Onsite Program
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Dear Colleagues,

I am very pleased to invite you to the International Congress on Neuromuscular Diseases (ICNMD 2018), which will take place in Vienna, Austria, from July 6 – 10, 2018.

The ICNMD is organized on behalf of the Applied Research Group on Neuromuscular Disorders of the World Federation of Neurology. Since 2014, the Congress has taken place in two-year cycles. This will be the 15th International Congress, following upon previous meetings in Nice (2014) and Toronto (2016). Future ICNMD meetings are expected to change continually between the continents.

The aim of the Congress is to present a wide spectrum of neuromuscular diseases from the perspectives of advances in research, diagnosis and treatment. The main scientific topics will cover:

- Muscle disease
- Peripheral nerves / Neuropathies
- Neuromuscular junction disorders
- Autonomic system
- Mononeuropathies
- Cranial nerves
- Nerve regeneration
- General diseases and neuromuscular disorders
- Cancer and the peripheral nervous system
- Patient related topics
- Pain
- History
- Neuromuscular disorders worldwide
- Palliative
The format will follow the previous successful ICNMD meetings and consist of scientific sessions including plenary lectures and scientific teaching courses, workshop and poster presentations. The Congress will also offer Virtual Conferences and of course there will also be time for attendees to interact and exchange knowledge and ideas with their esteemed colleagues.

The expected aim of the Congress is that the attendees will gain an updated view on neuromuscular disorders and that the networking opportunities will increase their international experience and collaborations. The scientific and program committee have been invited from all continents around the world to enable this wide spectrum.

Vienna is an excellent and highly successful Congress city in Europe, which is easily accessible and provides a rich structure of culture and ambience serving to foster informal communication.

The Local Organizing Committee along with the Austrian Society of Neurology and the Ludwig Boltzmann Institute are delighted to host the ICNMD 2018 and look forward to meeting you in Vienna.

Professor Dr. Wolfgang Grisold

ICNMD 2018 Congress President
Ludwig Boltzmann Institute for Experimental and Clinical Traumatology
Vienna, Austria
PRESIDENT

Prof. Dr. Wolfgang Grisold,
Ludwig Boltzmann Institute for Experimental and Clinical Traumatology
Vienna, Austria

LOCAL COMMITTEE MEMBERS

Friedrich Zimprich, Medical University of Vienna, Austria
Wolfgang Löscher, Medical University of Innsbruck, Austria
Heinz Redl, Ludwig Boltzmann Institute for Experimental and Clinical Traumatology, Vienna, Austria

INTERNATIONAL SCIENTIFIC COMMITTEE MEMBERS

Vera Bril, University Health Network University of Toronto, Canada
John England, LSUHSC School of Medicine, USA
Marianne de Visser, Academic Medical Center, The Netherlands
Eva L. Feldman, University of Michigan, USA
Bruno Giometto, Università della Padova, Italy
Riad Gouider, Razi Hospital, Tunisia
Ryuji Kaji, Tokushima University Graduate School of Medicine, Japan
Satish V. Khadilkar, Bombay Hospital and Medical Research Centre, India
Matthew Kiernan, University of New South Wales, Australia
Jean-Marc Léger, University Hospital Pitié-Salpêtrière, France
Brylev Lev, Moscow City Hospital, Russia
Albert C. Ludolph, Universitätshospital Ulm, Germany
Carlos Navarrete Maldonado, Clínica Davila, Chile
Davide Pareyson, Istituto Neurologico Carlo Besta, Italy
Mary Reilly, University College London, UK
Andreas Steck, University of Basel, Switzerland
Jung-Joon Sung, Seoul National University Hospital, Korea

INTERNATIONAL PROGRAM COMMITTEE MEMBERS

Juan Jesús Vilchez Padilla, Medical Research Institute Hospital La Fe, Spain
Chongbo Zhao, Huashan Hospital, Fudan University, China

INTERNATIONAL PROGRAM COMMITTEE MEMBERS

Anthony A. Amato, Brigham and Women’s Hospital, Dep. Of Neurology, United States
Corrado Angelini, Foundation San Camillo Hospital IRCCS, Italy
Zohar Argov, Hadassah University Hospital, Israel
Michaela Auer Grumbach, Medical University of Vienna, Austria
Richard Barohn, University of Kansas Medical Center, United States
David Bennett, University of Oxford/ Nuffield Dep. Of Clinical Neurosciences, United Kingdom
Günther Bernert, Sozialmedizinisches Zentrum Süd – Kaiser-Franz-Josef-Spital mit Gottfried von Preyer’schem Kinderhospital, Austria
Nazha Birouk, Neurophysiology Unit, University Hospital Ibn Sina, Rabat, Morocco
Saeed A. Bohlega, Saudi Neurology Society, Saudi Arabia
Chiara Brian, University of Padova, Dep. Of Neurosciences, Italy
Vera Bril, University of Toronto, Canada
Kristl Claeyts, ZU Leuven, Department of Neurology, Belgium
Claude Desnuelle, Centre Hospitalier Universitaire de Nice, France
Marianne de Visser, Department of Neurology, Academic Medical Centre, The Netherlands
James Dowling, Hospital for Sick Children (SickKids), Canada
P. James B. Dyck, Mayo Clinic, United States
Eva L. Feldman, University of Michigan, United States
Davis Fursdon, NHS Oxford, UK
Nils E. Gilhus, University of Bergen, Norway
Bruno Giometto, Universita die Padova, Italy
Hans Hilmar Goebel, Universitätsmedizin Mainz, Germany
Riadh Gouider, Razi Hospital, Tunisia
Wolfgang Grisold, Ludwig Boltzmann Institute for Experimental und Clinical Traumatology, Austria
Thomas Hausner, Lorenz Böhler Hospital, Austria
Max-Josef Hilz, Universitätsklinikum Erlangen, Germany
Monika Hofer, Neuropathology and Ocular Pathology, Oxford University Hospital, United Kingdom
Romana Höftberger, Medical University of Vienna, Institute of Neurology, Austria
Sonja Horn, University of Vienna, Austria
Isabel Illa, Hospital de la Santa Creu i Sant Pau, Neurological Diseases Unit, Spain
Ryujii Kaji, Tokushima University Graduate School of Medicine, Dep. Of Neurology, Japan
Matthew Kiernan, University of New South Wales, Australia
Giuseppe Lauria, IRCCS Foundation “Carlo Besta” Neurological Institute, Italy
Steven Lewis, Rush University Medical Center, USA
Hanns Lochmüller, Institute of Genetic Medicine, Newcastle University, International Centre for Life, United Kingdom
Albert. C. Ludolph, Universitätsklinikum Ulm, Germany
Andrew Mammen, NIAMS/NIH, United States
Michelle L. Maurer, Mayo Clinic, United States
Justin McArthur, The Johns Hopkins Hospital, Dep. Of Neurology, United States
Stefan Meng, University of Vienna, Center for Anatomy and Cell Biology, Austria
Eduardo Nobile Orazio, Humanita Research Hospital, Neurology Unit, Italy
David Oliver, University of Kent, United Kingdom
George W. Padberg, Radboud University Medical Center, Dep. Of Neurology, The Netherlands
Davide Pareyson, Istituto Neurologico Carlo Besta, Italy
Tatjana Paternostro-Sluga, Donauspital Wien, Sozialmedisches Zentrum Ost, Austria
Amanda C. Peltier, Vanderbilt University Medical Center, Dep. Of Neurology, United States
Alan Pestronk, Washington University School of Medicine, Neuromuscular Clinic, United States
Luis Querol Gutierrez, Hospital de la Santa Creu i Sant Pau, Neuromuscular Diseases Unit, Spain
Mary Reilly, University College London, United Kingdom
James Russell, University of Maryland School of Medicine, United States
Gordon Smith, University of Utah, United States
Claudia Sommer, Universitätshospital Würzburg, Neurologische Klinik und Poliklinik, Germany
Nathan Staff, Mayo Clinic, United States
Volker Straub, Institute of Human Genetics Newcastle, University of Newcastle upon Tyne, International Centre for Life, United Kingdom
Walter Struhal, Kepler Universitätshospital Linz, Abt. Neurologie 2, Austria
Antonio Toscano, University of Messina, Italy
Pieter van Doorn, Erasmus MC, University Medical Center Rotterdam, Center for Lysosomal and Metabolic Diseases, The Netherlands
Jan Verschuuren, Leiden University Medical Center, Dep. Of Neurology, The Netherlands
Angela Vincent, University of Oxford, Nuffield Dep. Of Clinical Neuroscience, United Kingdom
Hugh Willison, University of Glasgow, Institute of Infection, Immunity and Inflammation, United Kingdom
Anthony J. Windebank, Mayo Clinic, United States
Gil I. Wolfe, University of Buffalo, SUNY, United States
Chongbo Zhao, Huashan Hospital, Fudan University, China
PROGRAM AT A GLANCE

FRIDAY
July 6, 2018

10:00
TEACHING COURSES
1 - 5
08:00 - 09:50

12:00
NETWORKING BREAK
12:00 - 13:00

15:00
TEACHING COURSES
6 - 9
13:00 - 14:50


SATURDAY
July 7, 2018

08:00
PLENARY SESSION
MUSCLE
08:00 - 10:00

10:00
NETWORKING BREAK
10:00 - 10:45

15:00
LUNCH / INDUSTRY-SUPPORTED SEMINARS
12:15 - 13:45

Workshops
13:45 - 15:15

Scientific Sessions
13:45 - 15:15

Overarching Session
13:45 - 15:15

Guided Poster Session
17:15 - 18:30

OPENING CEREMONY
18:30 - 19:00

OPENING NETWORKING RECEPTION
19:00 - 21:00

EFAS SCHOOL
13:00 - 14:50

Offsite Teaching Course: Ultrasound of Nerves and Muscles
Hands-on Ultrasound Course on Volunteers and Anatomical Specimens
08:00 - 17:00

Networking Break
09:50 - 10:10

Networking Break
14:50 - 15:10

Networking Break
15:15 - 15:45

Networking Break
15:15 - 15:45
PROGRAM AT A GLANCE

07:00 - 08:00
INDUSTRY-SUPPORTED SEMINARS

08:00 - 10:00
PLENARY SESSION
NEUROPATHY (Joint ICNMD-PNS)

10:00 - 10:45
NETWORKING BREAK

10:45 - 12:15
SCIENTIFIC SESSIONS

12:15 - 13:45
LUNCH / INDUSTRY-SUPPORTED SEMINARS

13:45 - 15:15
WORKSHOPS

15:15 - 15:45
NETWORKING BREAK

15:45 - 17:15
SCIENTIFIC SESSIONS

17:15 - 18:30
GUIDED POSTER SESSION

SUNDAY
JULY 8, 2018

07:00 - 08:00
INDUSTRY-SUPPORTED SEMINARS

08:00 - 10:00
PLENARY SESSION
MOTOR NEURODISEASE

10:00 - 10:45
NETWORKING BREAK

10:45 - 12:15
SCIENTIFIC SESSIONS

12:15 - 13:45
LUNCH / INDUSTRY-SUPPORTED SEMINARS

13:45 - 15:15
WORKSHOPS

15:15 - 15:45
NETWORKING BREAK

15:45 - 17:15
SCIENTIFIC SESSIONS

17:15 - 18:30
GUIDED POSTER SESSION

MONDAY
JULY 9, 2018

07:00 - 08:00
INDUSTRY-SUPPORTED SEMINARS

08:00 - 10:00
PLENARY SESSION
NEUROMUSCULAR JUNCTION

10:00 - 10:45
NETWORKING BREAK

10:45 - 12:15
SCIENTIFIC SESSIONS

12:15 - 13:45
LUNCH / INDUSTRY-SUPPORTED SEMINARS

13:45 - 15:15
WORKSHOPS

15:15 - 15:45
NETWORKING BREAK

15:45 - 17:15
SCIENTIFIC SESSIONS

17:15 - 18:30
GUIDED POSTER SESSION

TUESDAY
JULY 10, 2018

07:00 - 08:00
INDUSTRY-SUPPORTED SEMINARS

08:00 - 10:00
PLENARY SESSION
NEUROMUSCULAR JUNCTION

10:00 - 10:45
NETWORKING BREAK

10:45 - 12:15
SCIENTIFIC SESSIONS

12:15 - 13:45
LUNCH / INDUSTRY-SUPPORTED SEMINARS

13:45 - 15:15
WORKSHOPS

15:15 - 15:45
NETWORKING BREAK

15:45 - 17:15
SCIENTIFIC SESSIONS

17:15 - 18:30
GUIDED POSTER SESSION

ENMC
SYMPOSIUM
15:45 - 17:15

CLOSING CEREMONY
17:15 - 18:15

15th International Congress on Neuromuscular Diseases  WWW.ICNMD2018.ORG
PLENARY SESSIONS

SATURDAY, JULY 7, 2018

08:00 - 10:00  PLENARY SESSION PL 1.0  MUSCLE
ROOM: Park Congress 1 - 2


08:00  PL 1.1: PATHOMECHANISMS
  - Volker Straub, GB

08:40  PL 1.2: INFLAMMATORY MECHANISMS
  - Andrew Mammen, US

09:20  PL 1.3: GENETICS / EPIGENETICS
  - Hanns Lochmüller, DE

SUNDAY, JULY 8, 2018

08:00 - 10:00  PLENARY SESSION PL 2.0  NEUROPATHY
ROOM: Park Congress 1 - 2

- CHAIRS: Eva Feldman, USA & Mary Reilly, GB

08:00  PL 2.1: INHERITED NEUROPATHIES: FROM GENES TO CLINICAL PHENOTYPE
  - Mary Reilly, GB

08:40  PL 2.2: IMMUNE MEDIATED NEUROPATHIES: AN EXPANDING FIELD OF TREATABLE NEUROPATHIES
  - Pieter Van Doorn, NL

09:20  PL 2.3: ADVANCES IN THE TREATMENT OF PERIPHERAL NEUROPATHY
  - David Cornblath, US

MONDAY, JULY 9, 2018

08:00 - 10:00  PLENARY SESSION PL 3.0  MOTOR NEURONE DISEASE
ROOM: Park Congress 1 - 2

- CHAIRS: Vera Bril, CA & Claude Desnuelle, FR

08:00  PL 3.1: EMERGING THERAPIES FOR MOTOR NEURONE DISEASES
  - Peter Andersen, SE

08:40  PL 3.2: CLINICAL CONCEPT OF ALS
  - Leonard Van Den Berg, NL

09:20  PL 3.3: ETHICAL ISSUES
  - David Oliver, GB

TUESDAY, JULY 10, 2018

08:00 - 10:00  PLENARY SESSION PL 4.0  NEUROMUSCULAR JUNCTION
ROOM: Park Congress 1 - 2

- CHAIRS: Julia Wanschitz, AT & Juan Jesús Vilchez Padilla, ES

08:00  PL 4.1: PHYSIOLOGY AND STRUCTURE
  - Steve Burden, US

08:40  PL 4.2: CONGENITAL MYASTHENIC SYNDROMES - NEW GENES AND BETTER TREATMENTS / ANTIBODIES
  - Kinji Ohno, JP

09:20  PL 4.3: AUTOIMMUNITY
  - Angela Vincent, GB
SPEAKER INFORMATION

SPEAKER READY ROOM

Schnitzler Room on the Mezzanine Floor is the designated Speaker Ready Room.

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<td>SATURDAY, JULY 7</td>
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<td>SUNDAY, JULY 8</td>
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<td>MONDAY, JULY 9</td>
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<td>TUESDAY, JULY 10</td>
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Important Information for Speakers

If you do not submit your presentation in advance, you are asked to bring it to the Speaker Ready Room to ensure the quality of your presentation(s) including fonts, bullets, outlines, animations, etc.

All Presenters (Invited Speakers and Free Communication Session Presenters) are required to submit and/or preview their slides at least 24 hours prior to their scheduled presentation to ensure compatibility with the Congress AV Equipment. Priority will be given to speakers who are presenting the next day.

Technical staff at the Speaker Ready Room will be available to assist with any audio-visual needs you may have in order to finalise your presentation. A technical specialist will upload your presentation to a server. Please do not bring your own laptop or attempt to upload your presentations in your presentation room.

The organisers cannot guarantee projection of presentation handed in later than 24 hours prior to the scheduled session.
# PRE-CONGRESS TEACHING COURSES ➤ FRIDAY JULY 6, 2018

**LOCATION**
All Teaching Course Sessions are located at the Hilton Vienna Am Stadtpark, Austria except TC 11.0 – ’Ultrasound of nerves and muscles: Hands-on ultrasound course on volunteers and anatomical specimens’ which is located at Center for Anatomy and Cell Biology, Medical University of Vienna, Austria (Off-site)

