages of development—many already in late-stage clinical trials.

Scholar Rock, Inc.

Booth #107 www. scholarrock.com

Scholar Rock is focused on the discovery and development of innovative medicines for treatment of serious diseases. Following positive results from TOPAZ, apitegromab, a selective inhibitor of active myostatin in skeletal muscle, is advancing through development with SAPPHIRE, a double-blind, placebo-controlled, phase 3 RCT, in patients with later onset SMA.

SERB Specialty Pharmaceuticals

Booth #206 www.serb.eu

SERB Specialty Pharmaceuticals is bringing lifesaving drugs that meet high unmet medical needs. SERB has extended its Rare Diseases portfolio in both Neurology (with treatments for severe epileptic and Lambert Eaton Myasthenic Syndrome) and Metabolic Disorders. SERB makes available the only EMA registered drug for symptomatic treatment of adult LEMS patients.

Takeda International AG

Booth #114 www.takeda.com

Takeda is a patient-focused, R&D-driven biopharmaceutical company committed to bringing better health to people worldwide since 1781. Takeda aspires to unlock the potential of plasma in our relentless drive to improve the lives of patients with chronic inflammatory demyelinating polyneuropathy, multifocal motor neuropathy, and other rare auto immune mediated diseases.

UCB

Booth #108 www.ucb.com

UCB, Brussels, Belgium is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions to transform the lives of people living with severe diseases of the immune system or of the central nervous system.

TELL ME MORE

Industry Supported Symposia

13:00 - 14:00

PTC THERAPEUTICS®

measured by moments

ISS01
Silver Hall

Long-term treatment experience in patients with nmDMD

Join our expert faculty in an interactive, peer-to-peer discussion treatment of patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). Through the use of real-world clinical case studies, the challenges patients face in transitioning from paediatric to adult care will also be discussed, alongside the key outcomes for these patients.

- Prof. Luca Bello
- Assistant Professor of Neurology, Department of Neurosciences DNS, University of Padova, Italy

- Prof. Ros Quinlivan
- MRC Centre for Neuromuscular Disease, National Hospital for Neurology and Neurosurgery, London, UK

NOVARTIS

ISS02
Copper Hall

Building on Real-world Spinal Muscular Atrophy Gene Therapy Experience to Transform Patient Care

Symposium Objectives:

- Provide an overview of the Gene Therapy landscape and examine current unmet needs for all SMA patient populations
- Demonstrate our evolving understanding on presymptomatic patients with SMA in the context of real-world evidence
- Discuss real-world evidence of the safety and efficacy of gene therapy, including patients with SMA beyond those studied in clinical trials

Agenda & Speakers:

13:00 – 13:05
Welcome & Introduction
- Dr. Sandra P. Reyna (Chair)
- VP, Global Medical Affairs and Head of Therapeutic Area SMA, Novartis Gene Therapies, USA

13:05 – 13:15
Gene Therapy Now: Current Landscape and Durable Treatment Responses
- Prof. Dr. Liesbeth De Waele
- Pediatric Neurologist, University Hospitals Leuven and Head of Neuromuscular Reference Centre (NMRC) for Children, Leuven, Belgium

Industry Supported Symposia

Wednesday 6 July, 2022

13:15 – 13:30
Newborn Screening or Presymptomatic Patients? Adapting The Nosology to Tailor Individualized Prognosis
- Prof. Laurent Servais
- Professor of Pediatric Neuromuscular Diseases, MDUK Oxford Neuromuscular Centre, Oxford, United Kingdom and Professor of Child Neurology University and University Hospital of Liège, Belgium

13:30 – 13:50
New Era of SMA Management: RWE and Clinically Meaningful Treatment Outcomes
- Dr. Andreas Ziegler
- Medical Doctor and Consultant for Pediatric Neurology, Department of Neuropediatrics and Metabolic Medicine, Centre for Childhood and Adolescent Medicine, University Hospital Heidelberg, Germany

13:50 – 14:00
Q&A and Closing Remarks
- Dr. Sandra P. Reyna (Chair)

SAREPTA THERAPEUTICS

ISS03
Studio 211+212

Exploring the Treatment Galaxy: AAV-based Gene Transfer Therapies for Patients Living with Neuromuscular Diseases

Explore the role of the dystrophin-associated protein complex in neuromuscular diseases and the development of gene transfer therapies in the management of Duchenne and limb-girdle muscular dystrophy with leading experts.

Chair:
- Volker Straub, MD, PhD
- Director, Dubowitz Neuromuscular Centre Co-Director, MRC Centre for Neuromuscular Diseases
- UCL Great Ormond Street Institute of Child Health & Great Ormond Street Hospital for Children
- NHS Foundation Trust, London, UK

Speakers:
- Eugenio Maria Mercuri, MD, PhD
- Professor of Paediatric Neurology Head of the Paediatric Neurology and Psychiatry Unit
- Gemelli Hospital
- Catholic University Foundation, Rome, Italy

- Craig Zaidman, MD
- Professor of Neurology and Paediatrics Washington University, Missouri, United States

Industry Supported Symposia

18:00 - 19:00

ISS04
Copper Hall

Intrahepatic Cholestasis in X-Linked Myotubular Myopathy (XLMTM)*

Agenda & Speakers:

18:00 – 18:15
Proposed mechanisms of intrahepatic cholestasis in XLMTM: lessons from animal models
- Emanuela Pannia, PhD
- The Hospital for Sick Children, Toronto, Ontario, Canada

18:15 – 18:25
Case studies of intrahepatic cholestasis as part of the natural history of XLMTM
- Andrés Nascimento, MD
- Hospital Universitari Sant Joan de Déu, Barcelona, Spain

18:25 – 18:40
Safety update for ASPIRO study
- Michael Lawlor, MD, PhD
- Medical College of Wisconsin, Milwaukee, Wisconsin, USA

18:40 – 19:00
Q&A and discussion
- Moderated by Michael Lawlor, MD, PhD

**Title and agenda are subject to change*

Industry Supported Symposia

Thursday 7 July, 2022

07:00 - 08:00

ISS06
Studio 214+216

Channelling the myotonia diagnosis

Can we refer patients with myotonic disorders for specialist care more quickly, and are we able to provide them with a more accurate diagnosis than has previously been possible?
Discover how prompt, accurate, disease recognition can help patients with myotonic disorders to understand (and reduce the impact of) their symptoms.

Agenda & Speakers

Recognising myotonic disorders
- Chair: Prof. Dr Kristl Claeys
- University Hospitals Leuven, Belgium

DM Scope Registry: Classifying myotonic disorders to improve diagnosis
- Dr Guillaume Bassez
- CHU Pitié Salpetrière, Assistance Publique Hôpitaux de Paris, France

Why don't we meet patients sooner?
- Prof. John Vissing
- Rigshospitalet and Copenhagen University, Denmark

Concluding comments
- Prof. Dr Kristl Claeys

ISS07
Copper Hall

Managing Generalised Myasthenia Gravis: A Tale of Two Targets

Join Profs. Pushpa Narayanaswami, James F. Howard, and Heinz Wiendl discuss the need for different targeted therapies in patients with generalised Myasthenia Gravis (gMG), and how these therapies could impact future therapeutic management.

The symposium will focus on improving clinicians':
- Knowledge of the factors that contribute to disease progression in gMG
- Ability to interpret emerging data for emerging therapies in the management of uncontrolled gMG and potential impact on practice
- Ability to individualise care of patients with gMG

Agenda & Speakers:

Welcome and Introduction
- Pushpa Narayanaswami, MD, FAAN

Pathogenic Contributions in gMG: Why More Than One Therapeutic Approach is Needed
- Heinz Wiendl, MD

Emerging Targeted Therapies for Uncontrolled gMG: A Tale of Two Targets
- James F. Howard Jr., MD

With the Patient at the Forefront, How Could Standard of Care in gMG Evolve in the Future?
- Entire Panel

"Ask the Faculty" and Take-Home Messages
- Entire Panel

Industry Supported Symposia

Thursday 7 July, 2022

13:00 - 14:00

ISS08
Copper Hall

From traditional to targeted: Innovations in the changing gMG landscape

The argenx-sponsored satellite symposium 'From traditional to targeted: Innovations in the changing gMG landscape' will delve into the current and emerging treatment landscape in gMG. The panel of experts will explore the significant impact on patients' lives of this rare neuromuscular disorder and the unmet needs associated with current therapies. They will also discuss how innovations could shift therapeutic strategies towards targeted, individualized treatment.