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<th>Session</th>
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<th>Chairs</th>
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<tr>
<td>08:00</td>
<td>TC 1.0 - HyperCKemia</td>
<td>Park Congress 1</td>
<td>Corrado Angelini, IT &amp; Marianne De Visser, NL</td>
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<tr>
<td>08:55</td>
<td>TC 1.1: CLINICAL PHENOTYPES AND HOW TO MANAGE PATIENTS WITH HIGH HYPERCKEMIA, STATIN MYOPATHY</td>
<td></td>
<td>Marianne De Visser, NL</td>
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<tr>
<td>09:50</td>
<td>TC 1.2: HOW TO DIAGNOSE A PATIENT WITH HYPERCKEMIA</td>
<td></td>
<td>Sabrina Sacconi, FR</td>
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<td>10:00</td>
<td>TC 1.3: HYPERCKEMIA IN MUSCULAR DISTROPHIES AND METABOLIC MYOPATHIES</td>
<td></td>
<td>Corrado Angelini, IT</td>
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<tr>
<td>10:55</td>
<td>TC 1.4: DIAGNOSTIC APPROACH AND GUIDELINES FOR OLIGOSYMPTOMATIC HYPERCKEMIA</td>
<td></td>
<td>Theodore Kyriakides, CY</td>
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<td>09:50</td>
<td>NETWORKING BREAK</td>
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<td>10:10</td>
<td>TC 2.1: INTRODUCTION TO STRUCTURE &amp; FUNCTION, MECHANISMS OF NMJ DEVELOPMENT AND EFFECTS OF AUTOANTIBODIES</td>
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<td>William Phillips, AU</td>
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<tr>
<td>11:05</td>
<td>TC 2.2: NEUROPHYSIOLOGY</td>
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<td>Clarke Slater, GB</td>
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<td>08:00</td>
<td>TC 2.0 - ’Basic Course’: Neuromuscular structure, transmission and the diseases that affect it</td>
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<td>08:55</td>
<td>RFC 2.3: PRESYNAPTIC RECEPTORS AND NEUROMUSCULAR TRANSMISSION</td>
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<td>Ana Sebastião, PT</td>
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<td>11:05</td>
<td>TC 2.4: DISEASES INVOLVING IMPAIRMENT AND LOSS OF NEUROMUSCULAR CONNECTION</td>
<td></td>
<td>Hanns Lochmüller, DE</td>
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<td>08:00</td>
<td>TC 2.5: ESSENTIAL CLUES TO EARLY RECOGNITION OF AMYLOID NEUROPATHIES</td>
<td></td>
<td>Davide Pareyson, IT</td>
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<td>09:50</td>
<td>NETWORKING BREAK</td>
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<td>10:10</td>
<td>TC 3.1: CMT NEUROPATHIES IN THE ELDERLY</td>
<td></td>
<td>Michaela Auer-Grumbach, AT &amp; Davide Pareyson, IT</td>
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<td>08:55</td>
<td>TC 3.2: ESSENTIAL CLUES TO EARLY RECOGNITION OF AMYLOID NEUROPATHIES</td>
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<td>Davide Pareyson, IT</td>
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<td>09:50</td>
<td>NETWORKING BREAK</td>
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<td>10:10</td>
<td>TC 3.3: SENSORY AND AUTONOMIC HEREDITARY NEUROPATHIES</td>
<td></td>
<td>Max Hilz, DE</td>
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<tr>
<td>11:05–12:00</td>
<td>TC 3.4: NEUROPATHIC MANIFESTATIONS IN PORPHYRIA</td>
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<td>P. James Dyck, US</td>
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<tr>
<td>08:00</td>
<td>TC 3.5: NEUROPATHIC MANIFESTATIONS IN PORPHYRIA</td>
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<td>08:00</td>
<td>TC 4.0 - Floppy infant syndrome: clinical assessment and decisions</td>
<td>Klimt 1</td>
<td>Guenther Bernert, AT &amp; James Dowling, CA</td>
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<tr>
<td>11:05–12:00</td>
<td>TC 4.1: EPIDEMIOLOGY AND DIFFERENTIAL DIAGNOSIS</td>
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<tr>
<td>Time</td>
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<td>08:55</td>
<td>TC 4.2: CLINICAL ASSESSMENT AND MUSCLE IMAGING: TECHNIQUES AND PITFALLS</td>
<td>Eugenio Mercuri, IT</td>
<td>Berg</td>
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<tr>
<td>09:50</td>
<td>NETWORKING BREAK</td>
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<td>10:10</td>
<td>TC 4.3: FLOPPY INFANT SYNDROME AND THE ROLE OF “NEXT GEN”</td>
<td>James Dowling, CA</td>
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<td>11:05</td>
<td>TC 4.4: WHAT YOU MAY MISS WITHOUT EMG</td>
<td>Matthew Pitt, GB</td>
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<td>08:00</td>
<td>TC 5.1: ENTRAPMENT SYNDROMES IN THE UPPER EXTREMITY – ELECTROPHYSIOLOGY</td>
<td>Christian Bischoff, DE</td>
<td>Berg</td>
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<tr>
<td>08:55</td>
<td>TC 5.2: ENTRAPMENT SYNDROMES IN THE UPPER EXTREMITY – SONOGRAPHY</td>
<td>Nens Van Alfen, NL</td>
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<td>09:50</td>
<td>NETWORKING BREAK</td>
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<td>10:10</td>
<td>TC 5.3: ENTRAPMENT SYNDROMES IN THE LOWER EXTREMITY – ELECTROPHYSIOLOGY</td>
<td>Simon Podnar, SI</td>
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<tr>
<td>11:05</td>
<td>TC 5.4: ENTRAPMENT SYNDROMES IN THE LOWER EXTREMITY</td>
<td>Alexander Grimm, DE</td>
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<td>12:00</td>
<td>NETWORKING BREAK</td>
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<td>13:00</td>
<td>TC 6.0 - Clinical: from pattern recognition towards clinical diagnosis</td>
<td>Richard Barohn, US</td>
<td>Berg</td>
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<td>Mazen Dimachkie, US</td>
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<td>15:10</td>
<td>TC 6.3: GENETIC APPROACH TO MUSCLE DISORDERS</td>
<td>Tahseen Mozaffar, US</td>
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<tr>
<td>16:05</td>
<td>TC 6.4: APPROACH TO TRANSLATIONAL RESEARCH AND CLINICAL TRIALS IN MUSCLE DISEASE</td>
<td>Michael Hanna, GB</td>
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<tr>
<td>13:00</td>
<td>TC 7.0 - Diagnostics and treatment of ALS and MND</td>
<td></td>
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<td>15:10</td>
<td>TC 7.3: BREAKING THE NEWS OF THE DIAGNOSIS</td>
<td>Dorothée Lulé, DE</td>
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<tr>
<td>16:05</td>
<td>TC 7.4: THE ROLE OF MULTIDISCIPLINARY CARE IN ALS</td>
<td>Christopher McDermott, US</td>
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<td>15:10</td>
<td>TC 7.1: HOW DO WE DISCUSS THE GENETIC IMPLICATIONS WITH ALS</td>
<td>Ryuji Kaji, JP</td>
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<td>14:50</td>
<td>NETWORKING BREAK</td>
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<td>15:10</td>
<td>TC 8.3: OUTCOME MEASURES IN NEUROPATHY</td>
<td>Ingemar Merkies, NL</td>
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<tr>
<td>16:05</td>
<td>TC 8.4: ROLE OF ELECTROPHYSIOLOGY IN THE CHARACTERIZATION AND</td>
<td>Peter Van den Bergh, BE</td>
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<tr>
<td></td>
<td>DIAGNOSIS OF NEUROPATHIES: NEW INSIGHTS</td>
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<tr>
<td>13:00</td>
<td>TC 8.1: MRI AND US IN THE DIAGNOSIS OF NEUROPATHY</td>
<td>Stephan Goedee, NL</td>
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<tr>
<td>13:55</td>
<td>TC 8.2: SKIN BIOPSY: NOT JUST FOR DIAGNOSAL</td>
<td>Grazia Devigili, IT</td>
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<td>14:50</td>
<td>NETWORKING BREAK</td>
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<tr>
<td>15:10</td>
<td>TC 8.5: UNDERSTANDING THE DRUG DEVELOPMENT PROCESS FOR RARE NEUROMUSCULAR DISEASES</td>
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<td>16:05</td>
<td>TC 8.6: OUTCOME MEASURES IN NEUROPATHY</td>
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<td>13:00</td>
<td>TC 9.0 - UNDERSTANDING THE DRUG DEVELOPMENT PROCESS FOR RARE NEUROMUSCULAR DISEASES</td>
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<tr>
<td>13:00</td>
<td>TC 9.1: REQUIREMENTS AND PITFALLS FOR SMALL MOLECULES</td>
<td>Mike Kelly, US</td>
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<td>14:50</td>
<td>NETWORKING BREAK</td>
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**Notes:**
- 8:00 - 12:00 TC 5.0 - Electrodiagnostic and sonographic evaluation of entrapment mononeuropathies (Hands-On)
- Machine in-kind donation by CADWELL
- Note: There is a variety of different similar products that can be used beyond the ones showcased in this session.
- Chairs: Wolfgang Löscher, AT & Nens Van Alfen, NL
- 12:00–13:00 NETWORKING BREAK
- 13:00–17:00 TC 7.0 - Diagnostics and treatment of ALS and MND
- Chairs: Albert Ludolph, DE & Dorothée Lulé, DE
- 13:00–17:00 TC 8.0 - New way to diagnose and assess neuropathies
- 13:00–17:00 TC 9.0 - Understanding the drug development process for rare neuromuscular diseases
- Chairs: Volker Straub, GB & Kanneboyina Nagaraju, US & Raffaella Willmann, CH
<table>
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<tr>
<th>Time</th>
<th>Session</th>
<th>Topic</th>
<th>Speaker(s)</th>
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<tr>
<td>13:20</td>
<td>TC 9.2</td>
<td>REQUIREMENTS AND PITFALLS FOR GENE THERAPY</td>
<td>Isabelle Richard, FR</td>
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<td>13:30</td>
<td>TC 9.3</td>
<td>REQUIREMENTS AND PITFALLS FOR STEM CELLS</td>
<td>Miranda Grounds, AU</td>
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<tr>
<td>13:40</td>
<td>TC 9.4</td>
<td>PRECLINICAL TRIAL DESIGN</td>
<td>Annamaria De Luca, IT</td>
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<td>TC 9.5</td>
<td>STATISTICAL POWER EVALUATION</td>
<td>Heather Gordish-Dressman, US</td>
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<td></td>
<td>TC 9.6</td>
<td>DATA INTERPRETATION, TRANSLATIONAL MEANING</td>
<td>Annamaria De Luca, IT</td>
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<td>13:40</td>
<td>TC 9.7</td>
<td>REGULATORY REQUIREMENTS</td>
<td>Didier Caizergues, FR</td>
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<td>13:50</td>
<td>TC 9.8</td>
<td>CLINICAL TRIAL READINESS</td>
<td>Kathryn Wagner, US</td>
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<td>14:00</td>
<td>TC 9.9</td>
<td>CARE ASPECTS IN CLINICAL TRIAL PLANNING</td>
<td>Anna Mayhew, GB</td>
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<td>14:10</td>
<td>TC 9.10</td>
<td>PATIENT PERSPECTIVE</td>
<td>Elizabeth Vroom, NL</td>
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<td>14:50</td>
<td>TC 9.11</td>
<td>BUSINESS MODEL</td>
<td>Cristina Csimma, US</td>
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<td>14:50</td>
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<td>NETWORKING BREAK</td>
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<td>15:00</td>
<td>TC 9.12</td>
<td>PLENARY SESSION: THE TACT PROCEDURE BY MEANS OF A MOCK TACT APPLICATION</td>
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<td>15:30</td>
<td>TC 10.1</td>
<td>ANS: THE INTERFACE BETWEEN OUTER AND INNER WORLD</td>
<td>Isabel Rocha, PT</td>
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<tr>
<td>15:50</td>
<td>TC 10.2</td>
<td>AUTONOMIC DISORDERS: WHAT GOES WRONG</td>
<td>Heinz Lahrmann, AT</td>
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<tr>
<td>16:10</td>
<td>TC 10.3</td>
<td>CARDIOVASCULAR AUTONOMIC FUNCTION TESTS AND ABPM</td>
<td>Giovanni Calandra-Buonaura, IT</td>
</tr>
<tr>
<td>16:30</td>
<td>TC 10.4</td>
<td>NEUROENDOCRINE AND NUCLEAR AUTONOMIC FUNCTION TESTS</td>
<td>Camilla Rocchi, IT</td>
</tr>
<tr>
<td>16:50</td>
<td>TC 10.5</td>
<td>PRACTICAL SESSION: HOW TO PERFORM A CARDIOVASCULAR AUTONOMIC FUNCTION TEST, PART I</td>
<td>Walter Struhal, AT</td>
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<td>15:00</td>
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<td>NETWORKING BREAK</td>
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<tr>
<td>15:10</td>
<td>TC 10.6</td>
<td>AUTONOMIC HISTORY TAKING: YOU NEED TO KNOW WHAT YOU’RE LOOKING FOR</td>
<td>Roland Thijs, NL</td>
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<tr>
<td>15:30</td>
<td>TC 10.7</td>
<td>SYCNOPE, PSEUDOSYNCOPE AND OTHER ORTHOSTATIC INTOLERANCE SYNDROMES</td>
<td>Anne Pavy-Le Traon, FR</td>
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<tr>
<td>15:50</td>
<td>TC 10.8</td>
<td>AUTONOMIC SYNDROMES: HOW TO PUT THE PIECES OF THE JIGSAW TOGETHER</td>
<td>Eva Lenzenweger, AT</td>
</tr>
<tr>
<td>16:10</td>
<td>TC 10.9</td>
<td>SYCNOPE AND PSEUDOSYNCOPE</td>
<td>J. Gert Van Dijk, NL</td>
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<td>16:30</td>
<td>TC 10.10</td>
<td>EBM TREATMENT OF ORTHOSTATIC HYPOTENSION AND RELATED SYMPTOMS</td>
<td>Gregor K. Wenning, AT</td>
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<tr>
<td>16:50</td>
<td>TC 10.11</td>
<td>EVALUATION FORMS AND CONCLUDING REMARKS</td>
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**Session 3: Clinical trial design**

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<tbody>
<tr>
<td>13:00</td>
<td>TC 10.0</td>
<td>EFAS School: Bed side management and testing for clinical autonomic disorders</td>
<td>Walter Struhal, AT &amp; Anne Pavy-Le Traon, FR</td>
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<tr>
<td>13:10</td>
<td>TC 10.1</td>
<td>ANS: THE INTERFACE BETWEEN OUTER AND INNER WORLD</td>
<td>Isabel Rocha, PT</td>
</tr>
<tr>
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<td>TC 10.2</td>
<td>AUTONOMIC DISORDERS: WHAT GOES WRONG</td>
<td>Heinz Lahrmann, AT</td>
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**Session 2: Preclinical efficacy data**

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<td>Camilla Rocchi, IT</td>
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<tr>
<td>13:40</td>
<td>TC 10.5</td>
<td>PRACTICAL SESSION: HOW TO PERFORM A CARDIOVASCULAR AUTONOMIC FUNCTION TEST, PART I</td>
<td>Walter Struhal, AT</td>
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**Session 2: Preclinical efficacy data**

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<tr>
<td>08:00</td>
<td>Welcome and Basic Anatomy and Intervention Tactics</td>
<td>Stefan Meng, AT &amp; Daniel Truong, US</td>
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<tr>
<td>08:30</td>
<td>Nerve Part 1.1: Hands-on Ultrasound Assessment of Local, Perineural Anatomy</td>
<td>Stefan Meng, AT</td>
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<tr>
<td>09:45</td>
<td>Nerve Part 1.2: Ultrasound Guided Injections in Anatomic Specimen</td>
<td>Stefan Meng, AT</td>
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<tr>
<td>11:00</td>
<td>Networking Break</td>
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<tr>
<td>11:30</td>
<td>Muscle: Practical Muscle Botulinum Toxin Injection Course</td>
<td>Daniel Truong, US</td>
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<td>14:30</td>
<td>Networking Break</td>
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<tr>
<td>16:30</td>
<td>Nerve Part 2.2: New Nerve Ultrasound Injections in Anatomic Specimen</td>
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<td>13:00–17:00</td>
<td>Public Patient Day</td>
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<tr>
<td>13:10 - 13:30</td>
<td>AUS DER SICHT DES PATIENTEN: ERKRANKUNGEN DES NEUROMUSKULAREN ÜBERGANGES (MYASTHENIA GRAVIS)</td>
<td>Evelyn Suritsch, AT</td>
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<td>13:30 - 13:50</td>
<td>Muskelerkrankungen bei Kindern, Neue Therapien</td>
<td>Guenther Bernert, AT</td>
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<td>13:50 - 14:10</td>
<td>Muskelerkrankungen bei Erwachsenen</td>
<td>Julia Wanschitz, AT</td>
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<td>14:10 - 14:30</td>
<td>Polyneuropathien</td>
<td>Wolfgang Löscher, AT</td>
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<td>15:00 - 15:20</td>
<td>Polyneuropathien - Fokus Diabetes Mellitus</td>
<td>Eva Feldman, USA</td>
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<td>15:20 - 15:40</td>
<td>“Die Eingeschlafene Hand”</td>
<td>Elisabeth Lindeck-Pozza, AT</td>
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<td>15:40 - 16:00</td>
<td>Erkrankungen des Neuromuskularen Überganges (Myasthenia Gravis)</td>
<td>Rafii Topakian, AT</td>
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<td>16:00 - 16:20</td>
<td>Amyotrope Lateral Sklerose (ALS)</td>
<td>Heinz Lahrmann, AT</td>
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<td>16:20-16:40</td>
<td>Erkrankungen des Autonomen Nervensystems</td>
<td>Walter Struhal, AUT</td>
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<td>18:30 - 21:00</td>
<td>Opening Ceremony</td>
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*13th International Congress on Neuromuscular Diseases*
CONGRESS PROGRAM ▶ SATURDAY JULY 7, 2018

07:00 - 18:30 EFAS ANNUAL MEETING 2018
ROOM ▶ Park Congress 3

Plenary Session II Peripheral autonomic neuropathies
08:00 PL 1: DIABETIC AUTONOMIC NEUROPATHY
Roy Freeman, US
08:30 PL 2: AUTONOMIC DYSFUNCTION IN GBS
Max Hilz, DE
08:50 PL 3: ANS AND AMYLOID
Isabel Rocha, PT

Scientific Session I
09:10 SS 1: AUTOIMMUNE AUTONOMIC GANGLIONOPATHY: THE NATIONAL HOSPITAL FOR NEUROLOGY AND NEUROSURGERY, QUEEN SQUARE EXPERIENCE
Shiwen Koay, GB
09:20 SS 2: MICRONEUROGRAPHIC EVIDENCE AGAINST BAROREFLEX BLUNTING WITH PERIPHERAL CHEMOREFLEX ACTIVATION IN HUMANS
Karsten Heusser, DE
09:30 SS 3: THE NATURAL HISTORY OF A COHORT OF ISOLATED AUTONOMIC FAILURE PATIENTS
Pietro Guaraldi, IT
09:35 SS 4: ORTHOSTATIC HYPERTENSION: A RARE KNOWN DYSAUTONOMIA
Alvaro Petersen, MX
09:40 SS 5: HOW TO OVERCOME POST-OPERATIVE ILEUS IN PERITONITS PATIENTS
Peter Lechner, AT
09:50 NETWORKING BREAK

Scientific Session II:
12:00 SS 6: AUTONOMIC DYSFUNCTION IN THE INITIAL MOTOR STAGE OF PARKINSON’S DISEASE: A 3-YEAR LONGITUDINAL STUDY
Iva Stankovic, RS
12:05 SS 7: ACQUIRED IDIOPATHIC GENERALIZED ANHIDROSIS IN A YOUNG AUSTRIAN PATIENT
Gerald Exler, AT

Plenary Session III) TLOC, Brain injury and ANS sequelae
12:10 NETWORKING BREAK
12:55 GUIDED POSTER WALK

Scientific Session II:
13:45 PL 8: DIFFERENTIAL DIAGNOSIS
J. Gert Van Dijk, NL
14:05 PL 9: AUTONOMIC DYSFUNCTION IN EPILEPSY
Roland Thijs, NL
14:25 PL 10: STROKE AND ANS
Max Hilz, DE
14:45 PL 11: SYNCOPEEDIA: TRAINING A NEW GENERATION OF SYNOPE SPECIALISTS
Jelle De Jong, NL

Scientific Session III:
15:05 SS 8: MUTUALLY EXCLUSIVE CRITERIA FOR NEUROGENIC ORTHOSTATIC HYPOTENSION AND VASOVAGAL SYNCOPE DURING TILT-TABLE TESTING
Maryam Ghariq, NL
15:10 SS 9: HEART RATE VARIABILITY IS ASSOCIATED WITH OUTCOME IN SPONTANEOUS INTRACEREBRAL HEMORRHAGE
Marek Sykora, AT

15:15 NETWORKING BREAK

Plenary Session III) Neurodegeneration & ANS
10:40 PL 4: PAF-MSA-PARKINSON HOW TO DIFFERENTIATE, HOW TO CONFUSE, SIMILARITIES AND SPECIFICS
Gregor K. Wenning, AT
11:00 PL 5: LINKS FROM PERIPHERY TO THE BRAIN
Alessandra Fanciulli, AT
11:20 PL 6: SLEEP DISORDERS & ANS
Pietro Guaraldi, IT
11:40 PL 7: AUTONOMIC DYSFUNCTION IN DEMENTIA
Walter Struhal, AT

Plenary Session IV) Autonomic Nervous system and Fatigue Background and Management
15:45 PL 12: AUTONOMIC NERVOUS SYSTEM IN SIMULATED AND REAL WEIGHTLESSNESS
Anne Pavy-Le Traon, FR
16:05 PL 13: PROGNOSTIC AUTONOMIC BIOMARKERS
Jean-Michel Senard, FR
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker</th>
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<tbody>
<tr>
<td>16:25</td>
<td>PL 14: FATIGUE AND AUTONOMIC NERVOUS SYSTEM DYSFUNCTION IN PEOPLE WITH MULTIPLE SCLEROSIS</td>
<td>Mario Habek, HR</td>
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<td>16:45</td>
<td>PL 15: GASTRIC DYSFUNCTION</td>
<td>Heinz Zoller, AT</td>
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<td>Scientific Session IV:</td>
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<td>17:05 SS 10: UROFLOWMETRY SHOWS SLIGHTLY IMPAIRED AUTONOMIC BLADDER FUNCTION IN PATIENTS WITH RELAPSING-REMITTING MULTIPLE SCLEROSIS</td>
<td>Ruihao Wang, DE</td>
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<td>17:15 CLOSING REMARKS AND POSTER PRIZE</td>
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<td>08:00</td>
<td>Plenary Session PL 1.0 Muscle</td>
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<td>ROOM ▶ Park Congress 1 - 2</td>
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<tr>
<td>08:00</td>
<td>PL 1.1: PATHOMECHANISMS</td>
<td>Volker Straub, GB</td>
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<td>08:40</td>
<td>PL 1.2: INFLAMMATORY MECHANISMS</td>
<td>Andrew Mammen, US</td>
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<td>09:20</td>
<td>PL 1.3: GENETICS / EPIGENETICS</td>
<td>Hanns Lochmüller, DE</td>
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<td>10:00</td>
<td>NETWORKING BREAK</td>
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<td>ROOM ▶ Bruckner - Strauss &amp; Klimt 2 - 3</td>
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<td>10:45</td>
<td>Scientific Session SS 1.0 New therapies</td>
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<td>ROOM ▶ Park Congress 1</td>
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<td>Chairs: Marianne De Visser, NL &amp; Kanneboyna Nagaraju, US</td>
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<td>10:45</td>
<td>SS 1.1: TREATMENT STRATEGIES IN GNE MYOPATHY</td>
<td>Zohar Argov, IL</td>
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<tr>
<td>11:15</td>
<td>SS 1.2: UPDATE OF RNA-BASED NMD THERAPIES</td>
<td>Kanneboyna Nagaraju, US</td>
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<tr>
<td>11:15</td>
<td>SS 1.3: PROGRESS IN THE TREATMENT OF IDIOPATHIC INFLAMMATORY MYOPATHIES?</td>
<td>Marianne De Visser, NL</td>
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<td>Scientific Session SS 2.0 Nerve Regeneration</td>
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<td>Chairs: Anthony Windebank, US &amp; Heinz Redi, AT</td>
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<td>10:45</td>
<td>SS 2.1: TISSUE ENGINEERING STRATEGIES FOR REPAIR OF PERIPHERAL NERVE</td>
<td>Anthony Windebank, US</td>
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<tr>
<td>11:15</td>
<td>SS 2.2: USE OF PHYSICAL METHODS IN NERVE REGENERATION</td>
<td>Thomas Hausner, AT</td>
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<tr>
<td>11:45</td>
<td>SS 2.3: NEW DEVELOPMENTS IN CONDUITS</td>
<td>David Hercher, AT</td>
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<td>10:45 - 12:15</td>
<td>Scientific Session SS 3.0 Paraneoplastic neuromuscular aspects</td>
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<td>Chairs: Bruno Giometto, IT &amp; Andrew Mammen, US</td>
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<td>10:45</td>
<td>SS 3.1: THE ROLE OF HU PROTEINS IN THE DEVELOPMENT OF PARANEOPLASTIC NEUROPATHIES</td>
<td>Bruno Giometto, IT</td>
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<tr>
<td>11:07</td>
<td>SS 3.2: PHYSIOPATHOLOGY OF PARANEOPLASTIC GANGLIONOPATHIES</td>
<td>Romana Hoelfberger, AT</td>
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<tr>
<td>11:29</td>
<td>SS 3.3: PARANEOPLASTIC MUSCLE DISORDERS</td>
<td>Andrew Mammen, US</td>
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<td>11:51</td>
<td>SS 3.4: Lambert-Eaton-Syndrome (LEMS)</td>
<td>Jan Verschuuren, NL</td>
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<td>12:15 - 13:45</td>
<td>LUNCH / INDUSTRY-SUPPORTED SEMINARS</td>
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<td>ROOMS ▶ PARK CONGRESS 1 / 2</td>
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<td>13:45</td>
<td>Scientific Session SS 4.0 New clinical entities</td>
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<td>Chairs: Antonio Toscano, IT &amp; Bjarne Udd, FI</td>
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<td>13:45</td>
<td>SS 4.1: UPDATE ON CLINICAL ASPECTS OF GLYCOGEN STORAGE DISORDERS</td>
<td>Antonio Toscano, IT</td>
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<tr>
<td>14:15</td>
<td>SS 4.2: NOVEL ENTITIES IN CONGENITAL MYOPATHIES OF ADULT ONSET</td>
<td>Baziel Van Engelen, NL</td>
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<tr>
<td>14:45</td>
<td>SS 4.3: NEW PHENOTYPES IN TITIN DEFECTS</td>
<td>Bjarne Udd, FI</td>
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<td>13:45 - 15:15</td>
<td>Workshops WS 1.0 Iatrogenic nerve lesions</td>
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<td>ROOM ▶ Klimt 1</td>
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<td>Chairs: Tatjana Paternostro-Sluga, AT &amp; Anthony Windebank, US</td>
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<td>13:45</td>
<td>WS 1.1: CHEMOTHERAPY INDUCED PNP</td>
<td>Anthony Windebank, US</td>
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<td>14:15</td>
<td>WS 1.2: IATROGENIC MONONEUROPATHIES AFTER SURGERY AND TRAUMA</td>
<td>Tatjana Paternostro-Sluga, AT</td>
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<td>14:45</td>
<td>WS 1.3: CASE REPORT</td>
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</tbody>
</table>
13:45 - 15:15  Scientific Session SS 5.0
Pediatric Myology
ROOM ➤ Berg
Chairs: Gunther Bernert, AT & Eugenio Mercuri, IT
13:45  SS 5.1: STANDARDS OF CARE: WHAT WE HAVE LEARNED FROM LONG TERM-MANAGEMENT IN CHILDREN WITH NMD’S
Guenther Bernert, AT
14:07  SS 5.2: FUTURE OF AAV-BASED GENE THERAPY APPROACHES FOR NMDS
Thomas Voit, GB
14:29  SS 5.3: NEW TREATMENTS IN PAEDIATRIC NMD’S: FAKE OR FACT?
Eugenio Mercuri, IT
14:51  SS 5.4: TRANSITION FROM PEDIATRIC INTO ADULT CARE: A LONG WAY TO GO?
Thomas Serjensen, SE
13:45 - 15:15  Overarching Session OA 1.0
Stem cell in neuromuscular disorders
ROOM ➤ Park Congress 2
Chairs: Zohar Argov, IL & Mayana Zatz, BR
13:45  OA 1.1: STEM CELLS AS TOOLS FOR RESEARCH AND THERAPY IN NEUROMUSCULAR DISORDERS
Rita Perlingeiro, US
14:15  OA 1.2: STEM CELLS IN MUSCULAR DYSTROPHIES
Mayana Zatz, BR
14:45  OA 1.3: STEM CELLS IN ALS
Dimitrios Karussis, IL
13:45 - 15:15 NETWORKING BREAK
ROOM ➤ Bruckner - Strauss & Klimt 2 - 3
15:15 - 17:15  Scientific Session SS 6.0
Basic principles of neuromuscular involvement in cancer
ROOM ➤ Klimt 1
Chairs: Ahmet Hoke, US & Guido Cavaletti, IT
15:45  SS 6.1: GENERAL PRINCIPLES AND CLINICAL ASPECTS OF CIPN
Nathan Staff, US
16:15  SS 6.2: BASIC MECHANISMS: AXONAL DEGENERATION AND CHEMOTHERAPY-INDUCED PERIPHERAL NEUROPATHY
Ahmet Hoke, US
16:45  SS 6.3: ANIMAL MODELS OF CHEMOTHERAPY-INDUCED PERIPHERAL NEUROPATHY
Guido Cavaletti, IT
15:45 - 17:15  Workshops WS 2.0
FSHD
ROOM ➤ Park Congress 1
Chairs: George Padberg, NL & Nicol Voermans, NL
15:45  WS 2.1: DUX4, ITS ROLE IN DEVELOPMENT AND MUSCLE DISEASE
Stephen J. Tapscott, US
16:15  WS 2.2: TRIALS READINESS IN FSHD: CLINICAL, IMAGING AND BIOMARKERS
Jeffrey M. Statland, US
16:45  WS 2.3: FSHD’S CHANGING LANDSCAPE AND FUTURE PERSPECTIVES
George Padberg, NL
15:45 - 17:15  Workshops WS 3.0
Palliative care in NMD
ROOM ➤ Berg
Chairs: David Oliver, GB & Christopher McDermott, US
15:45  WS 3.1: THE ROLE OF PALLIATIVE CARE – EARLY INVOLVEMENT OR END OF LIFE CARE?
David Oliver, GB
16:07  WS 3.2: THE ROLE OF THE MULTIDISCIPLINARY TEAM IN NMD – THE EVIDENCE FROM ALS
Christopher McDermott, US
16:29  WS 3.3: THE RECOGNITION OF THE END OF LIFE PHASE IN NMD – THE SUPPORT OF PATIENTS, FAMILIES AND PROFESSIONALS
Stefan Lorenzl, DE
16:51  WS 3.4: INTEGRATING PALLIATIVE CARE INTO SERVICE FOR ALS PATIENTS: REAL-LIFE EXPERIENCE
Lev Brylev, RU
15:45 - 17:15  Workshops WS 4.0
New diagnostic techniques/tools
ROOM ➤ Park Congress 2
Chairs: Anthony Amato, US & Jasper M. Morrow, GB
15:45  WS 4.1: UPDATE ON HISTOPATHOLOGICAL FEATURES OF INFLAMMATORY MYOPATHIES
Anthony Amato, US
16:15  WS 4.2: UPDATE ON MYOSITIS SPECIFIC AUTOANTIBODIES / DISCUSSION ON UTILITY OF MSAS IN DIAGNOSIS AND PROGNOSIS
Andrew Mammen, US
16:45  WS 4.3: UPDATE ON UTILITY OF SKELETAL MUSCLE MRI AND ULTRASOUND
Jasper M. Morrow, GB
17:15 - 18:30  POSTER SESSION PS1
GUIDED POSTER SESSION
ROOM ➤ MEZZANINE FLOOR GALLERY AND FOYERS
**CONGRESS PROGRAM ▶ SUNDAY JULY 8, 2018**