Agenda & Speakers:

Welcome and introduction, and patient testimonial video
- Prof. Jan De Bleecker

Today's gMG treatment reality: Time for a change?
- Prof. Benedikt Schoser

The emerging treatment landscape: Hope for tomorrow
- Prof. M Isabel Leite

In conversation: Navigating the changing landscape in gMG
- All speakers

ISS09
Silver Hall

Pompe Disease – How Could We Achieve Better Outcomes for Patients?

Join us at the Sanofi sponsored symposium "Pompe Disease – How Could We Achieve Better Outcomes for Patients?" at 13:00pm (CEST), Silver Hall of the Square Convention Centre. Experts Prof. Mark Roberts, Prof. Jordi Díaz Manera, and Prof. Pascal Laforêt will speak on the importance of early and ongoing patient management to prevent Pompe disease progression, and clinically meaningful changes in patient outcomes.

Agenda & Speakers:

Introduction
- Mark Roberts

Demonstrating importance of early treatment in Pompe disease: MRI data
- Jordi Díaz-Manera

Consequences of treatment interruption: Effects of the pandemic
- Pascal Laforêt

Clinically meaningful changes in outcomes in a new treatment
- Mark Roberts

Closing and Q&A
- Mark Roberts, Jordi Díaz-Manera, Pascal Laforêt

Industry Supported Symposia

ISS10
Gold Hall

Everyday SMA: its daily impact and optimal management from infancy to adulthood

Key objective:
- To highlight the real-world burden of SMA across all types and ages
- To understand the current standard of care in SMA, including an overview of the treatment landscape and available data in children, adolescents, and adults with Type 1, 2 or 3 SMA
- To share best practices for the management of individuals with SMA from birth to adulthood, and to discuss how its implementation may reduce some of the remaining burden for these individuals

Agenda:

Welcome and introductions
Prof Laurent Servais (Chair)

The broad spectrum of individuals with SMA
Prof Laurent Servais

Real-world burden for all types of SMA
All faculty

Q&A
All faculty

Summary and close
Prof Laurent Servais

Speakers:

Professor Laurent Servais
MDUK Oxford Neuromuscular Centre, Oxford, UK (Chair)

Dr Liesbeth Dewaele
University Hospitals Leuven, KU Leuven, Leuven, Belgium

Professor Maggie Walter
Ludwig-Maximilians-University, Munich, Germany

Industry Supported Symposia

Thursday 7 July, 2022

18:00 - 19:00

ISS11
Copper Hall

CIDP: Early treatment escalation in non IVIg responders.

Intravenous immunoglobulins (IVIg) have proven their efficacy and are considered as a first-line therapy in treating patients with chronic inflammatory demyelinating polyneuropathy (CIDP). Efficacy of IVIg is around 80% of all treated patients. For those who don't respond, different treatment options are possible. In this debate, we will compare two different approaches: aggressive and conservative approaches and discuss the optimal time to consider the response of CIDP patients to IVIg.

Speakers:
- Chairman: Prof. Hans-Peter Hartung – Germany
- Pros: Dr. Luis Querol – Spain
- Cons: Prof. Helmar Lehmann – Germany

ISS12
Silver Hall

Targeting complement to fight chronic burden in gMG

The chronic and fluctuating nature of generalized myasthenia gravis (gMG) has a substantial impact on a patient's daily life, including their physical, emotional, social, and economic wellbeing. This symposium will provide the opportunity to engage in discussion with our expert faculty, who will be considering the burden of this chronic disease and illustrating how terminal complement inhibition, which targets a primary driver of AChR Ab+ gMG, can improve patient outcomes.

Agenda:
- Dr. Pushpa Narayanaswami (Chair)

Managing gMG as a chronic disease
- Prof. Andreas Meisel

Complement inhibition: Targeting the primary driver of AChR Ab+ gMG
- Prof. Nico Melzer

Everyday experience with using a complement inhibitor
- Dr. Pushpa Narayanaswami

Panel Discussion
- All speakers

Industry Supported Symposia

07:00 - 08:00

Fulcrum Therapeutics

ISS13
Studio 211+212

Measuring Progression in FSHD: Implications for Clinical Trials

This symposium is focused on the importance and utility of appropriate outcomes measures in clinical trials to assess the progression of FSHD, a relentlessly progressive disease leading to significant disability and impact on quality of life. Reachable Workspace (RWS) is a valid and reliable clinical outcome measure of function whereby there has been a historical lack of tools to evaluate upper extremity function in patients with FSHD. RWS focuses on shoulder and proximal arm function (among the muscle groups most affected by FSHD), correlates with quality-of-life measures, and is sensitive to change in disease progression. RWS demonstrated nominally statistically significant and clinically meaningful benefit to muscle function in ReDUX4, a Phase 2 double blind placebo-controlled trial of losmapimod, a small molecule selective p38 MAP kinase inhibitor being developed by Fulcrum Therapeutics as the first disease modifying therapy for FSHD. Results from ReDUX4 and other clinical trials informed the study design of REACH, the first Phase 3 double blind placebo-controlled clinical trial in FSHD.

Speakers:
- Olga Mitelman, MD
- Senior Vice President, Medical Affairs Fulcrum Therapeutics, Inc., United States
- Hanns Lochmüller, MD, PhD, FAAN
- Professor of Neurology, University of Ottawa, Canada
- Sabrina Sacconi, MD, PhD
- Professor of Neurology, Nice University Hospital, France

Takeda

ISS14
Silver Hall

The Maze of Mimics: Can You Identify CIDP?

Can you recognise the signs of chronic inflammatory demyelinating polyradiculoneuropathy (CIDP)? Meet our patients and put your diagnostic skills to the test as we navigate through the maze of mimics.

Agenda & Speakers:

Welcome, introductions and objectives
- Claudia Sommer/Filip Eftimov

An improved approach to CIDP
- Claudia Sommer

Applying the CIDP guidelines: Making an accurate diagnosis
- Filip Eftimov

Applying the CIDP guidelines: An interactive patient journey
- Claudia Sommer/Filip Eftimov

Q&A with faculty
- Both speakers

Industry Supported Symposia

13:00 - 14:00

ISS15
Copper Hall

Uncovering the missing pieces of the SMA puzzle?

A review of current SMA treatments and how clinical and patient collaboration can provide the missing pieces to optimize outcomes for people with 5q spinal muscular atrophy (SMA)

Agenda & Speakers:
13:00 - 13:05
Chairperson's welcome and introduction: What do we still need to know to optimize outcomes in people with 5q SMA?
- Professor Valeria Sansone
- Centro Clinico NeMO, Milano, Italy

13:05 -13:25
Piecing together the evidence: What we have learned and what is missing?
- Professor Julie Parsons
- Children's Hospital Colorado, Aurora, Colorado, USA

13:25 - 13:45
Incorporating Patients' and carers' perspectives: Another missing piece of the puzzle?
- Professor Juan F Vázquez Costa
- Hospital Universitario de La Fe, Valencia, Spain

13:45 - 14:00
Chairperson-moderated Q&A: What do you think is missing?
- Professor Valeria Sansone
- Centro Clinico NeMO, Milano, Italy

Biogen have funded and organized this event but have had no other involvement in the ICNMD congress agenda.

The content for this symposium was approved by the Scientific Program Committee as an independent activity held in conjunction with the 17th International Congress on Neuromuscular Diseases. This symposium is not sponsored or endorsed by ICNMD 2022.

Biogen's treatment for 5q SMA will be discussed, alongside other treatments for 5q SMA. The Summary of Product Characteristics for its product will be available on request from Biogen staff at the event.

Biogen-164071 May 2022

ISS16
Silver Hall

Navigating success in ALS: Pathophysiological pathways, the patient journey, and treatment landscape

An opportunity to gain expert insights into the pathophysiology of ALS, the changing treatment landscape, and consider how we might have a positive impact on the patient journey.