7:00 - 8:00 ▶ INDUSTRY-SUPPORTED SEMINAR  
**ROOM: BERG**

8:00 - 10:00 ▶ Plenary Session PL 2.0  
**Neuropathy**  
**ROOM: Park Congress 1 - 2**  
Chairs: Mary Reilly, GB

08:00 ▶ PL 2.1: INHERITED NEUROPATHIES: FROM GENES TO CLINICAL PHENOTYPE  
Mary Reilly, GB

08:40 ▶ PL 2.2: IMMUNE MEDIATED NEUROPATHIES: AN EXPANDING FIELD OF TREATABLE NEUROPATHIES  
Pieter Van Doorn, NL

09:20 ▶ PL 2.3: ADVANCES IN THE TREATMENT OF PERIPHERAL NEUROPATHY  
David Cornblath, US

10:00 - 10:45 ▶ NETWORKING BREAK  
**ROOM: BRUCKNER - STRAUSS & KLIMT 2 - 3**

10:45 - 12:15 ▶ Scientific Session SS 7.0  
**From bench to bedside: inherited neuropathies**  
**ROOM: Park Congress 1**  
Chairs: Vincent Timmerman, BE & Michael Sereda, DE

10:45 ▶ SS 7.1: NEW GENES AND NEW MECHANISMS IN INHERITED NEUROPATHIES  
Vincent Timmerman, BE

11:15 ▶ SS 7.2: THERAPEUTIC OPTIONS IN CMT  
Michael Sereda, DE

11:45 ▶ SS 7.3: FAMILIAL AMYLOID NEUROPATHIES: A TREATABLE GENETIC NEUROPATHY  
Davide Pareyson, IT

12:15 - 13:45 ▶ LUNCH / INDUSTRY-SUPPORTED SEMINARS  
**ROOM: PARK CONGRESS 1 / 2**

13:45 - 15:15 ▶ Scientific Session SS 10.0  
**Diabetic neuropathies**  
**ROOM: Park Congress 3**  
Chairs: Eva Feldman, US & Vera Bril, CA

13:45 ▶ SS 10.1: 2017 ADA POSITION STATEMENT ON DIABETIC NEUROPATHY: DOES IT CHANGE YOUR PRACTICE?  
Vera Bril, CA

14:15 ▶ SS 10.2: SCIENTIFIC DISCOVERY LEADS TO CLINICAL TRIALS IN DIABETIC NEUROPATHY  
James Russell, US

14:45 ▶ SS 10.3: PAINFUL DIABETIC NEUROPATHY: A MANAGEMENT CENTERED APPROACH  
Eva Feldman, US
13:45 - 15:15 Workshops WS 5.0
Advances in the treatment of immune medi rated neuropathies
ROOM • Park Congress 1
Chairs: Eduardo Nobile-Orazio, IT & Jean-Marc Léger, FR
13:45 WS 5.1: DIAGNOSIS AND TREATMENT OF CIDP: LESSON FROM THE DATABASES
Eduardo Nobile-Orazio, IT
14:15 WS 5.2: PARAPROTEINEMIC NEUROPATHIES: WHEN AND HOW TO TREAT
Jean-Marc Léger, FR
14:45 WS 5.3: POEMS SYNDROME: FROM DIAGNOSIS TO THERAPY
Michelle Mauermann, US

13:45 - 15:15 Workshops WS 6.0
Ultrasound in cranial nerves
ROOM • Berg
Chairs: Stefan Meng, AT & Chieh-Han Tzou, AT
13:45 WS 6.1: ULTRASOUND IN CRANIAL NERVES
Stefan Meng, AT
14:15 WS 6.2: CLINICAL AND MICROSURGICAL SIGNIFICANCE OF CRANIAL NERVE VISUALIZATION
Andres Rodriguez, SE
14:45 WS 6.3: HIGH RESOLUTION MRI OF CRANIAL NERVES
Jennifer Kollmer, DE

13:45 - 15:15 Scientific Session SS 11.0
The Patient voice
ROOM • Klimt 1
Chairs: Kathy Oliver, GB & Stefan Oberndorfer, AT
13:45 SS 11.1: JAMES LIND ALLIANCE PRIORITY SETTING PARTNERSHIP IN NEURO-ONCOLOGY (RESEARCH PRIORITIES)
Kathy Oliver, GB
14:07 SS 11.2: HOW TO DO INVESTIGATOR-INITIATED TRIALS: PCORI
Richard Barohn, US
14:29 SS 11.3: THE PATIENT VOICE IN MYASTHENIA GRAVIS TRIALS- PCORI, CER, AND MORE ALPHABET SOUP
Pushpa Narayanaswami, US
14:51 SS 11.4: HOW CAN OUTCOMES OF CLINICAL STUDIES BE TRANSLATED FOR PATIENTS
Angela Genge, CA

13:45 - 15:15 Overarching Session OA 2.0
Dysphagia: a neglected field in neuromuscular diseases
ROOM • Park Congress 2
Chairs: Zohar Argov, IL
13:45 OA 2.1: NEUROMUSCULAR DISORDERS WITH DYSPHAGIA: AN OUTLINE
Marianne De Visser, NL
14:15 OA 2.2: HOW TO ASSESS AND MANAGE DYSPHAGIA
Zohar Argov, IL
14:45 OA 2.3: ETHICAL ISSUES IN DYSPHAGIA TREATMENTS
David Oliver, GB

15:15 - 15:45 NETWORKING BREAK
ROOM • BRUCKNER - STRAUSS & KLIMT 2 - 3

15:45 - 17:15 Scientific Session SS 12.0
From bench to bedside: inflammatory neuropathies
ROOM • Park Congress 1
Chairs: Hugh Willison, GB
15:45 SS 12.1: PATHOPHYSIOLOGY OF THE NODE OF RANVIER
Antonino Uncini, IT
16:15 SS 12.2: NEW PATHOGENETIC MECHANISMS UNDERLYING IMMUNE NEUROPATHIES
Hugh Willison, GB

Note: There is a variety of different similar products that can be used beyond the ones showcased in this session.
16:45 SS 12.3: ANTIBODIES TO THE NODE OF RANVIER IN CIDP: A NEW CLUE TO THERAPY
Luis Querol, ES

16:30 HIV-ASSOCIATED NEUROMUSCULAR MANIFESTATIONS: EXPERIENCE FROM CAPE TOWN
Jeannine Heckmann, ZA

15:45 - 17:15 Workshops WS 7.0
Cachexia, myopathy and rehabilitation
ROOM ➔ Berg

Chairs: Ioannis Gioulbasanis, GR & Christine Marosi, AT

15:45 WS 7.1: CACHEXIA AND LATE EFFECTS OF CANCER (FROM AN ONCOLOGICAL PERSPECTIVE)
Ioannis Gioulbasanis, GR

15:45 - 17:15 Workshops WS 8.0
Paraprotein in neuropathies
ROOM ➔ Park Congress 3

Chairs: Andreas Steck, CH & Ingemar Merkies, NL

15:45 WS 8.1: SPECTRUM OF PARAPROTEINEMIC NEUROPATHIES
Eduardo Nobile-Orazio, IT

15:45 - 17:15 ENMC symposium
ROOM ➔ Klimt 1

Chairs: George Padberg, NL & Baziel van Engelen, NL

15:45 WHY OPT FOR AN ENMC WORKSHOP?
Baziel Van Engelen, NL

15:45 WS 7.2: SARCOPENIA
Alan Pestronk, US

15:45 - 17:15 THE IMPACT OF 25 YEARS ENMC WORKSHOPS
Raffaella Willmann, CH

15:45 WS 7.3: OUTLINE ON MYOPATHIES / NEUROMUSCULAR COMPLICATIONS ASSOCIATED WITH CANCER, INCL. STEROID MYOPATHY
Anthony Amato, US

16:00 CHARCOT-MARIE-TOOTH DISEASE AT ENMC WORKSHOPS
Mary Reilly, GB

15:45 WS 7.4: CANCER REHABILITATION
Christine Marosi, AT

16:15 ENMC TRANSLATIONAL RESEARCH WORKSHOP
Kanneboyina Nagaraju, US

16:07 WS 7.5: OUTLINE ON MYOPATHIES / NEUROMUSCULAR COMPLICATIONS ASSOCIATED WITH CANCER, INCL. STEROID MYOPATHY
Anthony Amato, US

16:15 AIRWAY CLEARANCE TECHNIQUES IN NEUROMUSCULAR DISORDERS: TIPS FOR A SUCCESSFUL WORKSHOP
Michel Toussaint, BE

16:51 WS 7.6: OUTLINE ON MYOPATHIES / NEUROMUSCULAR COMPLICATIONS ASSOCIATED WITH CANCER, INCL. STEROID MYOPATHY
Anthony Amato, US

17:00 THE POSITION OF NEUROMUSCULAR PATIENTS IN SHARED DECISION MAKING
Anna Ambrosini, IT

17:15 - 18:30 POSTER SESSION PS2
GUIDED POSTER SESSION
ROOM ➔ MEZZANINE FLOOR GALLERY AND FOYERS

15:45 NEUROPATHIES IN AFRICA
Riadh Gouider, TN
CONGRESS PROGRAM  >  MONDAY JULY 9, 2018

7:00 - 8:00  INDUSTRY-SUPPORTED SEMINARS
ROOMS  >  Klimt 1/Berg

8:00 - 10:00  Plenary Session PL 3.0
Motor Neurone Disease
ROOM  >  Park Congress 1 - 2

Chairs: Vera Bril, CA & Claude Desnuelle, FR

08:00  PL 3.1: EMERGING THERAPIES FOR MOTOR NEURONE DISEASES
Albert Ludolph, DE

08:40  PL 3.2: CLINICAL CONCEPT OF ALS
Leonard Van Den Berg, NL

09:20  PL 3.3: ETHICAL ISSUES
David Oliver, GB

10:00 - 10:45  Networking Break
ROOM  >  Bruckner - Strauss & Klimt 2 - 3

10:45 - 12:15  Scientific Session SS 13.0
Spinal muscular atrophy (SMA) - New treatments
ROOM  >  Park Congress 1

Chairs: Peter Andersen, SE & Nathalie Goemans, BE

10:45  SS 13.1: OVERVIEW
Janbernd Kirschner, DE

11:07  SS 13.2: TREATMENTS TO IMPROVE SURVIVAL OF MOTOR NEURONS
Anne-Marie Hübers, DE

11:29  SS 13.3: SPLICING MODIFICATION FOR SMA
Nathalie Goemans, BE

11:51  SS 13.4: GENE THERAPY FOR SMA
Arthur Burghes, US

12:15 - 13:45  LUNCH / INDUSTRY-SUPPORTED SEMINARS
ROOMS  >  Park Congress 1 / 2

13:45 - 15:15  Scientific Session SS 16.0
Care: Non-invasive ventilation and nutritional aspects
ROOM  >  Park Congress 1

Chairs: Marianne De Visser, NL & Esther Hobson, GB

13:45  SS 16.1: NIV AND ALS
Marianne De Visser, NL

14:15  SS 16.2: NUTRITION AND ALS
Esther Hobson, GB
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<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
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<tr>
<td>14:45</td>
<td>SS 16.3: NUTRITION NIV AND COGNITION IN ALS</td>
<td>Vincenzo Silani, IT</td>
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<td>13:45 - 15:15</td>
<td>Scientific Session SS 17.0</td>
<td>Chairs: Wolfgang Grisold, AT &amp; Michelle Mauermann, US</td>
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<td>Peripheral nerve tumors</td>
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<td>ROOM ▶ Klimt 1</td>
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<td>13:45</td>
<td>SS 17.1: NEUROFIBROMATOSIS</td>
<td>Katharina Wimmer, AT</td>
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<td>14:07</td>
<td>SS 17.2: PERIPHERAL NERVE TUMORS</td>
<td>Gelareh Zadeh, CA</td>
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<td>14:29</td>
<td>SS 17.3: INTRANEURAL PERINEURIOMA: MAKING THE DIAGNOSIS AND CLUES TO THE PATHOGENESIS</td>
<td>Michelle Mauermann, US</td>
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<td>14:51</td>
<td>SS 17.4: CANCER AND PERIPHERAL NERVES</td>
<td>Wolfgang Grisold, AT</td>
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<td>Peripheral nerve surgery &amp; regeneration</td>
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<td>ROOM ▶ Berg</td>
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<td>13:45</td>
<td>WS 9.1: NERVE FIBRE TRANSFER VERSUS NERVE RECONSTRUCTION</td>
<td>Robert Schmidhammer, AT</td>
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<td>14:45</td>
<td>WS 9.3: BRAIN PLASTICITY IN NERVE REGENERATION</td>
<td>Thomas Hausner, AT</td>
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<td>Therapeutic landscape in MND</td>
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<td>ROOM ▶ Park Congress 3</td>
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<td>13:45</td>
<td>SS 18.1: MND: THERAPEUTIC LANDSCAPES</td>
<td>Matthew Kiernan, AU</td>
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<td>14:15</td>
<td>SS 18.2: TREATMENT STRATEGIES FOR HEREDITARY ALS</td>
<td>Timothy Miller, US</td>
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<td>14:45</td>
<td>SS 18.3: DIAGNOSIS AND TREATMENT OF MOTOR NEUROPATHIES</td>
<td>Alan Pestronk, US</td>
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<td>13:45 - 15:15</td>
<td>Overarching Session OA 3.0</td>
<td>Chairs: Zohar Argov, IL &amp; Corrado Angelini, IT</td>
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<td>Brain involvement in muscular disorders</td>
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<td>ROOM ▶ Park Congress 2</td>
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<td>13:45</td>
<td>OA 3.1: IMPACT AND EVOLUTION OF COGNITIVE IMPAIRMENT IN MUSCULAR DYSTROPHIES</td>
<td>Corrado Angelini, IT</td>
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<td>14:15</td>
<td>OA 3.2: BRAIN IMAGING TECHNIQUES FOR THE NEUROMUSCULAR PATIENTS</td>
<td>Nicola De Stefano, IT</td>
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<td>14:45</td>
<td>OA 3.3: THE GAP BETWEEN COGNITIVE ASSESSMENT AND BRAIN IMAGING: LESSONS FROM DMD</td>
<td>Erik Niks, NL</td>
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<td>15:15 - 15:45</td>
<td>Networking Break</td>
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<td>ROOM ▶ Bruckner - Strauss &amp; Klimt 2 - 3</td>
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<td>15:45 - 17:15</td>
<td>Workshops WS 10.0</td>
<td>Chairs: Davide Pareyson, IT &amp; Teresa Coelho, PT</td>
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<td>Hereditary ATTR amyloidosis and treatment</td>
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<td>ROOM ▶ Park Congress 2</td>
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<td>15:45</td>
<td>WS 10.1: HOW TRANSTHYRETIN BECOMES TOXIC</td>
<td>Laura Obici, IT</td>
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<td>16:15</td>
<td>WS 10.2: CHALLENGES IN DIAGNOSING AND MONITORING ATTR AMYLOIDOSIS</td>
<td>Davide Pareyson, IT</td>
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<tr>
<td>16:45</td>
<td>WS 10.3: THE NOVEL SCENARIO OF ATTR AMYLOIDOSIS TREATMENT</td>
<td>Teresa Coelho, PT</td>
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### Workshops WS 11.0

**Vitamin deficiencies**

**Room**: Park Congress 3  
**Chairs**: Steven Lewis, US & Nathan Staff, US

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<th>Time</th>
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<th>Chairs</th>
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<tr>
<td>15:45</td>
<td>WS 11.1:</td>
<td>VITAMIN DEFICIENCIES AND NEUROPATHY</td>
<td>Carrie Grouse, US</td>
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<tr>
<td>16:15</td>
<td>WS 11.2:</td>
<td>MINERAL DEFICIENCIES AND NEUROPATHY</td>
<td>Steven Lewis, US</td>
</tr>
<tr>
<td>16:45</td>
<td>WS 11.3:</td>
<td>VITAMIN AND MINERAL TOXICITIES AND NEUROPATHY</td>
<td>Nathan Staff, US</td>
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### Workshops WS 12.0

**Rehabilitation in ALS**

**Room**: Klimt 1  
**Chairs**: Orla Hardiman, IE & Dana Boering, DE

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<th>Session</th>
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<th>Chairs</th>
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<tr>
<td>15:45</td>
<td>WS 12.1:</td>
<td>MULTIDISCIPLINARY CLINICS—WHAT IS THE EVIDENCE AND HOW CAN WE DO BETTER?</td>
<td>Leonard Van Den Berg, NL</td>
</tr>
<tr>
<td>16:15</td>
<td>WS 12.2:</td>
<td>MEASUREMENT OF DISEASE PROGRESSION, CAN WE DO BETTER?</td>
<td>Orla Hardiman, IE</td>
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<tr>
<td>16:45</td>
<td>WS 12.3:</td>
<td>REHABILITATION INTERVENTIONS THAT CHANGE OUTCOME IN ALS/MND</td>
<td>Dana Boering, DE</td>
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### Workshops WS 13.0

**Neoplastic neuromuscular involvement**

**Room**: Berg  
**Chairs**: Wolfgang Grisold, AT & P. James Dyck, US

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<tbody>
<tr>
<td>15:45</td>
<td>WS 13.1:</td>
<td>CRANIAL NERVES</td>
<td>Wolfgang Grisold, AT</td>
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<tr>
<td>16:45</td>
<td>WS 13.3:</td>
<td>CANCER-ASSOCIATED MYOSITIS</td>
<td>Yves Allenbach, FR</td>
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### Scientific Session SS 19.0

**Distal SMA**

**Room**: Park Congress 1  
**Chairs**: Wolfgang Löscher, AT

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<tbody>
<tr>
<td>15:45</td>
<td>SS 19.1:</td>
<td>DISTAL HEREDITARY MOTOR NEUROPATHIES (DHMN)</td>
<td>Mary Reilly, GB</td>
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<tr>
<td>16:15</td>
<td>SS 19.2:</td>
<td>DISTAL MYOPATHIES</td>
<td>Anthony Amato, US</td>
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<td>16:45</td>
<td>SS 19.3:</td>
<td>ACQUIRED DISTAL MOTOR SYNDROMES</td>
<td>Matthew Kiernan, AU</td>
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### Poster Session PS3

**Guided Poster Session**

**Room**: Mezzanine Floor Gallery and Foyers
**CONGRESS PROGRAM**  >  **TUESDAY JULY 10, 2018**

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<thead>
<tr>
<th>Time</th>
<th>Session/Workshop</th>
<th>Room</th>
<th>Chairs/Speakers</th>
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<tbody>
<tr>
<td><strong>8:00 - 10:00</strong></td>
<td><strong>Plenary Session PL 4.0</strong>  &lt;br&gt;Neuromuscular Junction  &lt;br&gt;<strong>ROOM</strong> &gt; Park Congress 1 - 2</td>
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<td>Julia Wanschitz, AT &amp; Juan Jesús Vilchez Padilla, ES</td>
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<td>08:00</td>
<td><strong>PL 4.1: PHYSIOLOGY AND STRUCTURE</strong>  &lt;br&gt;Steve Burden, US</td>
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<td>08:40</td>
<td><strong>PL 4.2: CONGENITAL MYASTHENIC SYNDROMES - NEW GENES AND BETTER TREATMENTS / ANTIBODIES</strong>  &lt;br&gt;Kinji Ohno, JP</td>
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<tr>
<td>09:20</td>
<td><strong>PL 4.3: AUTOIMMUNITY</strong>  &lt;br&gt;Angela Vincent, GB</td>
<td></td>
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</tr>
<tr>
<td><strong>10:00 - 10:45</strong></td>
<td><strong>NETWORKING BREAK</strong>  &lt;br&gt;ROOMS &gt; BRUCKNER - STRAUSS &amp; KLIMT 2 - 3</td>
<td></td>
<td></td>
</tr>
<tr>
<td>10:45 - 12:15</td>
<td><strong>Scientific Session SS 20.0</strong>  &lt;br&gt;New therapies in myasthenia - clinical session  &lt;br&gt;<strong>ROOM</strong> &gt; Park Congress 1</td>
<td></td>
<td>Gil Wolfe, US &amp; Wolfgang Löscher, AT</td>
</tr>
<tr>
<td>10:45</td>
<td><strong>SS 20.1: EVOLUTION OF MONOCLONAL AB TREATMENT APPROACHES IN MG</strong>  &lt;br&gt;Gil Wolfe, US</td>
<td></td>
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<tr>
<td>11:15</td>
<td><strong>SS 20.2: OTHER NOVEL TREATMENT APPROACHES: EXERCISE</strong>  &lt;br&gt;Anna Punga, SE</td>
<td></td>
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</tr>
<tr>
<td>11:45</td>
<td><strong>SS 20.3: OVERVIEW OF TREATMENT GUIDELINES: GOING FORWARD</strong>  &lt;br&gt;Gil Wolfe, US</td>
<td></td>
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<tr>
<td>10:45 - 12:15</td>
<td><strong>Scientific Session SS 21.0</strong>  &lt;br&gt;Infectious neuropathies  &lt;br&gt;<strong>ROOM</strong> &gt; Park Congress 2</td>
<td></td>
<td>John D. England, US &amp; Gerard Said, FR</td>
</tr>
<tr>
<td>11:07</td>
<td><strong>SS 21.2: LEPROUS NEUROPATHIES</strong>  &lt;br&gt;Gerard Said, FR</td>
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<tr>
<td>11:29</td>
<td><strong>SS 21.3: NEUROPATHIES IN INFECTIOUS DISEASES</strong>  &lt;br&gt;Nazha Birouk, MA</td>
<td></td>
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<tr>
<td>11:51</td>
<td><strong>SS 21.4: INFECTION RELATED NEUROMUSCULAR DISORDERS (NEUROPATHIES AND MYOPATHIES)</strong>  &lt;br&gt;Chandrashekhar Meshram, IN</td>
<td></td>
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<tr>
<td><strong>12:15 - 13:45</strong></td>
<td><strong>LUNCH / INDUSTRY-SUPPORTED SEMINARS</strong>  &lt;br&gt;ROOMS &gt; PARK CONGRESS 1 / 2</td>
<td></td>
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</tr>
<tr>
<td>13:45 - 15:15</td>
<td><strong>Scientific Session SS 23.0</strong>  &lt;br&gt;Issues regarding thymectomy in myasthenia  &lt;br&gt;<strong>ROOM</strong> &gt; Park Congress 1</td>
<td></td>
<td>Richard Barohn, US &amp; Alexander Marx, DE</td>
</tr>
<tr>
<td>13:45</td>
<td><strong>SS 23.1: THYMECTOMY FOR MYASTHENIA GRAVIS</strong>  &lt;br&gt;Richard Barohn, US</td>
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<tr>
<td>14:15</td>
<td><strong>SS 23.2: RANDOMIZED CONTROLLED TRIAL OF THYMECTOMY: A DEEPER DIVE INTO DATA</strong>  &lt;br&gt;Gil Wolfe, US</td>
<td></td>
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</tr>
<tr>
<td>14:45</td>
<td><strong>SS 23.3: PATHOLOGY OF THE THYMUS GLAND IN MG</strong>  &lt;br&gt;Alexander Marx, DE</td>
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<tr>
<td>13:45 - 15:15</td>
<td><strong>Workshops WS 14.0</strong>  &lt;br&gt;Discussion on novel treatments and their mechanisms in different forms of NMJ disorders  &lt;br&gt;<strong>ROOM</strong> &gt; Park Congress 2</td>
<td></td>
<td>Markus Rüegg, CH &amp; David Beeson, GB</td>
</tr>
<tr>
<td>13:45</td>
<td><strong>WS 14.1: MOLECULAR MECHANISMS AFFECTING NEUROMUSCULAR JUNCTION DEVELOPMENT AND MAINTENANCE</strong>  &lt;br&gt;Markus Rüegg, CH</td>
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<td>14:15</td>
<td><strong>WS 14.2: SALBUTAMOL AND DOK-7 AS POTENTIAL TREATMENT OPTIONS FOR NEUROMUSCULAR DISORDERS</strong>  &lt;br&gt;David Beeson, GB</td>
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<tr>
<td>Time</td>
<td>Session/Workshop</td>
<td>Location</td>
<td>Chairs/Authors</td>
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<tr>
<td>13:45</td>
<td>WS 15.1: PLECTIN: OVERVIEW AND LESSONS FROM MOUSE MODELS</td>
<td></td>
<td>Gerhard Wiche, AT</td>
</tr>
<tr>
<td>14:07</td>
<td>WS 15.2: HUMAN PLECTIN AND CLINICAL ASPECTS</td>
<td></td>
<td>Rolf Schröder, DE</td>
</tr>
<tr>
<td>14:29</td>
<td>WS 15.3: PLECTIN AND MITOCHONDRIA (MICE AND MEN)</td>
<td></td>
<td>Lilli Winter, AT</td>
</tr>
<tr>
<td>14:51</td>
<td>WS 15.4: PLECTIN, CARDIOMYOPATHIES, AND THERAPEUTIC APPROACHES (MICE)</td>
<td></td>
<td>Oliver Mueller, DE</td>
</tr>
<tr>
<td>13:45 - 15:15</td>
<td>Workshops WS 17.0 Miscellaneous diseases</td>
<td>Klimt 1</td>
<td>Chairs: Andreas Steck, CH &amp; Hadi Manji, GB</td>
</tr>
<tr>
<td>13:45</td>
<td>WS 17.1: PRIMARY AND SECONDARY VASCULITIC NEUROPATHY</td>
<td></td>
<td>Gerard Said, FR</td>
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<td>14:15</td>
<td>WS 17.2: STATIN NEUROPATHIES</td>
<td></td>
<td>Hadi Manji, GB</td>
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<tr>
<td>15:15 - 15:45</td>
<td>NETWORKING BREAK</td>
<td>Bruckner - Strauss &amp; Klimt 2 &amp; 3</td>
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<tr>
<td>15:45 - 17:15</td>
<td>Scientific Session SS 24.0 Congenital Myasthenic Syndrome (CMS)</td>
<td>Park Congress 1</td>
<td>Chairs: David Beeson, GB &amp; Friedrich Zimprich, AT</td>
</tr>
<tr>
<td>15:45</td>
<td>SS 24.1: SYNAPTIC STABILITY IN CMS</td>
<td></td>
<td>David Beeson, GB</td>
</tr>
<tr>
<td>16:07</td>
<td>SS 24.2: RECENT PROGRESS IN UNDERSTANDING PRESYNAPTIC CONGENITAL MYASTHENIC SYNDROMES (CMS)</td>
<td></td>
<td>Hanns Lochmüller, DE</td>
</tr>
<tr>
<td>16:29</td>
<td>SS 24.3: CLINICAL ASPECTS OF CMS</td>
<td></td>
<td>Hakan Cetin, AT</td>
</tr>
</tbody>
</table>
| 16:51    | SS 24.4: THERAPEUTIC STRATEGIES FOR CONGENITAL MYASTHENIC SYNDROMES              |             | Jacqueline Palace, GB}
<table>
<thead>
<tr>
<th>Time</th>
<th>Session Description</th>
<th>Room</th>
<th>Chairs</th>
</tr>
</thead>
<tbody>
<tr>
<td>15:45 - 17:15</td>
<td>Workshops WS 18.0 Biomarkers in myasthenia gravis</td>
<td>Park Congress 2</td>
<td>Nils Erik Gilhus, NO &amp; Anna Punga, SE</td>
</tr>
<tr>
<td>15:45</td>
<td>WS 18.1: AUTOANTIBODIES IN MYASTHENIA GRAVIS</td>
<td></td>
<td>Nils Erik Gilhus, NO</td>
</tr>
<tr>
<td>16:15</td>
<td>WS 18.2: CIRCULATING MICRO-RNA AS BIOMARKERS IN MYASTHENIA GRAVIS</td>
<td></td>
<td>Anna Punga, SE</td>
</tr>
<tr>
<td>16:45</td>
<td>WS 18.3: THYMUS BIOMARKERS AND CLINICAL RELEVANCE</td>
<td></td>
<td>Alexander Marx, DE</td>
</tr>
<tr>
<td>15:45 - 17:15</td>
<td>Workshops WS 19.0 Muscle and nerve biopsies for neurologist</td>
<td>Park Congress 3</td>
<td>Rolf Schröder, DE &amp; Monika Hofer, GB</td>
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<tr>
<td>15:45</td>
<td>WS 19.1: MUSCLE BIOPSIES</td>
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<td>Rolf Schröder, DE</td>
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<tr>
<td>16:45</td>
<td>WS 19.3: SKIN BIOPSIES</td>
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<td>Giuseppe Lauria, IT</td>
</tr>
<tr>
<td>15:45 - 17:15</td>
<td>Workshops WS 20.0 Aspects of diabetes</td>
<td>Klimt 1</td>
<td>Eva Feldman US &amp; Brian Christopher Callaghan, US</td>
</tr>
<tr>
<td>15:45</td>
<td>WS 20.1: DIABETIC CRANIAL NEUROPATHIES</td>
<td></td>
<td>Tudor Lupescu, RO</td>
</tr>
<tr>
<td>16:07</td>
<td>WS 20.2: MUSCULOSKELETAL COMPLICATIONS OF DIABETES ARE COMMON AND UNDERDIAGNOSED</td>
<td></td>
<td>Anna Grisold, AT</td>
</tr>
<tr>
<td>16:29</td>
<td>WS 20.3: ATYPICAL NEUROPATHIES IN DIABETES: DIAGNOSIS AND MANAGEMENT</td>
<td></td>
<td>Amanda Peltier, US</td>
</tr>
<tr>
<td>16:51</td>
<td>WS 20.4: TREATMENT INDUCED AND INFLAMMATORY NEUROPATHIES IN DIABETES</td>
<td></td>
<td>Brian Christopher Callaghan, US</td>
</tr>
<tr>
<td>17:15 - 18:15</td>
<td>CLOSING CEREMONY</td>
<td>Park Congress 1 – 2</td>
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</tbody>
</table>
GENERAL INFORMATION FROM A-Z

CAMERAS AND CELL PHONES

No video cameras are allowed at any event during ICNMD 2018. As a courtesy to fellow attendees, please turn off cell phones during scientific sessions.

CATERING ONSITE

Networking Breaks

Networking breaks will take place from Friday 6 to Tuesday 10 July in the Exhibition & Poster Area, located on the Mezzanine Floor.

FRIDAY, JULY 6

09.50 – 10.10 AND 12.00-13.00 AND 14:50-15:10

SATURDAY, JULY 7 – TUESDAY, JULY 10

10.00-10:30 AND 12:30 – 13:30 AND 15:15-15:45

Snacks and Lunch

Luncheon industry-supported seminars are offering lunch. During lunch time light snacks will also be offered within the exhibition and poster area.

CERTIFICATE OF ATTENDANCE

To complete the session evaluation and obtain credits for your attendance at ICNMD 2018, please use the online evaluation link provided via email on the last day of the Congress.

CHARGING LOUNGE

Charging stations will be available in the Exhibit Hall (Rooms Bruckner - Strauss) to allow you to take a break while powering your mobile devices.

Supported by

CSL Behring

Biotherapies for Life®
CLOAK ROOM

The cloak room is located within the bag distribution area in the Park Congress Foyer Area on the Ground Floor.

CME CREDIT ALLOWANCE

The 15th International Congress on Neuromuscular Diseases (ICNMD 2018) is accredited by the European Accreditation Council for Continuing Medical Education (EACCME) to provide the following CME activity for medical specialists. The EACCME is an institution of the European Union of Medical Specialists (UEMS), www.uems.net.

ICNMD 2018 will be accredited with 34 European CME credits (ECMEC) by the European Accreditation Council for Continuing Medical Education (EACCME).

Through an agreement between the European Union of Medical Specialists and the American Medical Association, physicians may convert EACCME credits to an equivalent number of AMA PRA Category 1 Credits™. Information on the process to convert EACCME credit to AMA credit can be found at www.ama-assn.org/go/internationalcme.

DELEGATE HELP DESK

If you require assistance or any information regarding the Congress, see the staff at the Delegate Help Desk located in the registration area.

Supported by

AUDENTES

DELEGATE LOUNGES

Delegate Lounges are available in the Exhibit Hall (Klimt 2+3) to allow you to take a break, network with colleagues or check emails.

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Biotherapies for Life®
Duchenne muscular dystrophy*: working together to reduce the time to diagnosis and improve patient outcomes

A symposium at the 15th International Congress on Neuromuscular Diseases

Tuesday 10th July 2018, 12:30–13:30
Park Congress 1
Hilton Vienna, Vienna, Austria

*Ataluren is only indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older.

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 15th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2018.

Translarna™ (active ingredient: ataluren) is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene (nmDMD), in ambulatory patients aged 5 years and older. Efficacy has not been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing. Posology and administration: Translarna is available as granules for oral suspension in sachets of 125 mg, 250 mg or 1000 mg. The recommended dose is 10 mg/kg body weight in the morning, 10 mg/kg body weight at midday, and 20 mg/kg body weight in the evening (for a total daily dose of 40 mg/kg body weight). Patients should not take a double or extra dose if a dose is missed. It is important to administer the correct dose. Increasing the dose above the recommended dose may be associated with reduced effectiveness. Treatment with Translarna should only be initiated by specialist physicians with experience in the management of DMD. Ingredients: Active ingredient: ataluren. Excipients: polydextrose (E1200), macrogol, poloxamer, mannitol (E421), crosovidone, hydroxyethyl cellulose, artificial vanilla flavour (maltodextrin, artificial flavours and propylene glycol), silica, colloidal anhydrous (E551), magnesium stearate. Contraindications: Patients with hypersensitivity to the active substance or to any of the excipients; concomitant use of intravenous aminoglycosides. Special warnings and precautions for use: Patients who do not have a nonsense mutation should not receive Translarna. Patients with renal impairment should be closely monitored. It is recommended that total cholesterol, LDL, HDL, triglycerides be measured annually, and serum creatinine, BUN, cystatin C be measured every 6 to 12 months. Resting systolic and diastolic blood pressure should be monitored every 6 months in patients receiving Translarna concomitantly with corticosteroids. All clinical measures and/or laboratory testing may be conducted more frequently as needed based on clinical status. See precaution for use with other medicines in next “interactions” section. Interactions: Translarna should not be coadministered with intravenous aminoglycosides, and concomitant use of other nephrotoxic agents is not recommended. Caution should be exercised when Translarna is co-administered with medicinal products that are inducers of UGT1A9, or substrates of OAT1, OAT3 or OATP1B3 and when co-administered with adefovir. Based on in vitro studies Translarna is not expected to be an inducer of P450 isoenzymes. Fertility, pregnancy and lactation: It is recommended to avoid the use of Translarna in pregnancy. Breast-feeding should be discontinued during treatment with Translarna. Non-clinical data revealed no hazard for humans based on standard male and female fertility study in rats. Effects on ability to drive and use machines: Patients who experience dizziness should use caution when driving, cycling or using machines. Adverse reactions: Adverse events reported in clinical trials of predominantly paediatric nmDMD patients treated at the recommended dose of 10-, 10-, 20mg/kg/day according to frequency. Very common (≥1/10): vomiting. Common (≥1/100 to <1/10): decreased appetite, hypertriglyceridemia, headache, hypertension, cough, epistaxis, nausea, upper abdominal pain, flatulence, abdominal discomfort, constipation, rash erythematous, pain in extremity, musculoskeletal chest pain, haematuria, enuresis, pyrexia, weight decreased. Events with unknown frequency due to low numbers: increased blood urea nitrogen, cholesterol, creatinine, cystatin C, triglycerides. Marketing Authorisation number and holder: EU/1/13/002/001-002-003. PTC Therapeutics International Limited, 5th Floor, 3 Grand Canal Plaza, Grand Canal Street Upper, Dublin 4, Ireland. Please consult the SmPC before prescribing. Date of Preparation: April 2018.

This medicine is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system. Adverse events should also be reported to PTC at medinfo@ptcbio.com
DIGITAL INFO BOARD

A digital info board is located on the Mezzanine Level. It will offer the latest info on the Congress programme, and serve as a social media wall, keeping track of posts and interactions through Facebook and Twitter.

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EMERGENCY SERVICES AND FIRST AID

Emergency Number 112

In case of emergency, please contact the Front Desk of the Hilton Vienna am Stadtpark.

EXHIBITION & POSTER AREA

The Exhibition & Poster Area is located on the Mezzanine Floor at Hilton Vienna am Stadtpark, in and around Rooms Bruckner - Strauss & Klimt 2 - 3. Please find a floor plan of the exhibition and a detailed list of all exhibitors on pages 76-80.

EXHIBITION OPENING HOURS

<table>
<thead>
<tr>
<th>Day</th>
<th>Hours</th>
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<tbody>
<tr>
<td>Friday, July 6</td>
<td>19:00 – 21:00</td>
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<tr>
<td>Saturday, July 7</td>
<td>10:00 - 16:00 &amp; 17:00 - 18:30</td>
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<tr>
<td>Sunday, July 8</td>
<td>10:00 - 16:00 &amp; 17:00 - 18:30</td>
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<tr>
<td>Monday, July 9</td>
<td>10:00 - 16:00 &amp; 17:00 - 18:30</td>
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<tr>
<td>Tuesday, July 10</td>
<td>10:00 - 16:00</td>
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LANYARDS AND BADGES

Your personalized badge is your admission card to the congress. For organizational and security reasons, badges must be worn at the congress venue at all times. A lanyard will be given to you along with the congress materials. In case of loss, a replacement badge will be provided at an administrative charge of EUR 50.00.

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LOST AND FOUND

Lost and Found items should be returned/claimed at the Concierge Desk at the Hilton Vienna am Stadtpark.

ONSITE MOBILE APPLICATION

Download the ICNMD 2018 mobile app for a convenient way to stay up to date via your phone or tablet. View the full scientific schedule, abstracts, exhibit information (including floor plans) and general Congress and venue information on our easy-to-use app!

The app is compatible with all iOS devices (iPhone, iPod touch and iPad) and all Android mobile devices. Timely updates on program or room changes will be distributed through the mobile app via notification alerts.

Supported by

REGISTRATION INFORMATION

Registration for all attendees (delegates, invited speakers, exhibitors, sponsors, media and accompanying persons) is located at the Hotel Hilton Vienna am Stadtpark in the Park Congress Foyer Area on the Ground Floor.

<table>
<thead>
<tr>
<th>Day</th>
<th>Hours</th>
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<tbody>
<tr>
<td>Thursday, July 5</td>
<td>13:00 – 18:00</td>
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<tr>
<td>Friday, July 6</td>
<td>07:00 – 20:00</td>
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<tr>
<td>Saturday, July 7</td>
<td>06:30 – 18:00</td>
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<td>Sunday, July 8</td>
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<td>Monday, July 9</td>
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<tr>
<td>Tuesday, July 10</td>
<td>06:30 – 15:30</td>
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REGISTRATION COUNTERS

Pre-Registration

For all delegates who have registered online and paid in full before arriving at the Congress

Note: Delegates who have registered online but have not yet paid in full, please proceed to the Outstanding Payment Counter

Exhibitors

For all exhibitors/sponsors
Patients with DMD experience progressive decline in respiratory muscle function, which leads to respiratory complications that are among the leading causes of hospitalization and premature death.

Join us and a panel of international experts on Sunday July 8th, as we discuss the latest insights and treatment strategies to address respiratory function decline in DMD.

Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company committed to developing medicines to meet the needs of patients living with mitochondrial disorders and other rare diseases.

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 15th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2018.
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Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company committed to developing medicines to meet the needs of patients living with mitochondrial disorders and other rare diseases.

Job number: 0-MD-0008-0418-V1-1  Date of preparation: April 18th 2018

Sunday July 8th, 12:15 - 13:45
Room: Park Congress 2
Hilton Vienna
Chair:
Prof Thomas Voit
Speakers:
Prof Craig McDonald
Dr Oscar H Mayer
Prof Thomas Voit
Lunch will be provided

Join us at the Santhera Symposium:
Respiratory function decline in Duchenne muscular dystrophy (DMD) – Insights and evolving treatment strategies

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 15th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2018.

Onsite Registration/Outstanding Payments /Additional Tickets and Purchases

› For all delegates not yet registered for the Congress
› For all delegates who have pre-registered but not yet (fully) paid
› For all delegates wanting to purchase additional items (subject to availability):
   - Pre-Congress Teaching Course Tickets
   - Social Event Tickets

RESTAURANTS
There are plenty of restaurant choices in Vienna. The staff at the Concierge Information Desk will be happy to assist with recommendations and bookings.

SECURITY
Security measures have been implemented for the safety of participants. Name badges must be worn at all times during the Congress.

SMOKING
Smoking is prohibited in all areas of the Hilton Vienna am Stadtpark.

SOCIAL MEDIA
Join the Conversation @ICNMD2018 Official Social Media Accounts
Share your experience and images from ICNMD 2018 with colleagues and friends via Twitter and Facebook. Our official social media feeds throughout the Congress can be followed @ICNMD2018. We encourage you to tag your posts with #ICNMD2018.

STAFF & VOLUNTEERS
Staff and Volunteers are happy to assist with any questions delegates may have regarding the Congress or Vienna, Austria. Delegates can easily locate them by their staff/volunteer shirts.

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WIRELESS INTERNET
ICNMD is providing free Wi-Fi throughout the Hilton Vienna am Stadtpark. To ensure a positive Wi-Fi experience for all users please do not use your own wireless Hotspot device. These additional Wi-Fi devices create significant RF interference which can affect all Wi-Fi networks. Please turn these devices off, connect to the Wi-Fi network ICNMD2018 and open your web browser to connect to the Internet, with the password ICNMD2018.

Supported by

DISCLAIMER
The organizers have made every attempt to ensure that all information in this publication is correct. The organizers take no responsibility for changes to the program or any inconvenience that may occur as a result of changes to the program. Some of the information provided in this publication has been provided by external sources. Although every effort has been made to ensure the accuracy, currency and reliability of the content, the organizers accept no responsibility in that regard.
OFFICIAL NETWORKING EVENTS

OPENING CEREMONY & WELCOME RECEPTION

FRIDAY, JULY 6 18:30 – 21:00

Venue: Opening Ceremony (Park Congress I+II, Ground Floor) & Welcome Reception: (Exhibition & Poster Area, Mezzanine Level)

The evening will be a truly Austrian Affair! The Opening Ceremony will set the tone for the Congress with official remarks while featuring a glimpse into the multiculturalism that gives Vienna & Austria its uniqueness.

Following the Opening Ceremony, guests are invited to the Exhibit & Poster Area for the Welcome Reception and the opportunity to mix and mingle with exhibitors, colleagues and friends while enjoying local wines, beers and small snacks.