Speakers:
- Professor Philip van Damme (Chair)
- Professor Orla Hardiman
- Professor Susanne Petri

Industry Supported Symposia

ISS17
Studio 211+212

Optimising the care of individuals with DMD in gene therapy clinical trials: learnings from caregivers, PAGs and previous trial experience

A review of current SMA treatments and how clinical and patient collaboration can provide the missing pieces to optimize outcomes for people with 5q spinal muscular atrophy (SMA)

Objectives:
- Share insights and experiences from NMD and DMD gene therapy clinical trials in relation to site readiness and activation, and how to meet the safety needs for people with NMD and DMD who are receiving gene therapy
- Communicate patient organisation and caregiver insights on the needs of people with DMD in gene therapy clinical trials
- Highlight the role of patient organisations in raising awareness of clinical trials among people with DMD and their caregivers

Agenda:
13:00 – 13:05
Welcome and introduction
Prof. Dr Nicolas Deconinck

13:05 – 13:15
Introduction to DMD, gene therapies and DMD clinical trials
Dr Andrés Nascimento Osorio

13:15 – 13:30
Preparing sites for gene therapy clinical trials to ensure clinical and participant needs are met
Prof. Dr Nicolas Deconinck & Dr Andrés Nascimento Osorio & All Faculty

13:30 – 13:40
The role of patient organisations in clinical trials for DMD
Alejandra Pereda Alonso & All faculty

13:40 – 13:50
Audience Q&A
All faculty

13:50 – 14:00
Forum summary and close
Prof. Dr Nicolas Deconinck

Speakers:
Dr Andrés Nascimento Osorio
Paediatric Neurologist, Hospital Sant Joan de Déu, Barcelona, Spain

Prof Dr Nicolas Deconinck
Paediatric Neurologist, Ghent University Hospital – UZ Gent, Ghent, Belgium

Alejandra Pereda Alonso
Duchenne Parent Project, Spain

1. Novartis Gene Therapies (2021). ZOLGENSMA (onasemnogene abeparvovec) Summary of Product Characteristics. **2.** Mendell JR, *et al. N Engl J Med.* 2017;377(18):1713–22. **3.** Mendell JR, *et al. JAMA Neurol.* 2021;78(7):834–41. **4.** Day JW, *et al. Lancet Neurol.* 2021;20(4):284–93. **5.** Mercuri E, *et al. Lancet Neurol.* 2021;20(10):832–41. **6.** Strauss KA, *et al.* Oral presentation presented at: EAN Congress. June 19–22 2021. **7.** Strauss KA, *et al.* Poster presented at: MDA Clinical and Scientific Congress. April 13–16, 2022. **8.** Novartis (2022). Novartis delivers solid sales and profit growth. Strong performance of in-market brands supports confidence in mid-term growth outlook. Available at: https://www.novartis.com/sites/novartis_com/files/q1-2022-media-release-en.pdf. Date accessed: May 2022.

Navigating success in ALS: Pathophysiological pathways, the patient journey, and treatment landscape

Satellite Symposium, ICNMD 2022, Brussels

DON'T MISS THE OPPORTUNITY TO GAIN EXPERT INSIGHTS INTO THE PATHOPHYSIOLOGY OF ALS, THE CHANGING TREATMENT LANDSCAPE, AND CONSIDER HOW WE MIGHT HAVE A POSITIVE IMPACT ON THE PATIENT JOURNEY

Chair:

Professor Philip van Damme

Professor of Neurology, University Hospital Leuven and the Department of Neurosciences, University of Leuven, Belgium

Presenters:

Professor Orla Hardiman

Professor of Neurology, Trinity College, University of Dublin, Ireland

Professor Susanne Petri

Professor of Neurology, Hannover Medical School, Department of Neurology, Hannover, Germany

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13:00–14:00
SILVER HALL, SQUARE – BRUSSELS CONVENTION CENTRE
FRIDAY 8 JULY 2022

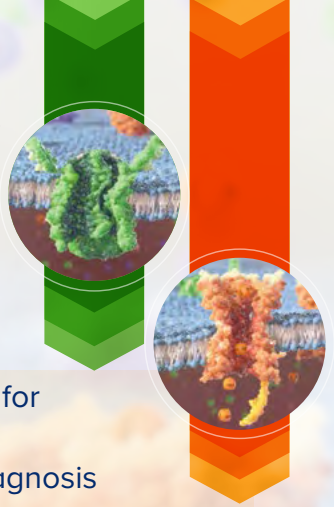
Amylyx symposium
AMYLYX®

Date of preparation: May 2022
Job number: NP-00176

Channelling the myotonia diagnosis

Thursday 7th July, 7–8 am

Room 214–216



Can we refer patients with myotonic disorders for specialist care more quickly?

Can we provide them with a more accurate diagnosis than has been possible?

Discover how prompt, accurate, disease recognition can help patients with myotonic disorders to understand (and reduce the impact of) myotonia.

Moderator/Chair

Prof. Dr Kristl Claeys
University Hospitals Leuven, Belgium.

Faculty

Dr. Guillaume Bassez
Pitié-Salpêtrière University Hospital, France.

Prof. John Vissing
Rigshospitalet and Copenhagen University, Denmark.

Presentations

Recognising myotonic disorders
Prof. Dr Kristl Claeys

DM Scope Registry: Classifying myotonic disorders to improve diagnosis
Dr Guillaume Bassez

Why don't we meet patients sooner?
Prof. John Vissing

Concluding comments
Prof. Dr Kristl Claeys

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LUPIN NEUROSCIENCES

Lupin Neurosciences is a Specialty Division of Lupin Atlantis Holdings SA

EU-NDM-2205-00003

Sarepta Therapeutics' Industry Symposium:

Exploring the Treatment Galaxy

AAV-based Gene Transfer Therapies for Patients Living with Neuromuscular Diseases

Wednesday, 6 July 2022
13:00 - 14:00 CEST

Studio 211-212
SQUARE Brussels Convention Centre
Brussels, Belgium

Explore the role of the dystrophin-associated protein complex in neuromuscular diseases and the development of gene transfer therapies in the management of Duchenne and limb-girdle muscular dystrophy with leading experts.

Chair

Volker Straub, MD, PhD
Harold Macmillan Professor of Medicine
Director, The John Walton Muscular Dystrophy Research Centre

Deputy Dean, Translational and Clinical Research Institute
Faculty of Medical Sciences
Newcastle University and Newcastle Hospitals NHS Foundation Trust
Newcastle upon Tyne, UK

Speakers

Eugenio Maria Mercuri, MD, PhD
Professor of Paediatric Neurology
Head of the Paediatric Neurology and Psychiatry Unit
Gemelli Hospital
Catholic University Foundation
Rome, Italy

Craig Zaidman, MD
Professor of Neurology and Paediatrics
Washington University
Missouri, United States

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SAREPTA THERAPEUTICS


There is always
more to be done
Our commitment to improving the lives of people with severe diseases runs deep. Our work doesn't stop when we close the lab door or analyse the last sample. Always looking for the next discovery, the next innovation – now and into the future.
Inspired by patients.
Driven by science.

ePosters Schedule

Wednesday 6 July, 2022

Wednesday, Screen 1

Chair: Stefano Previtali

eP01.01.01
Genetic, Proteomic and Morphological Characterisation of Two Children With Novel Nonsense Mutations of BVES (POPDC1)
Anne Schänzer

eP01.01.02
Cardiac Analysis Reveals Morphological Alterations in an Intermediate Mouse Model of Spinal Muscular Atrophy
Nithya Nair

eP01.01.03
Respiratory Follow-Up In Children With Spinal Muscular Atrophy - A Descriptive Study
Inês Pereira

eP01.01.04
Worldwide Prevalence of Home Mechanical Ventilation in Neuromuscular Disorders
Michel Toussaint

eP01.01.05
De Novo and Dominantly Inherited SPTAN1 Mutations Cause Spastic Paraplegia and Cerebellar Ataxia
Jonathan De Winter

eP01.01.06
Use of NGS for Diagnosis of Asymptomatic Hyperckemia in Childhood
Pilar Marti

eP01.01.07
Prevalence of Titinopathy in India
Aishwarya Dhall

eP01.01.08
Optimisation of a Cell-Based Strategy for Rapid Evaluation of Compounds in Myotonic Dystrophy Type I
Andrea López-Martínez

eP01.01.09
Spectrum of Muscular Dystrophies in India
Mehar Chand Sharma

eP01.01.10
Median Nerve Ultrasound in Carpal Tunnel Syndrome
Imen Kacem

Wednesday, Screen 2

Chair: Maike Dohrn

eP01.02.01
Clinical Features and the Novel p.K357E Mutation in a Cohort of Patients With mfn2-Related Neuropathy
Elena Abati