Included in the registration fee for delegates and accompanying persons.

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CONGRESS DINNER

SUNDAY, JULY 8 19:30 – 22:30

Venue: City Hall Vienna

Experience a traditional and unique Austrian dinner in one of the most stunning venues in Vienna. The City Hall will be the perfect setting to enjoy an evening with your friends and fellow colleagues.

Don’t miss a cocktail reception accompanied by a classical string quartet followed by a 3-course meal. Last, but not least we will introduce you to the famous Viennese Waltz.

Tickets are not included in the registration fee and have to purchased separately.
HEURIGEN EVENING

MONDAY, JULY 9
19:00 – 22:00
Venue: Heurigen Mayer am Pfarrplatz

Enjoy a relaxing evening at one of Vienna’s finest Wine Taverns - Mayer am Pfarrplatz. This event will treat you to excellent wines and traditional Viennese Heurigen Food - from traditional bread spreads, cold cuts to roast pork and Wiener Schnitzel. The evening will be accompanied by Viennese Heurigen Music.

Tickets are not included in the registration fee and have to purchased separately.

CLOSING CEREMONY

TUESDAY, JULY 10
17:00 – 17:30
Venue: Park Congress I, Ground Floor

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 XVI ICNMD Congress in 2020.

Included in the registration fee for delegates and accompanying persons.
## POSTER SESSIONS

**SUPPORTED BY SANOFI GENZYME**

Posters will be displayed in the Foyers and Corridors on the Mezzanine Level. All poster presenters need to report to the Poster Service Desk (at registration) to check in which area their poster is scheduled for display.

### POSTER DISPLAY CATEGORIES

<table>
<thead>
<tr>
<th>Topic Group 1 - Muscle Diseases of Genetic Origin and Acquired Myopathies: Clinical Features, Pathophysiology, Therapy</th>
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</thead>
<tbody>
<tr>
<td>• Dystrophinopathy</td>
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<td>• Muscle Dystrophies (Non-Dystrophinopathy)</td>
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<tr>
<td>• Congenital Muscular Dystrophy</td>
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<tr>
<td>• Congenital Myopathies / Myopathies with Prominent Muscle Contractures</td>
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<tr>
<td>• Distal Myopathy / Myofibrillar Myopathies</td>
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<td>• Myotonic Myopathies</td>
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<tr>
<td>• Facioscapulohumeral Muscular Dystrophies / Oculopharyngeal Muscular Dystrophy</td>
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<tr>
<td>• Metabolic Myopathies / Mitochondrial Myopathies</td>
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<tr>
<td>• Muscle Channelopathies and Related Disorders</td>
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<tr>
<td>• Other Myopathies including GNE – Hereditary Inclusion Body Myopathy</td>
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<tr>
<td>• Inflammatory / Dysimmune Myopathies</td>
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<tr>
<td>• Inclusion Body Myositis</td>
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<tr>
<td>• Toxic / Endocrine / Other Acquired Myopathies</td>
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<tr>
<th>Topic Group 5 – Novel Diagnostic Methods in Neuromuscular Diseases and Basic Sciences in Neuromuscular Diseases</th>
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<tbody>
<tr>
<td>• Ultrasound</td>
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<td>• MRI</td>
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<td>• Other Biomarkers</td>
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<tr>
<td>• Electrophysiology</td>
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<tr>
<td>• Small Nerve Fibre Evaluation</td>
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<tr>
<td>• Biochemical and Molecular Techniques</td>
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<tr>
<td>• Muscle Homeostasis / Muscle Regeneration</td>
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<tr>
<td>• Muscle Structure / Muscle Development / Muscle Growth</td>
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<tr>
<td>• Muscle Atrophy / Degeneration</td>
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<tr>
<td>• Nuclear Envelope / Nuclear Matrix of Muscle Cell</td>
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<td>• Ion Channel Function in Neuron and Muscle</td>
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<td>• Immune Mechanisms in Neuromuscular Diseases</td>
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<tr>
<td>• Fundamental Approaches to Motor Neuron, Axon and Related Structures</td>
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<tr>
<td>• Neuromuscular Junction: Basic Aspects</td>
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<td>• Others</td>
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<tr>
<th>Topic Group 2 - Diseases of Neuromuscular Junction: Clinical Features, Pathophysiology, Therapy</th>
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<tr>
<td>• Myasthenia Gravis</td>
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<tr>
<td>• Myasthenic Syndromes</td>
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<tr>
<td>• Congenital Myasthenia</td>
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<th>Topic Group 6 - Cancer related Disorders and General Diseases</th>
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<th>Topic Group 3 - Peripheral Neuropathy, Cranial Nerves, Mononeuropathies: Clinical Features, Pathophysiology, Therapy</th>
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<tbody>
<tr>
<td>• Inflammatory / Dysimmune / Associated with Monoclonal Gammopathy/Paraneoplastic</td>
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<tr>
<td>• Hereditary Peripheral Neuropathy</td>
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<td>• Metabolic / Toxice</td>
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<td>• Infectious Peripheral Neuropathy (including Leprosy, HIV)</td>
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<td>• Others</td>
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<th>Topic Group 7 - Patient Related Issues</th>
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<th>Topic Group 4 - Motor Neuron Diseases: Clinical Features, Pathophysiology, Therapy</th>
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<tbody>
<tr>
<td>• Biology, Genetics</td>
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<tr>
<td>• Biomarkers in MND</td>
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<tr>
<td>• Epidemiology, Clinic, Treatment</td>
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<tr>
<td>• Spinal Muscular Atrophy / Neuronopathies</td>
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<th>Topic Group 8 - History</th>
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<th>Topic Group 9 - Miscellaneous</th>
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<td>• Outcome Measures in Clinical Trials</td>
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<td>• Biomarkers in Neuromuscular Disorders</td>
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<td>• Home Care / Social Programs in Neuromuscular Diseases</td>
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<td>• Psychological and Neuropsychological Approaches in Neuromuscular Diseases</td>
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<td>• Ethics in Neuromuscular Disorders</td>
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<td>• Rehabilitation in Neuromuscular Diseases</td>
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<td>• Other</td>
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POSTER DISPLAY TIMES
SATURDAY, JULY 7 – MONDAY JULY 9, 2018
17:15 – 18:30
Poster presenters are requested to be beside their poster during the poster session

Important Information for Poster Presenters

Posters can only be mounted on the day on which they have been scheduled for presentation.

Posters must be dismounted at the end of each day after 18:30 but before 19:00 on Saturday, July 7 & Sunday, July 8 and after 15:45 but before 16:00 on Tuesday, July 10. Any posters that are not dismounted by the required time will be taken down and stored at the Poster Service Desk. Posters not picked up until Tuesday 18:00 will be discarded.

PS1 GUIDED POSTER SESSION
SATURDAY, JULY 7, 2018
17:15 - 18:30
ROOM > Mezzanine Floor Gallery and Foyers

PS1Group1-001 A FAMILY-BASED STUDY INTO PENETRANCE IN FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY TYPE 1
*M. Wohlgemuth*1, R.J. Lemmers2, M.A. Jonker3, E.L. Van der Kooi1, C.G. Horlings1, B.G. Van Engelen1, S.M. Van der Maarel1, G.W. Padberg6, N.C. Voermans1; 1Neurology/Leiden/NL, 2Human genetics/Leiden/NL, 3Health sciences/Nijmegen/NL, 4/Leeuwarden/NL, 5Human Genetics/Leiden/NL, 6Dep. of Neurology/Nijmegen/NL

PS1Group1-002 EVALUATING THE USEFULNESS OF NEW LINE IMMUNOASSAYS FOR MYOSITIS ANTIBODIES IN CLINICAL PRACTICE: A RETROSPECTIVE STUDY
*F. Montagnese*1, H. Babac1, P. Eichhorn2, B. Schoser3; 1Neurology/Munich/DE, 2Institute of Laboratory Medicine/Munich/DE, 3Friedrich-Baur Institute, Department of Neurology/Munich/DE

PS1Group1-003 EXPRESSION OF DP116 IS A PREDISPONING FACTOR FOR CARDIAC DISFUNCTION IN DUCHENNE MUSCULAR DYSTROPHY
*T. Yamamoto*1, A. Hiruyuki1, T. Imanishi1, Y. Nakamachi1, M. Matsuo2, K. Iijima3, J. Saegusa1; 1Department of Clinical Laboratory/--, Kusunoki-cho, Chuo-ku, Kobe/JP, 2Department of Physical Therapy, Faculty of Rehabilitation/Kobe/JP, 3Department of Pediatrics/Kobe/JP

PS1Group1-004 RIMEPORIDE: RESULTS FROM A PHASE Ib STUDY IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
T. Gidar1, L. Servais1, S. Previtali2, A. Zambon2, J. Pitchforth3, K. Maresch3, J. Diaz-Manera4, P.G. Carlier5, P.Y. Baudin6, B. Marty5, S. Carnesecchi7, C. Laveille8, J. Gray9, *F. Porte Thome*9, D. Labolle9, M. Annousamy1, V. Chê1, M.G. Natali Sora2, S. Gerevini10, N. Vidal4, K. Groves11, F. Muntoni3; 1-Motion/Paris/FR, 2Neurology/Milano/IT, 3/London/GB, 4Neuromuscular disorders Unit/Barcelona/ES, 5AIM & CEA NMR laboratory, Neuromuscular Investigation Center/Paris/FR, 6/Froyennes/BE, 7Department of Pathology and Immunology/Genova/CH, 8/Lyon/FR, 9R&D/Plan Les Ouates/CH, 10Department of Neuroradiology/Milano/IT, 11Great Ormond Street Hospital for Children/London/GB

PS1Group1-005 A LONG TERM PREVENTIVE EFFICACY STUDY WITH RIMEPORIDE, A SODIUM-PROTON EXCHANGER INHIBITOR, IN GRMD DOGS
I. Barthélémy1, J. Su2, Y. Fromes3, S. Carnesecchi, *F. Porte Thome*5, P.G. Carlier3, B. Ghaleh2, S. Blot1; 1U955 - IMRB, Inserm/Maisons-Alfort/FR, 2Inserm UMR 955/Créteil/FR, 3AIM & CEA NMR laboratory, Neuromuscular Investigation Center/Paris/FR, 4Department of Pathology and Immunology/Genova/CH, 5R&D/Plan Les Ouates/CH

PS1Group1-006 PREDICTORS OF EARLY LEFT VENTRICULAR SYSTOLIC DYSDNFUNCTION IN DMD PATIENTS

PS1Group1-007 GENE THERAPY BY CRISPR-CAS9 MEDIATED EXON SKIPPING IN A PRE-CLINICAL MODEL OF DMD, THE GRMD DOG
*I. Punzón*1, I. Barthélémy1, F. Auradé2, N. Blanchard-Gutton1, F. Pietri-Rouxel3, F. Relaix2, S. Blot1; 1Neurobiology/Maisons Alfort/FR, 2Henri Mondor Hospital School of Medicine/Créteil Cedex/FR, 3/Paris/FR

PS1Group1-008 IN VIVO CELL TRACKING OF CANINE MYOBLASTS BY SODIUM/IODIDE SYMPORTER GENE EXPRESSION

PS1Group1-009 CLINICAL OUTCOME STUDY OF DYSFERLINOPATHY: RELATIONSHIP BETWEEN MUSCLE MRI AND PHYSIOTHERAPY OUTCOME MEASURES
*J.D. Manera*1, R. Fernández-Torrón2, 3, M. James2, A. Mayhew2, M. Eagle2, R. Muni Lofra2, F. Smith4, H. Sutherland2, A. Groves11, F. Muntoni3; 1I-Motion/Paris/FR, 2Neurology/Paris/FR, 3/London/GB, 4Neuromuscular disorders Unit/Paris/FR, 5/Leuven/BE
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- The first and only subcutaneous Ig (SCIG) for CIDP
- CSL Behring: over 100 years of expertise in developing and delivering plasma-derived products
15th International Congress on Neuromuscular Diseases

POSTER SESSIONS

PS1Group1-010 MUSCLE IMAGING AND CLINICAL OUTCOME MEASURES IN OCULOPHARYNGEAL MUSCULAR DYSTROPHY

*H.M.J.M. Kroon*, C.G. Horlings2, J.G. Kalff1, B.G. Van Engelen2; 1Rehabilitation/Nijmegen/NL, 2Neurology/Nijmegen/NL

PS1Group1-011 DIGENIC SQSTM1-TIA1 MYOPATHY: CLINICAL AND PATHOLOGICAL FEATURES


PS1Group1-012 PATHOLOGICAL AND GENETIC STUDIES IN PATIENTS WITH MENDELIAN DISORDERS OF MTDNA MAINTENANCE

*D. Lehmann*1, A.E. Vincent2, H. Rosa2, M. Rocha2, S. Zierz3, R.W. Taylor2, D.M. Turnbull2; 1Department of Neurology/Ulm/DE, 2Institute of Neuroscience/Newcastle Upon Tyne/GB, 3Department of Neurology/Halle/S/DE

PS1Group1-013 EPILEPSY CHARACTERIZATION IN LAM2A-RELATED CONGENITAL MUSCULAR DYSTROPHY


PS1Group1-014 A PHASE 1B/2 STUDY OF THE ANTI-MYOSTATIN ADNETHIN RG6206 (BMS-986089) IN AMBULATORY BOYS WITH DUCHENNE MUSCULAR DYSTROPHY


PS1Group1-015 IN VITRO FUNCTIONAL CHARACTERIZATION OF FKRP PATIENT MISESENSE MUTATIONS

*S.F. Dias Henriques*, E. Giguel, J. Marsolier, I. Richard; /Evry/FR

PS1Group1-016 IDENTIFICATION OF MUTATIONS IN A COHORT OF UNCLASSIFIED INHERITED MUSCLE DISORDERS BY TARGETED NEXT GENERATION SEQUENCING

*K. Polavarapu*1, A. Joshi2, V. Preethish-Kumar3, A. Mathur2, S. Nayak2, S. Ambawat2, S. Nashi4, S. Vengali5, R. Santhosh6, G. Narayanappap6, M. Faruq2, A. Nalini6, 1Clinical Neurosciences, Neurology/Bangalore/IN, 2/New Delhi/IN, 3/Clinical neurosciences, Neurology/Bangalore/IN, 4/Neurology/Bangalore/IN, 5/Neuropathology/Bangalore/IN, 6/Neurology//IN

PS1Group1-017 L-CITRULLINE AND METFORMIN DELAY MUSCLE DEGENERATION IN DUCHENNE MUSCULAR DYSTROPHY: RESULTS FROM A RANDOMISED CLINICAL TRIAL

*P. Hafer*1, 2, U. Bonati1, 3, A. Klein1, 4, D. Rubin1, V. Gocheva1, S. Schmidt1, 3, V. Laugel6, A. Capone7, M. Gloor8, O. Bieri8, T. Zumbrunn9, N. Gueven10, D. Fischer1, 2, 3; 1Division of Paediatric Neurology/Basel/CH, 2Division of Neurology/Bruderholz/CH, 3Department of Neurology/Basel/CH, 4Division of Paediatric Neurology/Berne/CH, 5Division of Paediatric Neurology/Lausanne/CH, 6/Strasbourg/FR, 7Division of Paediatric Neurology/Aarau/CH, 8Department of Radiology/Basel/CH, 9Department of Clinical Research/Basel/CH, 10Pharmacy, School of Medicine/Hobart/AU

PS1Group1-018 MUTATION ANALYSIS IN MLPA NEGATIVE DUCHENNE MUSCULAR DYSTROPHY: NGS AS A DIAGNOSTIC TOOL PRIOR TO MUSCLE BIOPSY

*K. Polavarapu*1, M.K. Saroja2, V. Preethish-Kumar3, D. Sekar4, S. Nalini4, S. Vengali5, P.T. Thomas6, S.N. Rao2, A. Nalini7; 1Clinical Neurosciences, Neurology/Bangalore/IN, 2R&D Division/Bangalore/IN, 3Clinical neurosciences, Neurology/Bangalore/IN, 4/Bengaluru/IN, 5Neurology/Bangalore/IN, 6Department of Psychiatric Social Work/Bangalore/IN, 7Department of Neurology/Bangalore/IN

PS1Group1-019 EZUTROMID SIGNIFICANTLY REDUCES MUSCLE DAMAGE IN DUCHENNE MUSCULAR DYSTROPHY


PS1Group1-020 NUMBER NEEDED TO TREAT IN SPINAL MUSCULAR ATROPHY TYPE 1 WITH AVXS-101 RELATIVE TO NUSINERSEN


PS1Group1-021 A NOVEL NEXT-GENERATION THERAPY FOR POMPE DISEASE WITH IMPROVED EFFICACY IN MICE


PS1Group1-022 THE MULTIPLE FACES OF ANTI-HMGCR ANTIBODY-RELATED MYOPATHIES

*P. Masrori*, W. De Ridder, J. Baets; Neuromuscular Reference Centre, Department of Neurology/Edegem/BE

PS1Group1-023 A PLURIPOTENT STEM CELL-DERIVED MODEL OF MEROSIN DEFICIENT CONGENITAL MUSCULAR DYSTROPHY

K. Lyon1, A. Rickard2, M. Hayhurst Bennet1, *U. Schmidt*2; 1College of Engineering/San Luis Obispo/US, 2/San Diego, CA/US
PS1Group1-024  AN IN VITRO MODEL OF MYOTONIC DYSTROPHY TYPE 1 USING HUMAN EMBRYONIC STEM CELL-DERIVED SKELETAL MUSCLE
E. Solana-Guizar1, S. Labarge2, M. Hayhurst Bennett2, *U. Schmidt*2; 1/San Marcos, CA/US, 2/San Diego, CA/US

PS1Group1-025  DIMETHYLFUMARATE (DMF) DOWN-MODULATES INFLAMMATION IN SKELETAL MUSCLE
*K. Schmidt*1, S. Kaur1, A. Faust1, P. Balcarek2, J. Schmidt1; 1/Department of Neurology/Göttingen/DE, 2/Department of Trauma Surgery, Orthopaedics and Plastic Surgery/Göttingen/DE

PS1Group1-026  TAMOXIFEN IN DMD: RATIONAL AND PROTOCOL FOR A MULTICENTRE, RANDOMISED, DOUBLE-BLIND, PLACEBO-CONTROLLED, PHASE 3 TRIAL

PS1Group1-027  MUSCLE HISTOPATHOLOGY IN INFANTILE DNM1L-RELATED MITOCHONDRIAL EPILEPTIC ENCEPHALOPATHY IS KEY FOR CLINICAL DIAGNOSIS
*E. Bertini*1, D. Verrigni1, D. Battaglia2, L. Fusco3, L. Fighia Talamancac4, R. Carrozzi1, D. Diodato1, A. D’Amico1, L. Papetti1, D. Ghezzif5, C. Lamperti5; 1/Unit of Neuromuscular Disorders, Laboratory of Molecular Medicine/Rome/IT, 2/Institute of Pathology Medicina/Foggia/IT, 3/Department of Molecular Neurogenetics/Milano/IT

PS1Group1-028  EFFECT OF A LONG-TERM TREATMENT WITH METFORMIN IN DYSTROPHIC MDX MICE: A RECONSIDERATION OF ITS THERAPEUTIC INTEREST IN DMD
P. Mantuano1, R.F. Capogrossoi, F. Sanarica1, M.G. Morgese2, M. De Bellisi1, A. Cozzoli1, A. Fonzino1, E. Con-tel1, G.M. Camerino1, L. Trabace2, *A. De Luca*1; 1/Pharmacy - Drug Sciences/Bari/IT, 2/Experimental and Clinical Medicine/Foggia/IT

PS1Group1-029  IDENTIFICATION OF LATE-ONSET CELL-DERIVED SKELETAL MUSCLE

PS1Group1-030  CLINICAL COURSE OF ADULT POMPE DISEASE PATIENTS WHO DID NOT START OR DISCONTINUED ENZYME REPLACEMENT THERAPY
*H.A. Van Kooten*1, L. Harlaar1, N.A.M.E. Van Der Beek1, P.A. Van Doorn1, A.T. Van Der Ploeg2, E. Brussel1; 1/Department of Neurology, Center for Lysosomal and Metabolic Diseases/Rotterdam/NL, 2/Department of Paediatrics, Center for Lysosomal and Metabolic Diseases/Rotterdam/NL

PS1Group1-031  PROLONGED EXERCISE TEST IN PATIENTS WITH HISTORY OF THYROTOXICOSIS
*C.-Y. Tan*, H.-T. Tan, R.J. Ratnasingam, K.-J. Goh; /Kuala Lumpur/MY

PS1Group1-032  CLINICAL AND HISTOPATHOLOGICAL FINDINGS IN MYOTONIC MUSCULAR DYSTROPHY TYPE 2: RETROSPECTIVE REVIEW OF 50 DNA-CONFIRMED CASES
*B. Roy*1, Q. Wu2, C. Whitaker3, K. Felice3; 1/Neurology/New Haven, CT/US, 2/Pathology/Farmington, CT/US, 3/Neurology/New Britain, CT/US

PS1Group1-033  ASCERTAINMENT OF THE ADULT PATIENT COHORT WITH MITOCHONDRIAL DISEASE IN GLASGOW

PS1Group1-034  RESTING-STATE FMRI SHOWS ALTERED DEFAULT-MODE NETWORK FUNCTIONAL CONNECTIVITY IN DUCHENNE MUSCULAR DYSTROPHY PATIENTS

PS1Group1-035  SPECIFIC MUTATIONS IN MYBPC1 CAUSE MYOPATHY AND “MYOGENIC TREMOR”
POSTER SESSIONS

PS1Group1-036 DIAGNOSTIC APPROACH TO CHRONIC PROGRESSIVE EXTERNAL OPHTHALMOPLEGIA – FROM CLINICAL EVALUATION TO GENETIC CONFIRMATION
*B. Kierdaszuk*1, M. Kaliszewska2, K. Tonska2, E. Bartnik2, A.M. Kaminski1, A. Kostera-Pruszczyk1; 1Department of Neurology/Warsaw/PL, 2Institute of Genetics and Biotechnology, Faculty of Biology/Warsaw/PL

PS1Group1-037 FEMALE FERTILITY IN MYOTONIC DYSTROPHY TYPE 1 AND 2
*O. Parmova*1, I. Srotova1, M. Hulova1, E. Vlckova1, L. Mensova2, M. Podborska3, P. Stradalova1, E. Kralickova1, I. Crra4, R. Mazanec2, S. Vohanka1, J. Bednarik1; 1Neurology/Brno/CZ, 2Neurology/Prague/CZ, 3Clinical Biochemistry/Brno/CZ, 4Obstetrics and Gynecology/Brno/CZ

PS1Group1-038 EFFECT OF ANHK, A NOVEL MODULATOR OF RYANODINE RECEPTORS, IN DUCHENNE MUSCULAR DYSTROPHY

PS1Group1-039 ASPIRO PHASE 1/2 GENE THERAPY TRIAL IN X-LINKED MYOTUBULAR MYOPATHY (XLMTMM): PRELIMINARY SAFETY AND EFFICACY FINDINGS