eP01.02.02
Patient-Reported Symptom Burden of Charcot-Marie-Tooth Disease Type 1A (CMT1A): Findings from a Real-World Digital Study
Youcef Boutalbi

eP01.02.03
One Cause, Many Courses: Leveraging Whole-Genome Sequencing for Comprehensive Modifier Studies in CMT1A
Maike Dohrn

eP01.02.04
Work Impacts in Charcot-Marie-Tooth Disease Type 1A (CMT1A): Findings from a Real-World Digital Study
Youcef Boutalbi

eP01.02.05
R298C LMNA Mutation Can Cause either Peripheral Neuropathy, Cardiomyopathy or Both: A Case Series Study
Leila Tamaoui

ePosters Schedule

eP01.02.06
Overlap Between Hereditary Sensory-Motor Neuropathy (HSMN) And Chronic Inflammatory Demyelinating Polyradiculoneuropathy (CIDP)
Manon Hustinx

eP01.02.07
Plasma and Skin Biomarkers for Charcot Marie Tooth Disease
Michael Shy

eP01.02.08
Depression in Patients with Charcot-Marie-Tooth Disease Type 1A (CMT1A): Findings from a Real-World Digital Study
Youcef Boutalbi

eP01.02.09
Novel Variant in the Stalk Domain of KIF5A in a Patient With CMT2-Like Phenotype
Eleni Liouta

Wednesday, Screen 3

Chair: James Russell

eP01.03.01
Deacetylation of E3 Ubiquitin Ligase NEDD4-1 by Sirtuin1 Regulates Axonal Growth and Treats Diabetic Neuropathy
James Russell

eP01.03.02
CuidAME Registry: Using Process Automation and Machine Learning Technology to Build SMA Data Analytics Repository
Sonia Segovia

eP01.03.03
Spinal Muscular Atrophy Disease Registries: Overview and Recent Progress
Stephanie Raynaud

eP01.03.04
Cognitive Assessment of Spinal Muscular Atrophy
Sanae Akodad

eP01.03.05
SMA-REACH-UK, Adult-SMA-REACH and UK SMA-Patient-Registry an Integrated Model: Transition of Data and Longitudinal Data Collection.
Sonia Segovia

eP01.03.06
Proposal of New Functional Motor Scale to Evaluate Muscle Fatigue in Adult SMA Patients
Gabriele Siciliano

eP01.03.07
CuidAME: A New Registry for Longitudinal Data Collection of Spanish SMA Patients
Sonia Segovia

eP01.03.08
Swallowing Evaluation in Treated SMA patients – A Pilot Prospective Study
Nicolas Deconinck

eP01.03.09
Safety and Effectiveness of Onasemnogene Abeparvovec Alone or with Other Disease-Modifying Therapies: Findings from RESTORE
Laurent Servais

eP01.03.10
Brown-Vialetto-Van Laere Syndrome, Temporary Clinical Stabilization with Intravenous Immunoglobulin (IVIg) and MRI Abnormalities
Edna Julieth Bobadilla

eP01.03.11
A Compound Heterozygous Mutationin Calpain-1 Identifies a New Gene for Spinal Muscular Atrophytype-4
Gonzalo Perez Siles

eP04.05.01
Improved Quality of Life in Patients with X-Linked Myotubular Myopathy (XLMTM) Treated with Resamirigene Bilparvovec
Laurent Servais

ePosters Schedule

Wednesday, Screen 4

Chair: Anthony Amato

eP01.04.01
COVID-19: Retrospective Analysis in Neuromuscular Disease Patient's Impact on Healthcare, Quality of Life and Anxiety
Laura Buscemi

eP01.04.02
COVID-19-Related Neuropathy in Colombia: The Experience During the First 23 Months Of Pandemic
Laura Peña Guzmán

eP01.04.03
Myositis And Myocarditis With Anti-Jo-1 Antibodies Following SARS-CoV-2 mRNA Vaccination Or COVID-19 Infection
Myrthe Willems

eP01.04.04
Guillain-Barre Syndrome in 220 Patients with COVID-19
Josef Finsterer

eP01.04.05
IVIg Treatment in Chronic Inflammatory Neuropathies During the COIVD19 Pandemic
JUDYTA Barańska

eP01.04.06
Quality of Life of Myasthenia Gravis Patients During COVID-19 Pandemic – One Year Follow Up
Aleksandar Stojanov

eP01.04.07
COVID Spike Antibodies in Neuromuscular Conditions: A KU Experience
Mamatha Pasnoor

eP01.04.08
Parsonage-Turner Syndrome after COVID-19 Vaccination
Elisabeth Van Boxstael

eP01.04.09
Multi-Centre Study to Assess the Safety of Alglucosidasi and of Laronidasi in Home Infusion Setting
Maurizio Scarpa

eP01.04.10
Clinical Course of Four Neuromuscular Disease Patients Infected COVID-19
Akiko Ishii

Wednesday, Screen 5

Chair: Anneke van der Kooi

eP01.05.01
Muscle Inflammation Drives Mitochondrial Dysfunction in Inclusion Body Myositis
Stefanie Meyer

eP01.05.02
B Cell Receptor Profiling before and after IVIg Treatment in Idiopathic Inflammatory Myopathies
Anneke van der Kooi

eP01.05.03
Multi-Muscle Pathology Assessment in Inclusion Body Myositis: Post-mortem Study in Two Cases
Stefanie Glaubitz

eP01.05.04
Design of a Global Phase 2/3 Randomized, Placebo-Controlled Trial of Ravulizumab in Adult Dermatomyositis
Rohit Aggarwal

eP01.05.05
A Prospective Diagnostic Accuracy Study of Multi-Modality Testing in Patients Suspected of a Treatable IIM
Renske Kamperman

ePosters Schedule

eP01.05.06
Fibroblast Model Unveils New Molecular Insights in Inclusion Body Myositis
Judith Cantó-Santos

eP01.05.07
Prevalence and Clinical Correlation of Myositis-Specific and Myositis-Associated Autoantibodies in Indian Myositis Cohort
Bandana Jassal

eP01.05.08
Immune-Mediated Necrotizing Myopathy: An Emerging Disorder
Sofía Portela Sánchez

eP01.05.09
COVID-19 and Vaccination Against Sars-CoV-2 in Patients With Myasthenia Gravis From Belgrade, Serbia
Stojan Peric

Wednesday, Screen 6

Chair: Volker Straub

eP01.06.01
Two-Years Prospective Natural History Study in 24 Adult LGMDR12 Patients: Clinical and Radiological Outcome Measures
Bram De Wel

eP01.06.02
Assessing the Relationship of Patient Reported Outcome Measures with Functional Status in Dysferlinopathy
Heather Hilsden

eP01.06.03
Clinical and Genetic Features in Two Families Carrying Novel and Reported DYSF Variants
Birute Burnyte

eP01.06.04
Description of Motor Function in Duchenne Muscular Dystrophy in a Center of Expertise in Colombia
Sandra Castellar

eP01.06.05
Preliminary Results from MLB-01-003: An Open Label Phase 2 Study of BBP-418 in LGMD2I
Douglas Sproule

eP01.06.06
The Founder Mutation TRAPPC11 c.1287+5G>A is a Frequent Cause of Limb-girdle Muscular Dystrophy in Roma Population
Carlos Ortez

eP01.06.07
A Familial Case with Phenotypic Differences in a CAV3 Pathogenic Variant
Seung-ah Lee

eP01.06.09
Molecular Diagnosis of Muscular Dystrophy Using the LGMD Gene Panel in Adult Neurology
Seok-Jin Choi

eP01.06.10
Observational Study: the Quality of Life in Patients with Alpha-Sarcoglycan, Beta-Sarcoglycan and Gamma-Sarcoglycan Gene Mutation
Serena Paniga

ePosters Schedule

Thursday 7 July, 2022

Thursday, Screen 1

Chair: Sabrina Sacconi

eP02.01.01
Value of Muscle Ultrasound in Pediatric Neuromuscular Patients
Hanna Kuepper

eP02.01.02
Place of Muscle Ultrasound in the Diagnosis and Follow-up of Pediatricneuromuscular Diseases
Susana Quijano-Roy

eP02.01.03
Machine Learning in Ultrasound-Guided Differentiation of Myopathic From Neurogenic Patterns: A Pilot Study
Elisabeth Chroni

eP02.01.04
Novel Titin Mutation Responsible for Hereditary Myopathy With Early Respiratory Failure in Adult Moroccan Man
Leila Tamaoui

eP02.01.05
Deep Learning-Based Electrodiagnosis of Needle-Electromyography
Ilhan Yoo

eP02.01.06
Normative Values for Commonly Used Nerve Conduction Studies in Russian Population
Maria Kovalchuk

eP02.01.07
Cardiac MRI in Duchenne and Becker Muscular Dystrophy
Manu S G

eP02.01.08
Artificial Intelligence Based Automatic Muscle MRI Segmentation: Towards a Generalized Solution for Quantitative Imaging
Eduard Snezhko

eP02.01.09
Neuropathies Amidst the Pandemic: Remote Phenotype Validation and Assessment of Patient Needs
Maike Dohrn

eP02.01.10
Subclinical Status of Dysferlinopathy
Sergey Bardakov

Thursday, Screen 2

Chair: Imem Kacem

eP02.02.01
Overview of Patients With Chronic Inflammatory Demyelinating Polyneuropathy of the Neuromuscular Reference Center of LIèGE
Margaux Poleur

eP02.02.02
Intravenous Immunoglobulin Therapy in Patients with Chronic Inflammatory Demyelinating Polyneuropathy: A Systematic Literature Review
Colin Anderson-Smits

eP02.02.03
Chronic Inflammatory Demyelinating Polyradiculoneuropathy in Patients in Diabetic and Non-Diabetic Patients: A Comparative Study
Imen Kacem

eP02.02.04
Identical Late Responses in Early Stages of Guillain-Barré Syndrome: A-Waves or Repeater F-Waves
Dimitra Veltsista

eP02.02.05
An Unusual Clinical Evolution in Anti-contactin-1 Positive CIDP
Yann Vivier

ePosters Schedule

Thursday 7 July, 2022

eP02.02.07
Characteristics and Epidemiology of Patients with Multifocal Motor Neuropathy in Latvia
Marija Roddate

eP02.02.08
Herpes Zoster May Be a Trigger for Lumbosacral Radiculoplexus Neuropathy
Catarina Aragon Pinto

eP02.02.09
Acute Worsening of Anti-mag Neuropathy Following Treatment With Rituximab
Suraj Muley

eP02.02.10
Clinical and Prognostic Characteristics of Guillain-Barre Syndrome Associated With COVID-19, Is This Coincidental?
Behnaz Ansari

Thursday, Screen 3

Chair: Wolfgang Loscher

eP02.03.01
Risdiplam in Children With Spinal Muscular Atrophy: Real-World Experience After One Year of Treatment
Marta Gomez Garcia De La Banda

eP02.03.02
Cost-Utility Analysis of Risdiplam Compared with Onasemnogene Abeparvovec in Spinal Muscular Atrophy Type 1
Matthias Bischof

eP02.03.03
SAPPHIRE: Efficacy and Safety of Apitegromab in Later-Onset SMA; Phase 3 Trial in Progress
Thomas O. Crawford

eP02.03.04
Safety of Onasemnogene Abeparvovec in Patients With SMA in Real Clinical Practice
Kristina Nevmerzhitskaya

eP02.03.05
Treatment of Spinal Muscular Atrophy with Onasemnogene Abeparvovec in Switzerland
Georg M. Stettner

Thursday, Screen 4

Chair: TBC

eP02.04.01
Tocilizumab in Therapy Myasthenia Gravis Patients With COVID-19
Vesna Martic

eP02.04.02
Myasthenia Gravis Demographics Re-Visited
Nanna Witting

eP02.04.03
Incidence of Skin Changes in Patients with Myasthenia Gravis Prescribed Mycophenolate or Azathioprine
Anahit Mehrabyan

eP02.04.04
Quality of Life in Myasthenia Gravis and Correlation of MG-QOL15 With Other Functional Scales
Laura Porras

eP02.04.05
Barriers and Facilitators to Exercise in Auto-Immune Myasthenia Gravis
Simone Birnbaum

eP02.04.06
Clinical Characteristics of Patients With Seronegative Myasthenia Gravis
Rebecca Kjær Andersen

ePosters Schedule

eP02.04.07
TREAT-NMD Global Registry Network: Facilitating 12 Years of Neuromuscular Drug Development
Cathy Turner

eP02.04.08
Immunofluorescence Signal Intensity Measurements as a Semi-quantitative Tool to Assess Sarcoglycan Expression in Muscle Biopsy
Simona Zanotti

eP02.04.09
Three Cases of Congenital Fibre Disproportion With Etiology Other Than Congenital Myopathy
Emna Farhat

Thursday, Screen 5

Chair: Kristl Claeys

eP02.05.01
One-year ENDEAVOR Data (Ambulatory, ≥4 to <8-year-olds): Phase 1b Trial of Delandistrogene Moxeparvovec in DMD
Craig Zaidman

eP02.05.02
Whole Genome Sequencing in a Pair of Duchenne Muscular Dystrophy Siblings with Discordant Cognitive Phenotype
Luca Bello

eP02.05.03
Growth Patterns and Loss of Ambulation in Boys with Duchenne Muscular Dystrophy (DMD)
Georgia Stimpson

eP02.05.04
An Alu-Mediated Insertion in the DMD Gene Canceled Out by Exon 15 Splicing
Alberto Budillon

eP02.05.05
FU-5Cv: Inducible Muscle-Specific Downregulation of Utrophin in Dystrophic Mice to Better Mimic Duchenne Muscular Dystrophy
Laurence Anne Neff

eP02.05.06
Preclinical Assessment of Therapeutic Cocktails in Dystrophic Mice: Tamoxifen Combined to Metformin, Citrulline and Steroids
Laurence Anne Neff

eP02.05.07
Roles of Ltbp4 and Abcc6 on the Phenotype of Mouse Models of Duchenne Muscular Dystrophy
Laurence Anne Neff

eP02.05.08
Integrated Analyses of Data from Clinical Trials of Delandistrogene Moxeparvovec in DMD
Stefanie Mason

eP02.05.09
Phenotypic Variability of Becker Muscular Dystrophy: A Detailed Clinical Characterization Protocol Towards Trial Readiness
Gabrielle Siciliano

eP02.05.10
Non-neuromuscular Manifestations in a Colombian Patient with Megaconial Congenital Muscular Dystrophy
Edna Julieth Bobadilla

Thursday, Screen 6

Chair: Stojan Peric

eP02.06.01
DYNE-101 Corrects the DM1 Splicing Phenotype of hTfR1/DMSXL Mice and Is Well Tolerated in NHPs
Aaron Novack

eP02.06.02
Myotonic Dystrophy Type 2 and Autoimmune Diseases
Stojan Peric

eP02.06.03
Nuclear Envelope Dysfunction in Myotonic Dystrophy Type 1
Sandra Rebelo

eP02.06.04
Does Small Fiber Neuropathy Contribute to Chronic Muscle Pain in Patients with Myotonic Dystrophy?
Viviane Chantal Schmitt

eP02.06.05
Frequently Used Outcome Measures to Evaluate Muscle Strength in Patients with Myotonic Dystrophy Type 1
Sandra Rebelo

eP02.06.06
Clinical Features of the UK Myotonic Dystrophy Patient Registry
Helen Walker

eP02.06.07
Myofibrillar Myopathy: Clinico-Genetic Spectrum from an Indian Neuromuscular Center
Abel Thomas Oommen

eP02.06.08
Muscular Dystrophies Due to Collagen VI Mutations
Joana Coelho

ePosters Schedule

Friday 8 July, 2022

Friday, Screen 1

Chair: Marianne de Visser

eP03.01.01
Validation of the Individualized Neuromuscular Quality of Life Questionnaire in Korean Patients with Neuromuscular Diseases
Seung-ah Lee

eP03.01.02
Improving Clinical Trials in Neuromuscular Diseases: The TREAT-NMD Advisory Committee for Therapeutics (TACT)
Cathy Turner

eP03.01.03
Impact of Independence in Mobility on Independence Outdoors in People With Neuromuscular Conditions
Karen Wong

eP03.01.04
Neuropsychological and Behavioral Profile in a Cohort of Becker Muscular Dystrophy Pediatric Patients
Michele Tosi

eP03.01.05
Evolution of Gait Parameters and Influence of Orthotics on Gait in a Patient With Dysferlinopathy
Meredith James

eP03.01.06
Predictors of Gait in Patients with Late-Onset Pompe disease
Théo Maulet

eP03.01.07
Simplified and Non-invasive Optical Motion Tracking of Respiratory Dynamics in the MDX Mouse Model.
Jana Zschüntzsch

eP03.01.08
Serum Neurofilament Light Chain as a Marker of Nervous System Damage in Myopathies
Jochen Schaefer

eP03.01.09
Troponin T in Spinal and Bulbar Muscular Atrophy
Luca Bello

eP03.01.10
Urinary Titin as a Biomarker of Myotonic Dystrophy Type 1
Endre Pál

eP03.01.11
InGene 2.0: An Intelligent Technological Approach to Genotype-Phenotype Relationship Study in Rare Neuromuscular Diseases
Gabriele Siciliano