PS1Group1-040 MR IMAGING OF RESPIRATORY MUSCLE DYSFUNCTION IN POMPE DISEASE
*L. Harlaar*1, P. Ciet2, A. Pittaro2, P.A. Sanders, D. Frank; /Cambridge, MA/US

PS1Group1-041 A CASE OF VLCAD DEFICIENCY MYOPATHY WITH NEW MUTATION AND FAVORABLE RESPONSE TO L-CARNITINE, RIBOFLAVIN, AND COQ10
*F. Fatehi*1, Y. Nilipour2, S. Nafissi2; 1Neurology/Tehran/IR, 2Pathology/Tehran/IR

PS1Group1-042 IMPAIRED INSULIN SIGNALLING IN SKELETAL MUSCLE OF MYOTONIC DYSTROPHY PATIENTS
*L.V. Renna*1, F. Bose1, E. Brigonzi2, G. Meola2, R. Cardani1; 1Laboratory of Muscle Histopathology and Molecular Biology/San Donato Milanese [mil]/IT, 2Department of Biomedical Sciences for Health/San Donato Milanese [mil]/IT

PS1Group1-043 DISTAL MYOPATHIES WITH RIMMED VACUOLE IN IRAN, A CLINICAL, HISTOPATHOLOGICAL AND GENETIC REPORT OF A LARGE GROUP
*Y. Nilipour*1, S. Nafissi2, F. Fatehi2, Y. Ashoorian1, N. Beladi Moghadam2, R. Roostani3, M. Rohani2, B. Haghj Ashtiani2, B. Zamani2, 4, K. Basiri5, F. Ashtari6, D. Fathi2, H. Shamshiri2; 1Pathology/Tehran/IR, 2Neurology/Tehran/IR, 3Neurology/Mashhad/IR, 4/Tehran/IR, 5/Isfahan/IR, 6Neurology/Isfahan/IR

PS1Group1-044 GMPPB HOMOZYGOUS VARIANT IN ADULT ONSET LIMB GIRDLE MYASTHENIC SYNDROME: A LIKELY FOUNDER MUTATION
*V. Preethish-Kumar*1, A. Töpf2, K. Polavarapu1, A. Joshi3, S. Balaraju4, A. Roos2, R. Horvat2, S. Nashi5, S. Vengalil6, A. Mathur3, S. Nayak3, S. Ambawat3, M. Faruq3, A. Nalini6, H. Lochmüller7; 1Clinical Neurosciences, Neurology/Bengaluru/IN, 2John Walton muscular dystrophy research center/Newcastle/GB, 3/New Delhi/IN, 4John Walton Muscular Dystrophy Research Center/Newcastle/GB, 5Neurology/Bangalore/IN, 6Neurology/Bengaluru/IN, 7Department of Neuropediatrics and Muscle Disorders/Freiburg/DE

PS1Group1-045 NFAT5 AND P38 MAPKS INTERACT IN MUSCLE CELLS RESPONDING TO OSMOTIC AND INFLAMMATORY STRESS AND IN POLYMYSITIS

PS1Group1-046 IMPLICATION OF THE BREAKPOINTS POSITION IN PATIENTS WITH THE MACRODELETION OF EXONS 45 TO 55

PS1Group1-047 THE STUDY OF ALISKIREN IN MDX DYSTROPHIC MICE
T.A. Marin1, B.M. Bertassoii1, G. Petri1, J.F. Laporte2, L. Scapozza1, *E. Gayi*1, L.A. Neff1, H.M. Ismail1, X. Massa

PS1Group1-048 DUCHENNE MUSCULAR DYSTROPHY: DO BOYS WITH A SHORTER STATURE MAINTAIN AMBULATION LONGER?
L. Labove1, M. Jaworski1, C.-T.E. Nguyen2; 1/Montreal, QC/CA, 2Pediatric Neurology/Montreal, QC/CA

PS1Group1-049 LOW LEVELS OF DYSTROPHIN PROTEIN ASSOCIATED WITH ATTENUATION OF DUCHENNE MUSCULAR DYSTROPHY PROGRESSION

PS1Group1-050 TAMOXIFEN PROLONGS SURVIVAL, IMPROVES MOTOR FUNCTION AND REDUCES LEVELS OF DNM2 IN MTM1-NULL MICE, A MODEL OF XLCNM
*E. Gayi*1, L.A. Neff1, H.M. Ismail, X. Massa

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Bringing hope and more to patients with refractory gMG

Alexion is a global biopharmaceutical company developing life-changing therapies for patients with rare diseases.¹

For more than 20 years, Alexion has been the global leader in complement inhibition.² Over the last decade our terminal complement inhibitor has been an approved treatment for PNH and aHUS.²³ Today, we’re making breakthroughs in neurology with the approval of our complement inhibitor technology as a targeted treatment for anti-AChR+ refractory gMG.³ We are proud to join with the neurology community as we strive to bring hope to patients through persistence, dedication, and a relentless pursuit of the highest levels of medical innovation.

Abbreviations: aHUS, atypical hemolytic uremic syndrome; anti-AChR+, anti-acetylcholine receptor antibody–positive; gMG, generalized myasthenia gravis; PNH, paroxysmal nocturnal hemoglobinuria.


Visit Alexion at Booth 3 to learn more about our targeted treatment for anti-AChR+ refractory gMG, a devastating disease with life-threatening consequences.⁴⁵
PS1Group1-051  ARE THERE DIFFERENT CLINICAL ENTITIES WITH DISTINCT DISEASE COURSE AMONG D4Z4 REDUCED ALLELE CARRIERS?  
*G. Ricci*1, F. Mele2, L. Ruggiero3, E. Buccii4, L. Maggi5, M. Govi6, F. Serad6, L. Vercelli7, L. Santoro3, T. Mongini7, L. Villa8, M. Moggi8, M. Filotost9, M. Scarlato10, S.C. Prevaltali10, M. Cao11, E. Pegoraro11, 12, R. Telesei13, A. Di Muzio13, C. Rodolico14, G. Antonini15, M.G. D’Angelo15, A. Berardinelli16, R. Piras17, M.A. Maioli17, G. Tomelleri2, C. Angelini18, G. Siciliano1, R. Tupilr2; 1Department of Clinical and Experimental Medicine/Pisa/IT, 2Department of Life Sciences/Modena/IT, 3Department of Neurosciences, Reproductive and Odontostomatological Sciences/Naples/IT, 4Department of Neuroscience, Mental Health and Sensory Organs, S. Andrea Hospital/Rome/IT, 5/ Milan/IT, 6MRC Centre for Child Health/ London/GB, 7Department of Neuroscience, Center for Neuromuscular Diseases/Turin/IT, 8Fondazione IRCCS Ca’ Granda Ospedale Maggiore Polclinico, Dino Ferrari Center/Milan/IT, 9Neurology Clinic, Spedali Civili Hospital/ Brescia/IT, 10INNSPE and Division of Neuroscience/Milan/ IT, 11Department of Neurosciences/Padova/IT, 12/Padova/ IT, 13Center for Neuromuscular Disease, CeSi/Chieli/IT, 14Department of Neurosciences, Policlinico “G. Martino”,/ Messina/IT, 15Department of Neu rorehabilitation/Bosiso Parini/IT, 16Unit of Child Neurology and Psychiatry/Pavia/ IT, 17/Cagliari/IT, 18/Venice/IT

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PS1Group1-057  IMPACT OF IDEBENONE ON RESPIRATORY BURDEN, INCLUDING RISK OF BRONCHOPULMONARY COMPLICATIONS, IN DUCHENNE MUSCULAR DYSTROPHY  

PS1Group1-058  CLINICAL AND MOLECULAR FEATURES OF DISTAL MYOPATHIES IN A PORTUGUESE COHORT: REPORTING NOVEL GENE MUTATIONS  
*C. Falcão De Campos*, M. Oliveira Santos, I. Conceição, M. De Carvalho; /Lisbon/PT

PS1Group1-059  DUCHENNE MUSCULAR DYSTROPHY AND THE HEART - HOW TO VISUALIZE BETTER? CASE SERIES REPORT  

PS1Group1-060  RATIONALE FOR EDASALONEXENT DOSE SCHEDULE IN PHASE 2 OF THE MOVEDMD® TRIAL  

PS1Group1-061  REPURPOSING TAMOXIFEN FOR SEVERE MYOPATHIES: FROM PRECLINICAL EVALUATION IN ANIMAL MODELS TO CLINICAL TRIALS IN PATIENTS  

PS1Group1-062  THE CLINICAL VARIATION OF RYR1 GENE IN A LARGE FAMILY  
*S. Jankelowitz*; Central Clinical School/Sydney/AU

PS1Group1-063  CLINICAL OUTCOME STUDY OF DYSFERLINOPATHY: WHAT ARE THE BEST FUNCTIONAL AND STRENGTH OUTCOME MEASURES FOR THIS POPULATION?  
PS1Group1-064 BREATHING IS LIFE! INSPIRATORY MUSCLE TRAINING IN CHILDREN AND ADOLESCENTS LIVING WITH NEUROMUSCULAR DISEASES
*A. Human*1, J. Jelsma2, B.M. Morrow3; 1Physiotherapy/Garankuwa/ZA, 2Division Physiotherapy/Observatory/ZA, 3Paediatrics and Child Health/Rondebosch/ZA

PS1Group1-065 PERSONALIZED MOLECULAR THERAPY IN A NEW TRANSLATIONAL LARGE ANIMAL MODEL FOR DUCHENNE MUSCULAR DYSTROPHY
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PS1Group1-066 DILATED CARDIOMYOPATHY AND LIMB-GIRDLE MUSCULAR DYSTROPHY-DYSTROGLYCANOPATHY REVEALING NEW DPM3 GENE MUTATIONS
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PS1Group1-067 DYSFERLINOPATHY: PHENOTYPIC ASPECTS IN 24 MOROCCAN PATIENTS AND BENEFITS FROM MUSCLE EXERCISE AND CORTICOIDS IN SOME CASES

PS1Group1-068 LGMD DUE TO γ-SARCOGLYCAN DEFICIENCY (LGMD2C): STUDY OF NATURAL HISTORY IN 77 PATIENTS BELONGING TO 69 MOROCCAN FAMILIES

PS1Group1-069 DOES A NORMAL DYSTROPHIN STAINING IN MUSCLE BIOPSY RULE1 OUT THE MOLECULAR DIAGNOSIS OF DUCHENNE / BECKER MUSCULAR DYSTROPHY?
*M. Ginzberg*, T. Lerman-Sagie, D. Lev, R. Dabby, M. Sadeh, E. Leshinsky-Silver; /Holon/IL

PS1Group1-070 ESYN- AND CK9- PROMOTORS DRIVE MUSCLE-SPECIFIC EXPRESSION OF TRANSGENES IN VITRO AND IN VIVO
*J. Meng*, J. Counsell, S. Waddington, F. Muntoni, J. Morgan; /London/GB

PS1Group1-071 E-CADHERIN IS ECTOPICALLY EXPRESSED IN THE MUSCLE FIBER OF INCLUSION BODY MYOSITIS
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PS1Group1-072 CARE EVALUATION OF DUCHENNE MUSCULAR DYSTROPHY PATIENTS IN BRAZIL
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PS1Group1-073 GENOTYPE AND PHENOTYPE FEATURES OF BRAZILIAN PATIENTS WITH MCARDLE DISEASE

PS1Group1-074 PROPOSED CUT-OFF FOR REACTIVITY OF ANTI-HMGCR AND ANTI-SRP ANTIBODIES IN PATIENTS STATIN-EXPOSED AND STATIN-UNEXPOSED
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PS1Group1-075 NECROTIZING MYOPATHY AFTER DENGUE: CASE REPORT
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PS1Group1-076 EXPRESSION OF DYSTROPHIN ISOSFORMS IN NEW DUCHENNE MUSCULAR DYSTROPHY?
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PS1Group1-077 PERSONALIZED MOLECULAR THERAPY IN A NEW TRANSLATIONAL LARGE ANIMAL MODEL FOR DUCHENNE MUSCULAR DYSTROPHY
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PS1Group1-078 DILATED CARDIOMYOPATHY AND LIMB-GIRDLE MUSCULAR DYSTROPHY-DYSTROGLYCANOPATHY REVEALING NEW DPM3 GENE MUTATIONS
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PS1Group1-079 DYSFERLINOPATHY: PHENOTYPIC ASPECTS IN 24 MOROCCAN PATIENTS AND BENEFITS FROM MUSCLE EXERCISE AND CORTICOIDS IN SOME CASES

PS1Group1-080 LGMD DUE TO γ-SARCOGLYCAN DEFICIENCY (LGMD2C): STUDY OF NATURAL HISTORY IN 77 PATIENTS BELONGING TO 69 MOROCCAN FAMILIES
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PS1Group1-077 MYOTONIC DYSTROPHY TYPE 2 AS A MULTISYSTEM DISEASE

PS1Group1-078 HEART INVOLVEMENT IN MYOTONIC DYSTROPHY TYPE 2

PS1Group1-079 VARIANT REPEATS STABILIZE EXPANSION AND MODIFY AGE AT ONSET IN MYOTONIC DYSTROPHY TYPE 1
J. Pesovic, *S. Peric*, M. Brkusanic, G. Brajuskovic, V. Rakocevic-Stojanovic, D. Savic-Pavicевич; /Belgrade/RS

PS1Group1-080 FEATURES OF THE SERBIAN COHORT OF PATIENTS WITH CALPAINOPATHY
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PS1Group1-081 MALIGNANT HYPERTERMIA AND MHLIKE REACTIONS IN NEUROMUSCULAR DISEASES
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PS1Group1-082 ORO-PHARYNGEAL DYSPHAGIA IN MYOTONIC DYSTROPHY TYPE 1 (DM1): IDENTIFICATION OF SENSORY CHANGES IMPACTING SWALLOWING FUNCTION
*J.E. Allen*1, C. Turner2; 1Therapy and Rehabilitation /3bg/GB, 2Institute of Neurology/Bg/GB

PS1Group1-083 HMGCR-MYOPATHY. A RARE BUT STILL A SEVERE DISEASE
*J.M. Grau*1, P.J. Moreno1, S. Prieto-González1, A. Selva-O’Callaghan1, O. Viñas2, J.C. Milisenda1; 1Internal Medicine/Barcelona/ES, 2Immunology/Barcelona/ES

PS1Group1-084 VALUE OF DIAGNOSTIC ALGORITHM IN ASYMPTOMATIC HYPER-CK-EMIA AND ITERATIVE Rhabdomolysis
*J.M. Grau*1, J.C. Milisenda1, F. Cardellach1, J. Garcia2; 1Internal Medicine/Barcelona/ES, 2Biochemistry/Barcelona/ES

PS1Group1-085 NEXT GENERATION SEQUENCING OF DYSTROPHIN GENE IN A COHORT OF NON-DELETION/DUPLICATION DMD/BMD EGYPTIAN PATIENTS

PS1Group1-086 NATURAL HISTORY OF DISEASE-RELATED COMPLICATIONS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
J. Lynch, K. Tsai, J. Lu, C. Mix, *A.M. York*; /Cambridge, MA/US

PS1Group1-087 GOLDIRESN LEADS TO SARCOLEM-MAL DYSTROPHIN EXPRESSION IN PATIENTS WITH GENETIC MUTATIONS AMENABLE TO EXON 53 SKIPPING

PS1Group1-088 POMPE DISEASE IN FRANCE: MOLECULAR FEATURES, EPIDEMIOLOGY AND CLINICAL CORRELATIONS FROM A FOURTY-FIVE YEAR NATIONWIDE STUDY

PS1Group1-089 A SPANISH MYOTONIC DYSTROPHY TYPE I FAMILY CARRYING INTERRUPTIONS SHOWING A MILDERS AND ATYPICAL PHENOTYPE

PS1Group1-090 MERRF CLASSIFICATION: IMPLICATIONS FOR DIAGNOSIS, AND CLINICAL TRIALS
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PS1Group1-091 MITOCHONDRIAL MULTI-ORGAN DISORDER SYNDROME SCORE GENERATED FROM DEFINITE MITOCHONDRIAL DISORDERS
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PS1Group1-092 CLINICAL PRACTICE WITH STEROID THERAPY FOR DUCHENNE MUSCULAR DYSTROPHY, A CLINICIAN SURVEY IN ASIAN AND OCEANIA
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PS1Group1-093 EXPERIENCES WITH BARIATRIC SURGERY IN PATIENTS WITH FSHD AND DM1
*N. Voermans*; Neurology/Nijmegen/NL
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PS1Group1-094 AN UNUSUAL PRESENTATION OF GNE MYOPATHY WITH PROMINENT AXIAL MUSCLE WEAKNESS
*J.-M. Park*1, J.-S. Park2; 1Neurology/Gyeongju/KR, 2Department of Neurology/Daeju/KR

PS1Group1-095 HIGHER MRI MUSCLE FAT FRACTION AT SIMILAR AGE IS ASSOCIATED WITH EARLIER LOSS OF AMBULATION IN DUCHENNE MUSCULAR DYSTROPHY

PS1Group1-096 TORA VARIANTS CAUSE A SEVERE ARTHROGRYPOSIS WITH DEVELOPMENTAL DELAY, STRABISMUS AND TREMOR
A. Kariminejad1, M. Dahl-Halvarsson2, G. Ravenscroft3, F. Afroozan1, E. Keshavz4, H. Gouilleese, M. Davis, N. Laing, *H. Tajsharghi*1; 1/Tehran/IR, 2Department of Pathology/Gothenburg/SE, 3Centre for Medical Research/Nedlands/AU, 4Department of Radiology/Tehran/IR

PS1Group1-097 NDUFV3 VARIANTS THAT DISRUPT MITOCHONDRIAL COMPLEX I ASSEMBLY CAUSE ASSOCIATE WITH CAVITATING LEUKOENCEPHALOPATHY
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PS1Group1-098 RESTRICTION ENZYME CLEAVAGE OF PCR PRODUCTS ALLOWS GENOTYPING MDX3CV, MDX4CV AND MDX5CV ALLELES WITHOUT SEQUENCING
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PS1Group1-099 CLINICO-PATHOLOGICAL CORRELATIONS IN IDIOPATHIC INFLAMMATORY MYOPATHIES
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PS1Group1-100 FIRST REPORTED VARIANT AT THE TRIM32 RING DOMAIN IN A PEDIGREE WITH LIMB-GIRDLE MUSCULAR DYSTROPHY AND BARDET-BIELD SYNDROME

PS1Group1-101 MUSCLE SPECIFIC KINASE PROTECTS MDX MOUSE MUSCLES AGAINST ECCENTRIC CONTRACTION-INDUCED LOSS OF STRENGTH
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PS1Group1-102 NOVEL LMNA GENE MUTATION PRESENTING WITH DILATED CARDIOMYOPATHY AND LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 1B
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PS1Group1-103 LONGTERM APPLICATION OF HUMAN IMMUNOGLOBULIN G FOR EXPERIMENTAL TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

PS1Group1-104 BODY COMPOSITION ANALYSIS IN PATIENTS WITH MYOTONIC DYSTROPHY TYPE 1 AND 2
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PS1Group1-105 EVALUATING THE EFFECTS OF BASELINE VARIABLES ON THE RESPIRATORY FUNCTION BENEFIT OF IDEBENONE IN DUCHENNE MUSCULAR DYSTROPHY
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PS1Group1-106 NOVEL RYR-CALSTABIN STABILIZERS WITH THERAPEUTICAL POTENTIAL FOR DUCHENNE MUSCULAR DYSTROPHY

PS1Group1-107 THE EFFECT OF UNCARIA TOMENTOSA IN DIAPHRAGM MUSCLE OF MDX DYSTROPHIC MICE

PS1Group1-108 MYOBLOTS FOR THE EVALUATION OF NEW TREATMENTS IN NEUROMUSCULAR DISORDERS
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PS1Group1-109 PHENOTYPE-GENOTYPE RELATIONS IN FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY TYPE 1
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PS1Group1-110 SCN4A CAN ACT AS MODIFIER GENE IN PATIENTS WITH MYOTONIC DYSTROPHY TYPE 2
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PS1Group1-111 AAV-SERCA2A EXPRESSION AMELIORATED CARDIOMYOPATHY IN THE MDX MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY
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PS1Group1-112 QUANTITATIVE ANALYSIS OF THIGH MUSCLE BUNDLES OF PATIENTS WITH MYOTONIC DYSTROPHY TYPE 1 (DM1), USING CT IMPAIRMENT RATIO
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PS1Group1-113 LIMB-GIRDLE MUSCULAR DYSTROPHY IN THE CZECH REPUBLIC
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PS1Group1-114 PULMONARY FUNCTION IN ADVANCED DUCHENNE MUSCULAR DYSTROPHY: ETENERLIRS-EN-TREATED PATIENTS VERSUS A NATURAL HISTORY COHORT

PS1Group1-115 CLINICAL OUTCOME STUDY OF DYSFERLINOPATHY: TEENAGE EXERCISE AS A POTENTIAL MODIFIER OF DISEASE SEVERITY

PS1Group1-116 GLYCOGENOSIS TYPE V (MCARDLE DISEASE): THERAPY WITH VITAMIN B6
*A. Saak*, H. Reichmann, J. Schaafier; Neurology/Dresden/DE

PS1Group1-117 MASSIVE INCREASE IN CARDIAC TROPONIN T WITHOUT CARDIAC INVOLVEMENT IN NECROTIZING MYOPATHY WITH ANTI-HMGCR-ANTIBODIES
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PS1Group1-118 RESULTS OF NORTH STAR AMBULATORY ASSESSMENTS IN THE ACT DMD TRIAL IN IN PATIENTS WITH NMMD
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PS1Group1-119 NONSENSE AND SINGLE NUCLEOTIDE FRAMESHIFT MUTATIONS IN BECKER MUSCULAR DYSTROPHY
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PS1Group1-120 DEVELOPMENT OF A PROGNOSTIC MODEL FOR 1-YEAR CHANGE IN TIMED 4 STAIR-CLIMB IN DUCHENNE PATIENTS
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PS1Group1-121 AMBULATORY ELECTROCARDIOGRAPHIC LONGITUDINAL STUDY IN THE GRMD DOG MODEL OF DUCHENNE MUSCULAR DYSTROPHY
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PS1Group1-122 MIMICS OF INCLUSION BODY MYOSITIS: CASE PRESENTATIONS AND IDENTIFICATION OF TYPICAL PITFALLS

PS1Group1-123 LONG-TERM PULMONARY FUNCTION IN NON-AMBULATORY PATIENTS WITH NMDMD TREATED WITH ATALUREN

PS1Group1-124 USE OF A >= 5-SECOND THRESHOLD IN BASELINE TIME TO STAND FROM SUPINE TO PREDICT PROGRESSION IN DMD

PS1Group1-125 NOVEL THERAPEUTIC PERSPECTIVES FOR SARCOCYLCANOPATHY, IN VITRO AND IN VIVO STATE OF THE ART
*D. Sandonon*1, R. Sacchetton1, E. Bianchin1, M. Carotton1, M. Soardi1, C. Fecchioni2; 1/Padova/IT, 2/Biomedical science/Padova/IT