Friday, Screen 2

Chair: Edyardo Nobile Orazio

eP03.02.01
Coexistence of Charcot-Marie-Tooth Disease and Chronic Inflammatory Demyelinating Polyradiculoneuropathy
Zoran Vukojevic

eP03.02.02
HINT1-Related Autosomal Recessive Axonal Neuropathy With Neuromyotonia (ARAN-NM). Presentation of a Greek Pedigree
Maria Moschou

eP03.02.03
The Importance of Functional Assessment In Hereditary Sensory and Autonomic Neuropathy
Luisa Fernanda Castaño Herrera

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ePO3.02.04
Dose-Exposure-Efficacy Response Relationships for Intravenous Immunoglobulin,10% in Patients with Multifocal Motor Neuropathy
Zhaoyang Li

ePO3.02.05
Long-Term Course of Chronic Inflammatory Demyelinating Polyneuropathy: Clinical and Neurophysiological Outcomes
Evgeniya Melnik

ePO3.02.06
Hematological Effects of Intravenous Immunoglobulin Therapy in Patients with Neuromuscular Diseases – A Retrospective Analysis
Pieter Olivier

ePO3.02.07
Man-In-The-Barrel Syndrome as a Manifestation of Multiple Myeloma Relapsed
Dionis Vallejo

ePO3.02.08
Sensory Polyneuropathy Associated With Vitamin D Deficiency
Sa-yoon Kang

ePO3.02.09
Diagnosing Small Fiber Neuropathy Remains Challenging in Sarcoidosis - Preliminary Data
Lisette Raasing

ePO3.02.11
In Vitro Comparison Between Different 10% Intravenous Immunoglobulin Preparations
Rabye Ouaja

Friday 8 July, 2022

Friday, Screen 3

Chair: TBC

ePO3.03.01
MANATEE: GYM329 (RO7204239) in Combination with Risdiplam Treatment in Ambulant Children with Spinal Muscular Atrophy
Francesco Muntoni

ePO3.03.02
Progression ofthe Revised Hammersmith Scale items in patients with Spinal Muscular Atrophy treated with Nusinersen
Sandra Castellar

ePO3.03.03
Critical Review of the Spanish Pharmacoclinic Protocol for the Monitoring of Nusinersen Treatment
Laura Carrera

ePO3.03.04
Experience in the Treatment With Pyridostigmine Monotherapy, in Patients With Spinal Muscular Atrophy in Colombia
Sandra Mesa

ePO3.03.05
Onasemnogene Abeparvovec Treatment Outcomes by Patient Weight at Infusion: Initial Findings from the RESTORE Registry
Laurent Servais

ePO3.03.06
Treatments and Outcomes for Patients with Spinal Muscular Atrophy Type 2: Findings from RESTORE Registry
Laurent Servais

ePO3.03.07
Characteristics and Epidemiology of Amyotrophic Lateral Sclerosis in a Health-Care Area in Northwestern Spain
Alejandra Espinosa Trujillo

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Friday 8 July, 2022

eP03.03.08
Characterization of the ALS patients' population in a large Italian Centre
Francesca Bianchi

eP03.03.09
Descriptive Analysis of Bulbar Amyotrophic Lateral Sclerosis (ALS) In the Northern Area of Tenerife
Helena Pérez Pérez

eP03.03.10
Atypical Onset of Amyotrophic Lateral Sclerosis: Seven Cases Report
Leila Tamaoui

eP03.03.11
Cognitive Decline in ALS Patients. MoCA Score and CSF Biomarkers (T-Tau, P-Tau, ß 42)
Sofía Portela Sánchez

Friday, Screen 4

Chairs: Isabel Leite & Pushpa Narayanswami

eP03.04.01
Effectiveness of Eculizumab Treatment for Generalized Myasthenia Gravis in Us Clinical Practice: gMG Registry Data
Srikanth Muppidi

eP03.04.02
Pharmacokinetics and Pharmacodynamics of Nipocalimab in Healthy Participants and Patients with Generalized Myasthenia Gravis
Anne-Gaëlle Dosne

eP03.04.03
Zilucoplan in Myasthenia Gravis: Safety and Tolerability Results From the Phase 3 Randomised RAISE Study
Maria Isabel Leite

eP03.04.04
Baseline Characteristics of Patients with Myasthenia Gravis Enrolled in an Expanded Access Programme for Efgartigimod
Jan L. De Bleecker

eP03.04.05
Promise-MG: Results of a Multicenter Comparative Effectiveness Study of Myasthenia Gravis Treatments
Pushpa Narayanaswami

eP03.04.06
The Association Between QMG Scores and Health-related Quality of Life in Myasthenia Gravis Patients
Cynthia Qi

eP03.04.07
Oral Tobacco, but Not Smoking, Is Associated With an Increased Risk of Myasthenia Gravis
Malin Petersson

eP03.04.08
Productivity Losses for Generalized Myasthenia Gravis Patients and their Caregivers: Association with Disease Severity
Saiju Jacob

eP03.04.09
Efficacy and Safety of Tolebrutinib in Adults with Generalized Myasthenia Gravis: Phase 3 Study Design
Sana Syed

eP03.04.10
Serological Diagnostics of MuSK Myasthenia Gravis in South Korea: Comparison of ELISA, RIPA and CBA
Young Nam Kwon

eP03.04.11
Clinical Differences Between Ocular and Generalized Myasthenia Gravis
Kasper Holst Axelsen

ePosters Schedule

Friday, Screen 5

Chair: Corrado Angelini

eP03.05.01
Effects of ERT on Cardiac Function in Classic Infantile Pompe Disease- 19 Years of Follow-up
Linda Scheffers

eP03.05.02
Safety of Home-Based Infusion of Alglucosidase Alfa in Adults With Late-Onset Pompe Disease
Imke Ditters

eP03.05.03
Minimal Clinical Important Difference for Lung Function and Walking Ability in Adult Pompe Patients
Aglina Lika

eP03.05.04
Effect of Alglucosidase Alfa Dosage on Survival and Walking Ability in Classic Infantile Pompe Disease
Imke Ditters

eP03.05.05
Czech Nationwide Screening of Pompe Disease – Case Reports
Livie Mensova

eP03.05.06
Involvement of Muscle Capillaries in Late Onset Pompe Disease (LOPD) With Childhood Onset
Corrado Angelini

eP03.05.07
Neuromuscular Symptoms and Gene Variants From a Long-Chain Fatty Acid Oxidation Disorder Gene Panel Program
Vanessa Rangel Miller

eP03.05.08
Natural History of Muscular Forms of Fatty Acid Beta-Oxidation Disorder: Description of 44 Patients
Alice Rouyer

eP03.05.09
A Case of VCP Mutation Featuring With Lobulated Myofiber, Motor Neuron Disease and Frontotemporal Dementia
Ju-hee Chae

eP03.05.10
Head Drop and Hyperckemia Associated With a Carnitine Palmitoyltransferase II (CPT2) Deficiency
Michela Bisciglia

eP03.05.11
Mitochondrial Network Disruption in Skeletal Muscles of the McArdle Mouse Model
Mónica Villarreal-Salazar

Friday, Screen 6

Chair: Josef Finsterer

eP03.06.01
Etiology, Genetics and Prevalence of Myopathies in the Population of Alicante (Spain)
Pablo Ros-Arlanzón

eP03.06.02
Far More than Vacuoles? Proteomic Profiling of Danon Disease Reveals a Striking Mitochondrial Phenotype
Felix Kleefeld

eP03.06.03
Global FKRP Registry - the Research Database for Limb Girdle Muscular Dystrophy R9 (2I))
Volker Straub

eP03.06.04
First Description of a Caveolin-3-Related Rippling Disease in a Peruvian Family
Edna Bobadilla

eP03.06.05
Paucisymptomatic Hyperckemia as a Phenotype of Myopathy Associated with ano5: Case Report
Cristian Correa

eP03.06.06
Artificial Intelligence Electrocardiogram-Derived Age Detects Accelerated Aging in LMNA Patients
Shahar Shelly

eP03.06.07
Design of 'Time Is Muscle': RCT on IVIg Add-on to Prednisone in Newly Diagnosed Myositis
Renske Kamperman

eP03.06.08
The PACE-DM1 tool: An Adapted Home-Based Physical Activity Program for DM1 to Counter Physical Deficiencies
Anne-marie Fortin

eP03.06.09
Photosensitive Epilepsy and Polycystic Ovary Syndrome as Manifestations Of MERRF
Josef Finsterer

ePosters Schedule

Saturday 9 July, 2022

Saturday, Screen 1

Chair: TBC

eP04.01.01
The Burden of Duchenne Muscular Dystrophy in Belgium: A Registry-Based Study
Marjan Cosyns

eP04.01.02
Improving and Harmonising Care Standards for Duchenne Muscular Dystrophy in the UK
Cathy Turner

eP04.01.03
Illness Perceptions And Quality Of Life In Adolescents With Neuromuscular Disorders And Caregivers
Sam Geuens

eP04.01.04
A Pilot T1-Weighted MRI Study to Evaluate Chronic Corticosteroid-Use in Duchenne Muscular Dystrophy on Brain
Sam Geuens

eP04.01.05
Immune Function Indices in Patients with Amyotrophic Lateral Sclerosis
Oksana Kononets

eP04.01.06
Duchenne Muscular Dystrophy Diagnostic Gaps in Primary Medical Chain
Tatiana Gremiakova

Saturday, Screen 2

Chair: Kristl Claeys

eP04.02.01
Nothing to Laugh About
Frédéric Supiot

eP04.02.02
Acute Arsenic Intoxication Presentation Guillain-Barre Syndrome Mimic in Oncohematological Patient
Antonio J. Gutiérrez Martínez

eP04.02.03
Six Months of Electroneumyography in Ouagadougou, a Great Experience
Olivier Kapto

eP04.02.04
A Case Report on Orbital Myositis
Eni Reka

eP04.02.05
Belgian Retrospective Survey of Hereditary Transthyretin-Mediated (hATTR) Amyloidosis Patients Treated With Patisiran in Real-World Practice
Jan de Bleecker

eP04.02.06
Predictive Factors of Response to Tafamidis in a Cohort of Non-endemic ATTRv Patients
Laura Martínez-Vicente

eP04.02.07
Quantitative Sensory Testing in patients diagnosed with ATTR Amyloidosis in Colombia: A Case Series
Edicson Ruiz

ePosters Schedule

Saturday 9 July, 2022

Saturday, Screen 3

Chair: TBC

eP04.03.01
Respiratory-Onset of ALS in a Pregnant Woman With a Novel SOD1 Mutation
Pegah Masrori

eP04.03.02
Association of APOE ε4 Allele With Survival in Amyotrophic Lateral Sclerosis Among Tunisian Cases
Imen Kacem

PS02.1
Comparison of Inflammation and Neurodegeneration Markers in CSF as Predictors of Survival in ALS Patients
Pegah Masrori

eP04.03.04
Systemic Genetic Screening of Korean Patients With Amyotrophic Lateral Sclerosis
Jin-ah Kim

eP04.03.05
New Mutation (Val31Gly) In PFN1 Gene Responsible for the Development of ALS18 in Bulgarian Pedigree
Teodor Angelov

eP04.03.06
Clinical Characteristics of c.63C>G (p.Phe21Leu) Variant in SOD1 Gene in Colombian Patients with ALS
Christian Correa

eP04.03.07
Serum Creatine Kinase and Creatinine in the Diagnosis and Prognostic Prediction of Amyotrophic Lateral Sclerosis
Imen Kacem

eP04.03.08
Potential Role of Mitochondrial Dysfunction in an Unusual Co-occurrence of ALS and Primary Biliary Cirrhosis
Pritikanta Paul

eP04.03.09
Hereditary Spastic Paraparesis Type 9A Mimicking ALS: A Case Report
Cristian Correa

eP04.03.10
International Phase 3 Trial Evaluating Sodium Phenylbutyrate/Taurursodiol in Amyotrophic Lateral Sclerosis (PHOENIX): Enrollment Update
Leonard H. van den Berg

Saturday, Screen 4

Chair: Schara-Schmidt Ulrike

eP04.04.01
Long Term Preliminary Safety and Efficacy Outcomes for X-Linked Myotubular Myopathy with Gene Replacement Therapy
Nancy L. Kuntz

eP04.04.02
FORCETM Platform Demonstrates Durable Dystrophin Expression in Mdx Mice and Favorable Safety Profile in NHPs
Aaron Novack

eP04.04.03
Givinostat for the Management of Becker Muscular Dystrophy: A Randomised, Placebo-Controlled, Double-Blind Study
Daniele Velardo

eP04.04.04
Post-Authorisation Safety Study of Mexiletine Treatment in Patients with Non-Dystrophic Myotonia: Methodology Overview
Alla Zozulya-Weidenfeller

eP04.04.05
An Open-Label, Non-Comparative Study of Mexiletine in Children and Adolescents with Myotonic Disorders: Methodology Overview
Alla Zozulya-Weidenfeller

ePosters Schedule

eP04.04.06
Readily Available Low-Cost Highly Effective Treatment for Inherited Muscle Disorders Diagnosed by Whole Exome Sequencing
Ioannis Tsiverdis

eP04.04.08
Diagnosing Necrotizing Myopathy With Hmgcr Antibodies – A Quest Proving Time is Muscle
Zoltan Zsigmond Major

eP04.04.09
Anti-SRP-Positive Immune-Mediated Necrotizing Myopathy Accompanied by Hashimoto Thyroiditis
Seol-Hee Baek

eP04.04.10
Necrotizing Autoimmune Myopathy Anti-HMGCR Antibodies and Demyelinating Polyneuropathy
Dionis Vallejo

eP04.04.11
Hereditary Inclusion Body Myopathy (HIBM) as a Rare Clinical Entity: A Case Report
Ivan Barbov

Saturday, Screen 5

Chair: Jeniffer Shoskes

eP04.05.02
Clinical and Genetic Features of the Myotubular and Centronuclear Myopathy Patient Registry Cohort
Joanne Bullivant

eP04.05.03
Novel Splicing Mutation in MTM1 Leading To Two Abnormal Transcripts Causes Severe Myotubular Myopathy
Luca Bosco

eP04.05.04
Severe Congenital Myopathy With Type II Fibers Atrophy Due to MYL1
Jesica Maria Exposito Escudero

eP04.05.05
Clinical and Pathologic Findings of Korean Patients With Selenon-Related Myopathy
Seung-ah Lee

eP04.05.07
RYR1-Related Congenital Myopathy in a Cohort of Peruvian Patients
Edna Bobadilla

eP04.05.08
Risk of Malignant Hyperthermia in Patients Carrying a Variant in the Ryanodine Receptor 1 Gene
Sarah Herdewyn

eP04.05.09
Congenital Myopathy Caused by Mutations in the Neubulin Gene Associated to Schizophrenia: A Case Report
Sofía Portela Sánchez

eP04.05.10
Clinical Features of the UK FSHD Patient Registry
Helen Walker

eP04.05.11
Safety and Tolerability of Losmapimod for the Treatment of FSHD
Jennifer Shoskes