PS1Group1-126 ATALUREN IN PATIENTS AGED ≥ 2 TO 15 YEARS WITH DUCHENNE PATIENTS

PS1Group1-127 A FEMALE CARRIER OF BECKER MUSCULAR DYSTROPHY PRESENTING WITH A MYOPATHY WITH PIPESMET CAPILLARIES
*G. Cosentino*1, F. Brighina1, B. Fierro1, L. Piliat1, M. Mirabella2, C. Rodolico3; 1/Palermo/IT, 2/Roma/IT, 3/Messina/IT

PS1Group1-128 URINARY TITIN IS A NON-INVASIVE BIOMARKER TO DIAGNOSE DUCHENNE MUSCULAR DYSTROPHY EVEN IN ADVANCED STAGE

PS1Group1-129 A NOVEL MUSCLE PHENOTYPE IN A PATIENT WITH TROPOMYOSIN-RECEPTOR KINASE-FUSED GENE (TFG) DISEASE
*N.N. Madigan*1, J.A. Tracy1, W.J. Litchy1, Z. Niu2, M. Milone1; 1Neurology/Rochester, MN/US, 2/Laboratory Genetics & Genomics/Rochester, MN/US

PS1Group1-130 GLYCOGEN STORAGE DISEASE TYPE IV PRESENTING AS CONGENITAL MYOPATHY WITH CONTRACTURES AND RIGID SPINE
*M.C. Walter*1, S. Wenninger1, A. Abicht2; 1Dept. of Neurology, Ludwig-Maximilians-University/Munich/DE, 2/Munich/DE

PS1Group1-131 STATIN-ASSOCIATED AUTOIMMUNE NECROTIZING MYOPATHY

PS1Group1-132 PHENOTYPIC HETEROGENEITY IN THREE PATIENTS WITH M.3243A>G MUTATION
*B. Burnyte*,1, K. Grigalioniene1, A. Vaitkevicius2, D. Petroska3, 4, L. Cimbaliustiene1, V. Kucinskas1, A. Utkus1; 1Institute of Biomedical Sciences of the Faculty of Medicine/Vilnius/LT, 2Institute of Clinical Medicine of the Faculty of Medicine/Vilnius/LT, 3Department of Pathology/Vilnius/LT, 4Affiliate of Vilnius University Hospital Santaros Klinikos/Vilnius/LT

PS1Group1-133 DOUBLE TROUBLE: A CHILD WITH DUCHENNE MUSCULAR DYSTROPHY AND NOONAN SYNDROME

PS1Group1-134 THE HETEROZYGOUS R155C VCP MUTATION: TOXIC IN HUMANS, HARMLESS IN MICE
*J.-Y. Shin*1, K.-H. Strucksberg3, C. Berwanger1, M. Türk4, L. Eichinger3; The German Mouse Clinic Consortium5, R. Schröder6; 1Department of Neurology, Heimer Institute for Muscle Research/Bochum/DE, 2Center for Anatomy and Cell Biology/Vienna/AT, 3Department of Pathology/Erlangen/DE, 4Department of Neurology/Erlangen/DE, 5/Munich/DE, 6Neuropathology/Erlangen/DE

PS1Group1-135 ADAPTIVE PROTEIN QUALITY CONTROL RESPONSE IN DESMINOPATHY SKELETAL MUSCLE CELLS AND TISSUE
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PS1Group1-136 PHENOTYPIC HETEROGENEITY IN THREE PATIENTS WITH M.3243A>G MUTATION
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PS1Group1-137 MODEL FOR 1-YEAR CHANGE IN TIMED 4 STAIR-CLIMB IN THE GRMD DOG

PS1Group1-138 NOVEL MUSCLE PHENOTYPE IN A PATIENT WITH TROPOMYOSIN-RECEPTOR KINASE-FUSED GENE (TFG) DISEASE
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PS1Group1-139 STATIN-ASSOCIATED AUTOIMMUNE NECROTIZING MYOPATHY

PS1Group1-140 PHENOTYPIC HETEROGENEITY IN THREE PATIENTS WITH M.3243A>G MUTATION
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PS1Group1-141 THE HETEROZYGOUS R155C VCP MUTATION: TOXIC IN HUMANS, HARMLESS IN MICE
*J.-Y. Shin*1, K.-H. Strucksberg3, C. Berwanger1, M. Türk4, L. Eichinger3; The German Mouse Clinic Consortium5, R. Schröder6; 1Department of Neurology, Heimer Institute for Muscle Research/Bochum/DE, 2Center for Anatomy and Cell Biology/Vienna/AT, 3Institute of Biochemistry, Medical Faculty/Cologne/DE, 4Department of Neurology/Erlangen/DE, 5/Munich/DE, 6Neuropathology/Erlangen/DE
PS1Group1-136  CLINICAL BACKGROUND OF 94 ADULT PATIENTS WHO RECOGNIZED NEMALIN RODS IN MUSCLE TISSUE  
*A. Hashiguchi*1, K. Kodama2, I. Higuchi3, H. Takashima2;  
1Neurology, Neurological Disease Center/Kagoshima City/JP, 2Department of Neurology and Geriatrics/Kagoshima/JP, 3Physical Therapy foundation course/Kagoshima/JP

PS1Group1-137  P62 IMMUNOSTAINING COULD HELP IN DIFFERENTIATING POLYMYSITIS FROM SIBM  
*J.C. Milisneda*1, C. Jou2, I. Pinal-Feranndez3, J.M. Grau4;  
1Internal Medicine/Barcelona/ES, 2/Barcelona/ES, 3National Institute of Arthritis and Musculoskeletal and Skin Diseases/Bethesda, Maryland, AL/US, 4Internal Medicine Service/Barcelona/ES

PS1Group1-138  HOMOZYGOUSITY OF THE AUTOSOMAL DOMINANT VCP P.ARG159H MUTATION  
*W. De Ridder*1, T. Deconinck1, P. De Jonghe1, K. Johnsson2, A. Töpf2, M. Bertoli2, L. Phillips2, D. Macarthur3, J. Baets1;  
1Neurogenetics Group, VIB-UAntwerp Center for Molecular Neurology/Antwerp/BE, 2John Walton Muscular Dystrophy Research Center/Newcastle Upon Tyne/GB, 3/Cambridge, MA/US

PS1Group1-139  GENOTYPE CHARACTERIZATION OF CZECH PATIENTS WITH FACIOSCAPULOHUMERAL DYSTROPHY  
*J. Zidkova*1, S. Vohanka2, R. Mazanec3, L. Fajkusova4;  
1Centre of Molecular Biology and Gene Therapy/Brno/CZ, 2Neurology/Prague/CZ, 3/Brno/CZ

PS1Group1-140  OCULOPHARYNGEAL MUSCLE WEAKNESS AFTER TREATMENT WITH CHECKPOINT INHIBITORS: PATHOLOGY IS BEYOND THE NEUROMUSCULAR JUNCTION  
*A.C. Mehrabyan*1, A. Ahmed2, C. Specht3;  
1Neurology/Chapel Hill, /US, 2Neurology/Hershey, PA/US, 3/Hershey, PA/US

PS1Group1-141  A CASE OF CENTRAL CORE DISEASE WITH NOVEL RYR1 MUTATION IN KOREAN PATIENT  
*J.-Y. Shin*1, C. Huh2, S.H. Ahn3, K.H. Kwun1, J.A. Kim1, A.W. Kim1, Y.-H. Hong2, J.-J. Sung1;  

PS1Group1-142  MYOADENILATE DEAMINASE DEFICIENCY IN PATIENTS WITH MYALGIA  
*G. Siciliano*, C. Simoncini, G. Ricci; Department of Clinical and Experimental Medicine/Pisa/IT

PS1Group1-143  A PHASE IIA STUDY OF TAS-205, A NOVEL INHIBITOR OF HEMATOPOIETIC PROSTAGLANDIN D SYNTHASE, IN DUCHENNE MUSCULAR DYSTROPHY  
*T. Matsumura*1, H. Komaki2, S. Kuru3, T. Nakayama4, S. Takeda5;  

PS1Group1-144  USE OF HUMAN INDUCED PLURIPOTENT STEM CELLS FOR MODELLING SKELETAL MUSCULAR DEFECTS ASSOCIATED TO MYOTONIC DYSTROPHY TYPE 1  
*T. Tahraoui*1, L. Lescuer2, C. Pinset2, X. Nguyen3, D. Furling3, C. Martinat2;  
1-Stem/Corbeil Essonnes/FR, 21-STEM/Corbeil Essonnes/FR, 3Institut de Myologie/Paris/FR

PS1Group1-145  A CASE OF FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY TYPE 2 WITH NOVEL FRAME-SHIFT MUTATION OF SMCHD1 GENE IN KOREA  
*J.H. Lee*1, M.-W. Seong2, C.-S. Ki3, Y.C. Choi1;  
1Department of Neurology/Seoul/KR, 2Department of Laboratory Medicine/Seoul/KR, 3Department of Laboratory Medicine and Genetics/Seoul/KR

PS1Group1-146  GENOTYPE CHARACTERIZATION OF CZECH PATIENTS WITH FACIOSCAPULOHUMERAL DYSTROPHY  
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1Centre of Molecular Biology and Gene Therapy/Brno/CZ, 2Neurology/Prague/CZ, 3/Brno/CZ

PS1Group1-147  CAN NECK FLEXION WEAKNESS PREDICT CHANGES IN SWALLOWING AND COUGH PEAK FLOW IN PATIENTS WITH MYOTONIC DYSTROPHY TYPE 1?  
*C. Massey*1, J.E. Allen2, U. Vivekananda3, N. Nikolenko4, C. Jimenez-Moreno4, C. Turner5;  
1Therapy and Rehabilitation/Bg/GB, 2Therapy and Rehabilitation /3bg/GB, 3/Bu/GB, 4/Bz/GB, 5Institute of Neurology/Bg/GB

PS1Group1-148  THE ADDITIONAL VALUE OF HISTOPATHOLOGICAL FASCIA EXAMINATION IN DIAGNOSING MYOSITIS  
*A. Van Der Kooi*; Neurology/Amsterdam/NL

PS2 GUIDED POSTER SESSION  
SUNDAY, JULY 8, 2018  
17:15 - 18:30  
ROOM Mezzanine Floor Gallery and Foyers

PS2Group3-001  A TALE OF TWO CHARCOT’S JOINTS: LONDON AND DAR-ES-SALAM  
*C.Y. Kok*, H. Manji; MRC Neuromuscular Centre/London/GB

PS2Group3-002  HETEROGENEITY OF THE PATIENT PATHWAY AND CHARACTERISTICS FOR HATTR AMYLOIDOSIS: PERSPECTIVES FROM CENTRAL AND EASTERN EUROPE  
*Y. Parman*1, I. Tournev2, D. Coriu3, M. Arad4, M. Lipowska5, S. Nikitin6, S. Sarafov2, J. Zidar7;  
1/Istanbul/TR, 2/Sofia/BG, 3/Bucharest/RO, 4/Tel-Hashomer/IL, 5/Warsaw/PL, 6/Moscow/RU, 7/Ljubljana/SI

PS2Group3-003  AUTONOMIC AND SENSORY NEUROPATHY: CHALLENGES IN THE ETIOLOGY AND TREATMENT OF A PEDIATRIC CASE  
*M. Sampaio1, C. Garrido1, M. Santos1, *A. Sousa*2, S. Figueiroa1;  
1Neuropediatrics/Porto/PT, 2Neurophysiology/-/PT
A CLINICAL APPROACH IN 
myotonic dystrophies 
and non-dystrophic myotonias

SUNDAY 8 JULY 2018, 07.00–08.00
ROOM BERG, HILTON VIENNA, VIENNA, AUSTRIA

WELCOME

Dear colleagues

I look forward to welcoming you to this Lupin-sponsored educational seminar during which we will discuss the wide range of patient types that experience myotonia, the broad spectrum of the disease, and the challenges in its diagnosis. We will also review different methods of assessing myotonia and their relevance in clinical practice.

Most importantly, we hope to highlight the criticality of myotonia management for the well-being of patients; myotonia can significantly impact a patient’s quality of life, making daily activities extremely challenging. Can you imagine what it feels like to live in fear of simple actions such as shaking another person’s hand or picking up your child? In this seminar, we hope to convey to you not only the clinical science of myotonia, but also the patient’s perspective of living with this debilitating condition.

This promises to be both an engaging and informative seminar.

Giovanni Meola (Italy)
Chair

This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 15th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2018.

Date of preparation: April 2018
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Lup_EMEA_Neuro_NP/2/26-04-2018
PS2Group3-004  EXTENSIVE GENETIC ANALYSIS IN A TAIWANESE COHORT WITH CHARCOT-MARIE-TOOTH DISEASE
K.-P. Lin1, Y.-C. Liao2, *Y.-C. Lee*2; 1/Taipei/TW, 2Neurology/Taipei/TW

PS2Group3-005  IMPACT OF PATISIRAN ON AUTONOMIC NEUROPATHY IN HEREDITARY TRANSTHYRETIN-MEDIATED AMYLOIDOSIS PATIENTS

PS2Group3-006  THE FUNCTIONAL AND STRUCTURAL EVALUATION OF SMALL FIBERS IN ASYMPTOMATIC PATIENTS WITH VAL30MET MUTATION
E. Yildiz1, *C.E. Bekircan-Kurt*1, S. Kocabeyoglu2, F.G. Yildiz1, M. Irkec2, E. Tan1, S. Erdem-Ozdamar1; 1/Neurology/Ankara/TR, 2/Ophtalmology/Ankara/TR

PS2Group3-007  CLINICAL AND ELECTROPHYSIOLOGICAL FEATURES OF AMAN FROM THE SONORAN OUTBREAK
*S.A. Muley*2, S. Ladha; Neurology/Phoenix, AZ/US

PS2Group3-008  METABOLIC SYNDROME COMPONENTS AND NEUROLOGIC OUTCOMES IN A BARIATRIC SURGERY POPULATION

PS2Group3-009  A SUCCESSFUL TREATMENT OF IDIOPATHIC BRACHIAL NEURITIS WITH SONO-GUIDED INJECTION AND LOW DOSE STEROID THERAPY
*C.-H. Kim*; Physical & Rehabilitation Medicine/Inchon/KR

PS2Group3-010  APOLLO PHASE 3 STUDY: IMPACT OF BASELINE NEUROPATHY SEVERITY ON RESPONSE TO PATISIRAN

PS2Group3-011  CLINICAL SPECTRUM OF HEREDITARY SPASTIC PARAPARESIS BY MUTATION IN KIF1A GENE

PS2Group3-012  THE EFFECT OF REHABILITATION ON SLEEP, RESPIRATORY AND LIFE QUALITY
F.M. Sertpoyraz1, *F. Baydan*2, M. Turanoglu1; 1/Physical Therapy and rehabilitation clinic/Izmir/TR, 2/Pediatric Neurology/Izmir/TR

PS2Group3-013  MULTIFOCAL MOTOR NEUROPATHY WITH CONDUCTION BLOCK: A CASE SERIES
*A.A. Alshareef*; Neurology/Jeddah/SA

PS2Group3-014  PHARMACOKINETICS AND SAFETY OF A SELECTIVE ANTIBODY-SCAVENGING GLYCOPOLYMER FOR THE TREATMENT OF ANTI-MAG NEUROPATHY
*D. Demeestere*1, B. Aliu1, W. Heuserman1, B. Ernst1, P. Hänggi2, R. Herrendorff2; 1/Institute of Molecular Pharmacy/Basel/CH, 2/R&D/Basel/CH

PS2Group3-015  A NOVEL MISSENSE MUTATION OF TRANSTHYRETIN CAUSING AMYLOIDOSIS
*H.-C. Chao*1, Y.-T. Liu2, Y.-C. Lee2, K.-P. Lin3; 1/Medicine/Taiyuan/TW, 2/Neurology/Taipei/TW, 3/Taipei/TW

PS2Group3-016  CHRONIC MOTOR AXONAL NEUROPATHIES: A CHALLENGING DIAGNOSIS
*Y. Anziska*1, I. Lasner2; 1/Neurology/Brooklyn, NY/US, 2/Neurology/Brooklyn, AL/US

PS2Group3-017  QUALITY OF LIFE IN A CLINICAL STUDY OF MAINTENANCE TREATMENT OF CIDP WITH IGPR020: THE PATH STUDY

PS2Group3-018  Feasibility of switching from intravenous to subcutaneous Ig therapy in Cidp: Path trial results versus clinical experience
D. Cociol1, E. Peci1, A. Romagnolo1, V. Brii2, N. Van Geloven3, *H.-P. Hartung*4, R.A. Lewis5, G. Sobue6, J.-P. Lawo7, O. Mielke8, B.L. Durn9, D.R. Cornblath9, I. Merkies10, I. Van Schaik11; 1/Turin/IT, 2/Department of Medicine (Neurology)/Toronto/CA, 3/Department of Medical Statistics and Bioinformatics/Leiden/NL, 4/Department of Neurology/Duesseldorf/DE, 5/Department of Neurology/Los Angeles/US, 6/Department of Neurology/Nagoya/JP, 7/Marburg/DE, 8/Fuquay Varina, NC/US, 9/Department of Neurology/Baltimore, MD/US, 10/Department of Neurology/Maastricht/NL, 11/Department of Neurology/Amsterdam/NL

PS2Group3-019  Two novel variants in the scl25a46 gene causing optical atrophy and peripheral neuropathy in czech siblings
*M. Šedivá*1, P. Laššuthová2, J. Kaňáková3, J. Haberlova1, J. Hänger2, R. Herrendorf2; 1/Institute of Molecular Pharmacy/Basel/CH, 2/R&D/Basel/CH

PS2Group3-020  Two novel variants in the scl25a46 gene causing optical atrophy and peripheral neuropathy in czech siblings
*M. Šedivá*1, P. Laššuthová2, J. Kaňáková3, J. Haberlova1, J. Hänger2, R. Herrendorf2; 1/Institute of Molecular Pharmacy/Basel/CH, 2/R&D/Basel/CH
Paediatric Neurology/Prague/CZ, 2Department of Paediatric Neurology, DNA Laboratory/Prague/CZ, 3Paediatric Department/Klatovy/CZ, 4Department of Biology and Medical Genetics/Prague/CZ

PS2Group3-021 GENOME-WIDE DNA METHYLATION PROFILING OF HUMAN DIABETIC PERIPHERAL NEUROPATHY
*E. Feldman*; /Ann Arbor/US

PS2Group3-022 TRANSCRIPTIONAL SIGNATURE OF DIABETIC PERIPHERAL NEUROPATHY CONSERVED ACROSS HUMAN AND MOUSE
*E. Feldman*; /Ann Arbor/US

PS2Group3-023 RESTABILISATION TREATMENT AFTER IVIG WITHDRAWAL IN CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY: THE PATH STUDY RESULTS
O. Mielke1, V. Bri12, N. Van Geloven3, H.-P. Hartung4, R.A. Lewis5, G. Sobue6, J.-P. Lawo1, B.L. Durn7, D.R. Cornblath8, *I. Merkies*9, A. Shebl1, I. Van Schaik10; 1/Marburg/DE, 2Department of Medicine (Neurology)/Toronto/CA, 3Department of Medical Statistics and Bioinformatics/Leiden/NL, 4Department of Neurology/Duesseldorf/DE, 5Department of Neurology/Los Angeles/US, 6Department of Neurology/Nagoya/JP, 7/Fuquay Varina, NC/US, 8Department of Neurology/Baltimore, MD/US, 9Department of Neurology/Maastricht/NL, 10Department of Neurology/Amsterdam/NL

PS2Group3-024 EFFICACY AND SAFETY OF INTRAVENOUS IMMUNOGLOBULIN IGPRO10 IN CIDP: COMBINED ANALYSIS OF THE PRIMA AND PATH STUDIES
*I. Merkies*1, J.-M. Léger2, V. Bri3, N. Van Geloven4, H.-P. Hartung5, R.A. Lewis6, G. Sobue7, J.-P. Lawo8, B.L. Durn9, D.R. Cornblath10, J. De Bleecker11, C. Sommer12, W. Robberecht13, M. Saarela14, J. Kamieniecki15, Z. Stelmasia16, B. Tackenberg17, O. Mielke18; 1Department of Neurology/Maastricht/NL, 2National Referral Center for Neuromuscular Diseases/Paris/FR, 3Department of Medicine (Neurology)/Toronto/CA, 4Department of Medical Statistics and Bioinformatics/Leiden/NL, 5Department of Neurology/Duesseldorf/DE, 6Department of Neurology/Los Angeles/US, 7Department of Neurology/Nagoya/JP, 8/Fuquay Varina, NC/US, 9Department of Neurology/Baltimore, MD/US, 11Neurology/Ghent/BE, 12Neurologische Klinik und Poliklinik/Würzburg/DE, 13/Leuven/BE, 14Department of Neurology/Helsinki/FI, 15/Wrocław/PL, 16/Lublin/PL, 17Department of Neurology/Marburg/DE, 18Department of Neurology/Amsterdam/NL

PS2Group3-025 A FATAL CASE OF HEPATITIS E-ASSOCIATED GUILLAIN-BARRE SYNDROME
*H.M.J. Wong*1, W.S.K. Kwan2, C.Y. Say2, W.Y.W. Wong2; 1Medicine and Geriatrics/Hong Kong/HK, 2Medicine & Geriatrics/Hong Kong/HK

PS2Group3-026 PUTATIVE DIGENIC INHERITANCE OF CHARCOT-MARIE-TOOTH DISEASE INVOLVING TWO NOVEL HETEROZYGOUS MUTATIONS IN MARS AND HARS
*M. Krenn*1, A. Grisold1, G. Zulehner1, J. Rath1, H. Cetin1, M. Tomskich1, B. Zagrovic2, L. Bartonek2, T. Kokotovic3, V. Nagy3, M. Wagner4, T.M. Strom4, F. Zimprich1; 1Department of Neurology/Vienna/AT, 2Department of Structural and Computational Biology, Max F. Perutz Laboratories/Vienna/AT, 3/Vienna/AT, 4Institute of Human Genetics/Munich/DE

PS2Group3-027 A RANDOMIZED, BLINDED, LIFESTYLE INTERVENTION STUDY IMPROVES THE EXPIRATION:INSPIRATION RATIO IN DIABETIC NEUROPATHY
J. Russell1, *L. Zilliox*2, P. Kumar1; 1/Baltimore/US, 2/Baltimore/MD/US

PS2Group3-028 PREDICTORS OF EARLY RETIREMENT IN PATIENTS WITH CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY
B. Bjelica1, I. Bozovic1, I. Bastai1, A. Kacar1, A. Nikolic1, A. Dominovic-Kovacevic2, Z. Vukojevic2, V. Martic1, A. Stojanov3, G. Djordjevic3, M. Petrovic4, M. Stojanovic4, *S. Peric*1; 1/Belgrade/RS, 2/Banja Luka/BA, 3/Nis/RS, 4/Kragujevac/RS