Saturday, Screen 6

Chair: TBC

eP04.06.01
Automated Integrative Splicing Predictor Tool: Focus on Deep Intronic Variants Prioritization for the DMD Gene
Mariateresa Zanobio

eP04.06.02
The Epidemiology of Mutations of Dystrophin in the Hungarian Population
Szabolcs Udvari

eP04.06.03
Targeting NAPDH oxidases in Duchenne Muscular Dystrophy: Diapocynin Therapeutic Effect on Adult MDX Mice
Ghali Guedira

eP04.06.04
Eteplirsen Safety, Tolerability, and Pharmacokinetics in Young Patients with DMD Amenable to Exon 51 Skipping
Eugenio Mercuri

eP04.06.05
Description of Osmolyte Pathways in Maturing MDX Mice Reveals Altered Taurine and Sodium/Myo-Inositol Co-transporter Levels
Caroline Merckx

eP04.06.06
Non-dystrophic Myotonia; The Patient Journey to Diagnosis
Tim Aldwinckle

eP04.06.07
Sensory Polyneuropathy in Oculopharyngeal Muscular Dystrophy, It This a Novel Phenotypical Findings?
Gauthier Remiche

eP04.06.08
Effects of FGF21 Supplementation in Muscle Cells from Mitochondrial Disease Patients
Margarita Chudenkova

eP04.06.09
Clinical Characterization of Familial Hyperkalemic Periodic Paralysis with a SCN4A Met1592Val Mutation
Sa-yoon Kang

eP04.06.11
Evaluation of the Relationship between Genotype and Phenotype of Dystrophinopathy in Iranian Race
Behnaz Ansari

General Information

Access/Security

Name Badges will be provided to all delegates and participants and can be picked up at the ICNMD 2022 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 meeting space without their badge. There is a €100.00 reprint fee for any lost or misplaced badges.

Official Language

The official language of the ICNMD 2022 Congress is English. All sessions will be conducted in English.

Exhibits & ePoster Hall - Hours

Location: Grand Hall 2

Wednesday 6 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 1)
Thursday 7 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 2)
Friday 8 July	10:30 – 16:15 13:00 – 14:00 (Poster Session 3)
Saturday 9 July	13:00 – 14:00 (Poster Session 4 - Level 0 Foyer)

Poster Session Supported by

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ICNMD 2022 Mobile App

Download the congress mobile app today to connect with other attendees, create a detailed agenda, and to check out the ICNMD 2022 speakers and sponsors.

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SAREPTA THERAPEUTICS

Lost Property

Please report any lost or unattended items immediately to the ICNMD 2022 Registration Desk. Should you lose anything while at the ICNMD 2022 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 SQUARE Brussels Convention Centre.

General Information

Photographer / Videographer

An official photographer / videographer will be present during the Congress. By registering for the ICNMD 2022 Congress, you agree to have your picture taken. Photography and video captured may be used for marketing purposes for future ICNMD Congresses and Events.

Refreshment Breaks & Lunches

Location: Exhibits & Poster Hall – Grand Hall 2

6 - 9 July

AM Refreshment Break	10:30 – 11:15
General Lunch Break	12:45 – 14:15
PM Refreshment Break	15:45 – 16:15

Saturday 9 July Refreshment Breaks & Lunches - Level 0 Foyer

In addition to these breaks, water stations will be available throughout the venue.

Water Stations Supported by

Registration Desk Hours

Location: Registration Hall

Monday 4 July	14:00 – 18:00
Tuesday 5 July	07:00 – 21:00
Wednesday 6 July	07:30 – 18:30
Thursday 7 July	06:30 – 18:30
Friday 8 July	06:30 – 18:30
Saturday 9 July	08:00 – 17:00

Delegate Help Desk Supported by

measured by moments

Speaker Ready Room Hours

Location: Magritte Office

Monday 4 July	14:00-18:00
Tuesday 5 July	07:00-18:00
Wednesday 6 July	07:30-18:00
Thursday 7 July	07:30-18:00
Friday 8 July	07:30-17:45
Saturday 9 July	08:00-15:45

Testing Centres – COVID-19

For a list of local testing centres, hours of operation and to make an appointment, please visit www.brussels.testcovid.be/en

WiFi

Network SSID: ICNMD2022
Password: Brussels

WiFi Supported by:

Disclaimer

All reasonable endeavors will be made to hold the ICNMD 2022 Congress and to present the program as scheduled under circumstances which assure the comfort and safety of the Congress Participants. However, the ICNMD and its branches, 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, Belgium in connection with the Congress. Participants are advised to consider procuring their own insurance against any such occurrences.

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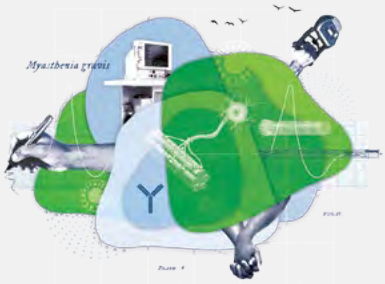

From traditional to targeted: Innovations in the changing gMG landscape

Join us for this satellite symposium as we discuss:

- The impact of gMG on patients
- Unmet needs and current and emerging treatments in gMG
- How innovations could shift therapeutic strategies towards targeted therapy

Date: 7 July 2022 | **Time:** 13:30 CEST | **Room:** Copper Hall or Online

We are delighted to introduce our panel of experts:

Prof. Jan De Bleecker, Belgium (Chair)

Prof. Benedikt Schoser, Germany

Prof. M Isabel Leite, UK

Agenda

Welcome and introduction, and patient testimonial video (10 minutes)	Prof. Jan De Bleecker **(Chair)**
Today's gMG treatment reality: Time for a change? (20 minutes)	Prof. Benedikt Schoser
The emerging treatment landscape: Hope for tomorrow (15 minutes)	Prof. M Isabel Leite
In conversation: Navigating the changing landscape in gMG (15 minutes)	All speakers

argenx welcomes you to join this interactive discussion session, where you can pose questions to the expert panel

gMG: generalized myasthenia gravis

MED-BE-EFG-2200009, April 2022

17th International Congress on Neuromuscular Diseases
Brussels, Belgium

Roche- and Sarepta-sponsored symposium

Optimising the care of people with DMD in gene therapy clinical trials: Learnings from caregivers, patient organisations and previous trial experience

Friday 8th July 2022 | 13:00–13:50 CEST

Meeting studio 211–212, SQUARE – Brussels Convention Centre

For gene therapy clinical trials in Duchenne muscular dystrophy (DMD), it is crucial that sites prepare optimally to meet both the needs of participants and the logistical challenges of the trial.

It is also important that those living with DMD and their caregivers have access to information about gene therapy and the clinical trial itself, and have sufficient support for making decisions.

How can we take advantage of prior trial experience, patient organisations and caregiver insights to support those living with DMD and their caregivers during clinical trials?

FACULTY

Prof. Dr Nicolas Deconinck
Paediatric Neurologist, Ghent University Hospital – UZ Gent, Ghent, Belgium

Dr Andrés Nascimento Osorio
Paediatric Neurologist, Hospital Sant Joan de Déu, Barcelona, Spain

Alejandra Pereda Alonso
Duchenne Parent Project, Spain

Join us and our panel of experts to hear more about this crucial topic.

We are looking forward to welcoming you to our symposium!

Lunch bags will be provided during this symposium.

This symposium is sponsored by F. Hoffmann-La Roche and developed in collaboration with Sarepta Therapeutics, Inc., and is intended for healthcare professionals only. This symposium is not intended for physicians practicing in the US.

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 17th International Congress on Neuromuscular Diseases (ICNMD). This presentation is not sponsored or endorsed by ICNMD 2022.

M-BE-00001454 | Date of preparation: May 2022

Focusing on a cure for neuromuscular and neurodegenerative disorders

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AUGUSTINE therapeutics

Save the Date

www.icnmd.org #ICNMD2024

ICNMD 2024

18TH INTERNATIONAL CONGRESS ON NEUROMUSCULAR DISEASES

25-29 October 2024 Perth, Australia

PTC Therapeutics

invites you to join the expert faculty at our symposium on

Wednesday, 6 July 2022
13:00–14:00 CEST (Silver Hall)

Visit our **booth (105)** to learn about
Duchenne muscular dystrophy

This material has been developed and funded by PTC Therapeutics for healthcare professionals only. This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 17th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2022.

GL-DMD-0624 | May 2022

PTC THERAPEUTICS™

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HELP YOUR POMPE PATIENTS STAY AHEAD OF DISEASE PROGRESSION

This year's Sanofi's symposium **"Pompe Disease - How Could We Achieve Better Outcomes for Patients"** will be live on July 7th at 13:00 CEST, in the Silver Hall.

Together, we can navigate the evolving landscape of Pompe disease.

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 17th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2022.