PS2Group3-029 NEUROPATHY AS AN ADVERSE EFFECT OF VASCULAR ENDOTHELIAL GROWTH FACTOR TYROSINE KINASE INHIBITORS, A META-ANALYSIS

PS2Group3-030 AXONAL FUNCTION PREDICTS RESPONSE TO SUBCUTANEOUS IMMUNOGLOBULIN IN CIDP: THE PATH STUDY
*V. Bri*1, H.-P. Hartung2, G. Sobue3, J.-P. Lawo4, O. Mielke4, B.L. Durn5, I. Merkies6; 1Department of Medicine (Neurology)/Toronto/CA, 2Department of Neurology/Duesseldorf/DE, 3Department of Neurology/Nagoya/JP, 4/Marburg/DE, 5/Fuquay Varina, NC/US, 6Department of Neurology/Maastricht/NL

PS2Group3-031 ELECTROPHYSIOLOGICAL TESTING IN PATIENTS WITH CIDP TREATED WITH SUBCUTANEOUS IMMUNOGLOBULIN: THE PATH STUDY
*V. Bri*1, I. Van Schaik2, N. Van Geloven3, H.-P. Hartung4, R.A. Lewis5, G. Sobue6, J.-P. Lawo7, O. Mielke7, B.L. Durn8, I. Merkies9; 1Department of Medicine (Neurology)/Toronto/CA, 2Department of Neurology/Duesseldorf/DE, 3Department of Neurology/Nagoya/JP, 5/Amsterdam/NL, 6Department of Medical Statistics and Bioinformatics/Leiden/NL, 7Department of Neurology/Nagoya/JP, 8/Fuquay Varina, NC/US, 9Department of Neurology/Maastricht/NL

PS2Group3-032 CHRONIC RELAPSING INFLAMMATORY OPTIC NEUROPATHY (CRION): A MANIFESTATION OF MYELIN OLIGODENDROCYTE GLYCOPROTEIN ANTIBODIES

PS2Group3-033 PREVALENCE AND RISK FACTORS OF CARPAL TUNNEL SYNDROME IN OYSTER-SHUCKERS
PS2Group3-034  NEXT GENERATION SEQUENCING TECHNOLOGIES IN THE GENETIC DIAGNOSIS OF EARLY ONSET HEREDITARY SPASTIC PARAPLEGIAS
*L. Carrera-García*1, D. Natera De Benitol1, D. Itzep1, A.L. Frongia1, A. Sariego1, D. Yubero2, E. Maqueda1, L. Martorell3, C. Ortez1, J. Colomer3, G. Stevanin4, A. Nascimento1, 1Neuromuscular Disorders Unit/Barcelona/ES, 2Department of Genetics/Barcelona/ES, 3/Barcelona/ES, 4/Paris/FR

PS2Group3-035  A CASE OF SEROPOSITIVE NEUROMYELITIS OPTICA IN A PATIENT WITH CO-EXISTING MYASTHENIA GRAVIS AND SYSTEMIC LUPUS ERYTHEMATOSUS

PS2Group3-036  NERVE ULTRASOUND ASSESSMENT IN A NOVEL MUTATION C.379DELG (P1A127LEUFS*52) IN DRP2 GENE
*S. Wenninger*1, R. Jankovits2, A. Brucker2, 1Neurology, Ludwig-Maximilians-University/Munich/DE, 2/Prien/DE

PS2Group3-037  PHRENIC NERVE DEMYELINATION CAUSED BY ACUTE RESPIRATORY FAILURE IN MADSAM
C. Baldwini, *K. Ng*2, 1Neurology/Sydney, NSW/AU, 2Neurology/St Leonards, NSW/AU

PS2Group3-038  BACLOFEN, NALTREXONE AND SORBITOL ALL CONTRIBUTE TO THE EFFICACY OF PXT3003 IN CMT1A RATS
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PS2Group3-039  IDEAL TIME FOR A REPEAT CONDUCTION STUDY TO SUBTYPE VARIANTS IN GUILLAIN BARRE SYNDROME
*A.K. Meena*, R. Chepuru, S. Sarva, S. Yareeda, N. Mathukomali, Neurology/Hyderabad/IN

PS2Group3-040  COST OF ILLNESS IN CHARCOT-MARIE-TOOTH NEUROPATHY: RESULTS FROM GERMANY
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PS2Group3-041  CLINICAL HETEROGENEITY OF ANTIGM2-GANGLIOSIDE ANTIBODY SYNDROME

PS2Group3-042  LATE-ONSET AXONAL CHARCOT-MARIE-TOOTH ASSOCIATED WITH AUTOSOMAL RECESSIVE MME MUTATIONS

PS2Group3-043  POPULATION PHARMACOKINETICS (PK) OF INVESTIGATIONAL PATISIRAN IN HEALTHY VOLUNTEERS AND IN PATIENTS

PS2Group3-044  CHARACTERIZATION OF NEURONAL MOLECULAR MECHANISMS UNDERLYING CMT2Z NEUROPATHY
P. Sancho1, L. Bartesaghi2, O. Miossec2, F. García-García3, L. Ramírez-Jiménez1, *V. Lupo*1, R. Chrast2, C. Espinós1, 1Unit of Genetics and Genomics of Neuromuscular and Neurodegenerative Disorders and Service of Genomics and Translational Genetics/Valencia/ES, 22Department of Neuroscience and Department of Clinical Neuroscience/Stockholm/SE, 3/Unidad de Bioinformática y Bioestadística/Valencia/ES

PS2Group3-045  INCREASED DEACETYLATION OF PROTEINS BY SIRTUIN 1 PROTEIN OVER EXPRESSION REVERSES T2D PERIPHERAL NEUROPATHY
*J. Russell*, K. Chandrasekaran, C.-Y. Ho, P. Kumar, S.S. Reddy; /Baltimore/US

PS2Group3-046  PGC-1α REGULATES NEURONAL RESPIRATORY CHAIN SUPERCOMPLEXES IN MITOCHONDRIA

PS2Group3-047  THE VALIDITY OF SUDOSCAN IN SCREENING OF UPPER EXTREMITY NEUROMYCETOPLASIES: A PILOT STUDY
*G.-Y. Park*, S. Im, H.J. Koo, Y. Jang, C.S. Chae; Rehabilitation Medicine/Bucheon-si/KR

PS2Group3-048  IPSC MOTOR NEURONS FROM AN X-LINKED CMT TYPE 6 PATIENT AS A MODEL FOR STUDYING AXONAL DEGENERATION AND DEVELOPING THERAPIES
*G. Perez Siles*1, R. Screnci2, A. Cutrupi1, M. Ellis1, C. Ly1, G.A. Nicholson3, M.L. Kennerson1; 1/Notthcott Laboratory (Neurobiology)/Sydney, NSW/AU, 2School of Life Sciences/Sydney, NSW/AU, 3Concord Hospital/Sydney, NSW/AU

PS2Group3-049  PARANODAL ANTIBODIES IN AUSTRIAN PATIENTS WITH ACUTE ONSET INFLAMMATORY NEUROPATHY

PS2Group3-050  ULNAR NERVE CONDUCTION STUDY USING THE FIRST DORSAL INTEROSSEOUS MUSCLE RESPONSE AS A MARKER TO SCREEN FOR PERIPHERAL NEUROPATHY IN HEALTHY KOREAN SUBJECTS

PS2Group3-051  PATIENT ASSISTED INTERVENTION FOR NEUROPATHY: COMPARISON OF TREATMENT IN REAL LIFE SITUATIONS (PAIN-CONTROLS)
*L. Carrera-García*1, D. Natera De Benitol1, D. Itzep1, A.L. Frongia1, A. Sariego1, D. Yubero2, E. Maqueda1, L. Martorell3, C. Ortez1, J. Colomer3, G. Stevanin4, A. Nascimento1, 1Neuromuscular Disorders Unit/Barcelona/ES, 2Department of Genetics/Barcelona/ES, 3/Barcelona/ES, 4/Paris/FR
*R. J. Barohn*1, B. Gajewski2, M. Pasnoor1, L. Brown2, L. Herbelin1, K. Kimminau3, O. Jawdat1, T. Liu1, C. Park1, P. Shlemon4, M. Dimachkie1, P. Pain-CONTROL’S Study Team1; 1Neurology/Kansas City, KS/US, 2Department of Biostatistics/Kansas City/US, 3Family Medicine/Kansas City/US, 4/Kansas City/US

**PS2Group3-052**  A NEW MUTATION IN MORC 2 CAUSES SCAPULOPERONEAL CHARCOT-MARIE-TOOTH NEUROPATHY: DAVIDENKOW SYNDROME

**PS2Group3-053**  AUTOPHAGIC NEUROMYOPATHY CAUSED BY P.R140G HEAT SHOCK PROTEIN B1 (HSPB1) MUTATION

**PS2Group3-054**  HEREDITARY TRANSTHYRETIN AMYLOIDODIS evaluable with the SCARE-01, 2Department of Neurology/Fuquay Varina, NC/US, 3Department of Neurology/Baltimore, MD/US, 4Department of Neurology/Maastricht/NL, 5Department of Neurology/Nagoya/JP, 6Department of Neurology/Baltimore, MD/US, 7Department of Neurology/Amsterdam/NL

**PS2Group3-058**  EPIDEMIOLOGY OF HEREDITARY MOTOR-SENSORY NEUROPATHIES IN THE POPULATION OF THE REPUBLIC OF BASHKORTOSTAN
*E. Saifullina*1, R. Magzhanoov2, I. Khidiatyova2, E. Khusnutdinova2; 1Neurology and Medical Genetics/Ufa/RU, 2/Ufa/RU

**PS2Group3-059**  INFLUENCE OF THE EXTRACT OF SUCUPIRA BRANCA (PTERODON EMARGINATUS VOGEL) IN SURAL NERVE OF DIABETIC RATS

**PS2Group3-060**  EXPANDING THE PHENOTYPIC SPECTRUM OF GARS MUTATIONS
*G. Ricci*, V. Boczonadi, B. Bansagi, R. Horvath; Institute of Genetic Medicine/Newcastle/GB

**PS2Group3-061**  REDDISH SKIN COLOR CHANGE IN A PATIENT WITH COMPRRESSIVE RADIAL NEUROPATHY

**PS2Group3-062**  UNCLASSIFIED CONGENITAL AXONAL NEUROPATHY IN GIPSY FAMILIES IN PORTUGAL
*T. Moreno*1, R. Siva1, I. Conceição3, J. Castro3, I.D. Castro3, F. Furtado4, O. Moldovan2; 1Unidade de Neuropediatrics/Lisboa/PT, 2Servico Genetica/Lisbon/PT, 3Laboratorio Neurofisiologia/Lisboa/PT, 4Servicio de Pediatría/Beja/PT

**PS2Group3-063**  ETIOLOGY AND OUTCOME IN NEUROMUSCULAR PATIENTS PRIMARILY PRESENTING WITH DIAPHRAGMATIC DYSFUNCTION
*M. Türk*1, I. Weber2, G. Vogt-Ladner2, R. Schröder3, M. Winterholler2; 1Department of Neurology/Erlangen/DE, 2Department of Neurology/Schwarzendbruck/DE, 3Neuropathology/Erlangen/DE

**PS2Group3-064**  IQYMUNE® IS EFFECTIVE AS MAINTENANCE TREATMENT FOR MMN: A RANDOMISED, DOUBLE-BLIND, CROSS-OVER STUDY Versus KIOVIG®
R. Ouaja1, O. Alfa Cissé2, E. Nobile-Orazio3, *J.-M. Léger*4; 1Immunology Franchise/Les Ulis/FR, 2Global Medical Affairs Unit/Les Ulis/FR, 3Neurology/Santo Andre/BR

**PS2Group3-065**  ANTI-MAG TITERS PRE/POST DEGLYCOXYLATION IN SURAL NERVE OF DIABETIC RATS
*S. Attarian*3, J. Boucraut3, *J.-M. Léger*1; 1Immunology Franchise/Les Ulis/FR, 2Global Medical Affairs Unit/Les Ulis/FR, 3Neurology/Santo Andre/BR

**PS2Group3-066**  ULTRASONOGRAPHY IS USEFUL IN EVALUATION FOR ULNAR NEUROPATHIES WITH NO LOCALIZATION IN ELECTROPHYSIOLOGICAL STUDIES
*J.Y. An*1, D.W. Bae2; 1Department of Neurology/Suwon/KR, 2/Suwon/KR

**PS2Group3-067**  CLINICAL AND GENETIC ANALYSIS OF HEREDITARY PERIPHERAL NEUROPATHY IN EGYPTIAN POPULATION
*E. Saifullina*1, R. Magzhanoov2, I. Khidiatyova2, E. Khusnutdinova2; 1Neurology and Medical Genetics/Ufa/RU, 2/Ufa/RU
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**PS2Group3-068** THE USEFULNESS OF NERVE CONDUCTION STUDIES IN CHEIRALGIA PARESTHETICA
*I.S. Joo*; Neurology/Suwon/KR

**PS2Group3-069** BACLOFEN, NALTREXONE AND SORBITOL ALL CONTRIBUTE TO PXT3003-INDUCED MYELINATION IN CMT1A DRG CO-CULTURES
*N. Cholet*1, J. Laffaire2, R. Hajj1, D. Cohen3; 1Experimental Biology/Issy-les-Moulineaux/FR, 2Biostatistics/Issy Les Moulineaux/FR, 3CEDE/Issy-les-Moulineaux/FR

**PS2Group3-070** AN AUTOSOMAL DOMINANT FAMILY WITH UNCOMPROMICATED SPASTIC PARAPLEGIA DUE TO A STOP MUTATION IN HARS

**PS2Group3-071** CONDUCTION STUDIES OF PHRENIC NERVE IN HEALTHY CHILDREN
*V. Voitenkov*1, E. Eksheva2, N. Skripchenko3; 1Clinical Neurophysiology/Saint-Petersburg/RU, 2/Moscow/RU, 3/Saint-Petersburg/RU

**PS2Group3-072** FIBER TRACTOGRAPHY OF FACIAL NERVE: IMPLICATIONS FOR HEMIFACIAL SPASM
*Y.H. Koh*1, L.L. Chan2, E.W. Lim2; 1Neurology/Singapore/SG, 2/Singapore/SG

**PS2Group3-073** NEUROMYOPATHY CAUSED BY LONG TERM COLCHICINE THERAPY
*M.Y. Chun*1, J.H. Lee, K.-D. Park; Neurology/Seoul/KR

**PS2Group3-074** CHARCOT MARIE TOOTH DISEASE TYPE 2CC DUE TO A MUTATION IN THE NEUROFILAMENT-HEAVY GENE IN A GERMAN FAMILY
*E. Ikenberg*1, P. Reilich1, C. Heller2, A. Abicht3, B. Schoser1, M.C. Walter3; 1Friedrich-Baur Institute, Department of Neurology/Munich/DE, 2/Tübingen/DE, 3.Department of Neurology, Ludwig-Maximilians-University/Munich/DE

**PS2Group3-075** ISOLATED ABDUCENS PALSY AS TOLSA-HUNT SYNDROME IN A SYSTEMIC LUPUS ERYTHEMATOSUS PATIENT
*V. Serban*; /Cluj/RO

**PS2Group3-076** MULTIFOCAL MOTOR NEUROPATHY IN AUSTRIA: A NATIONWIDE SURVEY
E.-M. Oberreiter1, S. Quasthoff2, M. Erdler3, R. Topa2, C. Gabrielse1, A. Verhey1, A. Brandstetter1, G. Schreiner1, A. Wilczek1, M. Toth1, M. Doerr1, T. Winter1; 1Department of Neurology and Psychiatry/Austria/EG, 2Department of Molecular Neuroscience/London/GB, 3Department of molecular neuroscience/London/GB, 4Department of neurology and psychiatry/Assiut/EG

**PS2Group3-077** AUTOSOMAL RECESSIVE CHARCOT MARIE TOOTH DISEASE: CLINICAL, ELECTROPHYSIOLOGY AND GENETIC SPECTRUM IN A TUNISIAN SERIES
I. Kacem1, E. Ellouz2, M. Ben Djebara1, E. Leguerre3, A. Gargouri1, *R. Gouider*4; 1/Tunis/ZN, 2/Neurology/Ibn Khaldoun Street Gabes/ZN, 3/Paris/FR, 4/Neurology/Ma-nouba/ZN

**PS2Group3-078** COLCHICINE INDUCED NEUROMYOPATHY IN A PATIENT USING CONCOMITANT DIURETICS
*Y.-D. Kim*; Neurology/Incheon/KR

**PS2Group3-079** A CASE OF MULTIFOCAL MOTOR NEUROPATHY: COMPLEMENTARY ROLE OF ULTRASOUND
*J.I. Suk*1, H.J. Rha2; 1Neurology/Seoul/KR, 2/Seoul/KR

**PS2Group3-080** PHARMACOKINETICS OF PATISIRAN IN PATIENTS WITH HEREDITARY TRANSTHYRETIN-MEDIATED AMYLOIDOSIS
*X. Zhang*1, V. Goel, G. Robbie; /Cambridge, MA/US

**PS2Group3-081** ROLE OF THE ER STRESS TRANSCRIPTION FACTOR XBP1 IN CHARCOT-MARIE-TOOTH DISEASE TYPE 1B
*T. Touvier*1, C. Ferri1, R. Mastrangelo1, C. Rivellini2, L. Gümcher3, L. Wrabetz4, M. D’Anontio1; 1Division of Genetics and Cell Biology/Milan/IT, 2Division of Neuroscience/Milan/IT, 3Department of Cancer Immunology and Virology/Boston, MA/US, 4Hunter James Kelly Research Institute/Buffalo, NY/US

**PS2Group3-082** OBITUTIONUMAB, A NEW ANTI-CD20 ANTIBODY, IS ACTIVE AND EFFECTIVE IN ANTI-MAG ANTIBODY POLYNEUROPATHY
*C. Brian1*, A. Visentini2, A. Salvalaggio1, M. Ruiz1, M. Cacciavillani3; 1Neurology Unit, Department of Neuroscience/Padua/IT, 2Hematology and Clinical Immunology Unit, Department of Medicine/Padua/IT, 3/Padova/IT

**PS2Group5-001** LATE-ONSET POMPE DISEASE ASSOCIATED WITH POLYNEUROPATHY
*M. Lamartine Monteiro*1, G. Remiche2; 1Neurology/Brussels/BE, 2Centre de Référence Neuromusculaire, Service de Neurologie/Brussels/BE

**PS2Group5-002** CORRELATION BETWEEN ELECTRICAL IMPEDANCE MYOGRAPHY AND TWO QUANTITATIVE ULTRASOUND PARAMETERS IN DUCHENNE MUSCULAR DYSTROPHY

**PS2Group5-003** PHYSIOLOGICAL DIFFERENCES IN SARCOLEMMA EXCITABILITY MEASUREMENTS BETWEEN HUMAN MUSCLES
R. Boland-Freitas, J. Lee, *K. Ng*; Neurology/St Leonards/AU
PS2Group5-004  LABEL-FREE IMAGING OF ABNORMAL LIPID ACCUMULATION IN PERIPHERAL NERVES FROM FABRY DISEASE PATIENTS USING RAMAN SPECTROSCOPY
*Y. Nagashima*1, A. Iwata1, K. Yoshioka2, J. Omachi3, J. Shimizu1, T. Tada1, J. Yumoto3, M.K. Gonokami4; 1Department of Neurology/Tokyo/JP, 2School of Engineering/Tokyo/JP, 3School of Science/Tokyo/JP, 4/Tokyo/JP

PS2Group5-005  MODULATING EFFECTS OF UNSATURATED FATTY ACIDS ON GENE EXPRESSION OF MYOSIN HEAVY CHAIN CLASS I AND IIB IN C2C12 MYOCYTES
*J. Yamaji*1, Y. Morii2; 1Department of Nutrition Sciences/Kashiwara/JP, 2Department of Rehabilitation Sciences/Kashiwara/JP

PS2Group5-006  ELECTROPHYSIOLOGICAL DIAGNOSTIC OF NEUROMUSCULAR DISEASES IN NEW-BORNs, INFANTS AND TODDLERS
*P.J. Broser*1, O. Hasselmann2, O. Maier1, J. Lütschg1; 1Neuropediatrics/St Gallen/CH, 2Neuropediatrics/St. Gallen/CH

PS2Group5-007  DISCOVERY OF PROTEIN BIOMARKERS FOR DYSFERLINOPATHY
*L. Rubich*1, Z. Hollander2, 3, D.L. Dai2, 3, V. Chen2, 3, A. Singh2, 3, D. Albrecht1, B. Williams1, H. Windish1, E. Lee1, P. Mittal1, A. Mayhew4, M. Jacobs5, 6, J.W. Day7, K.J. Jones8, D.X. Bharucha-Goebel9, 10, M. Harms11, A. Pestronk11, M.C. Walter12, T. Stojskek13, S. Sparks14, E. Bravver14, J. Diaz-Manera15, 16, E. Pegoraro17, C. Paradis18, J. Mendell19, H. Lochmuller4, K. Busby4, V. Straub4, S. Assadian2, 3, J.E. Wilson-Mcmanus20, D.S. Smith21, C.H. Borchers21, 22, 23, 24, B. McManus2, 3, R. Ng2, 25; 1Seattle, WA/US, 2Vancouver, Bc/CA, 3UBC James Hogg Research Centre/Vancouver, Bc/CA, 4John Walton Muscular Dystrophy Research Centre/Newcastle Upon Tyne/GB, 5Division of Biostatistic and Study Methodology, Children’s National Health System/Dc, 6/Washington/DC, 7Department of Neurology and Neurological Sciences/Stanford, CA/US, 8/institute for Neuroscience and Muscle Research, Children’s Hospital of Westmead/Sydney/AU, 9Department of Neurology/Washington DC/US, 10ININDS/Bethesda/US, 11Department of Neurology/St. Louis, MO/US, 12Friedrich-Baur Institute, Department of Neurology/Munich/DE, 13/Paris/FR, 14/Charlotte, Nc/US, 15/Neuromuscular disorders Unit/Barcelona/ES, 16/Barcelona/ES, 17/Department of Neuroscience/Padova/IT, 18/Neuromuscular Unit, Department of Neurology/Sevilla/ES, 19/Center for Gene Therapy/Columbus, OH/US, 20/NetCAD/Vancouver, Bc/CA, 21/Genome BC Proteomics Centre/Victoria, Bc/CA, 22/Proteomics Centre, Segal Cancer Centre, Lady Davis Institute, Jewish General Hospital/Montreal, QC/CA, 23/Gerald Bronfman Department of Oncology/Montreal, QC/CA, 24/Department of Biochemistry and Microbiology/Victoria, Bc/CA, 25/Department of Computer Science/Vancouver, Bc/CA

PS2Group5-008  NERVE ULTRASOUND FOR THE IDENTIFICATION OF TREATMENT-RESPONSIVE CHRONIC NEUROPATHIES WITHOUT NERVE CONDUCTION ABNORMALITIES
*S. Goedee*1, I. Herrera1, L. Visser2, T. Van Asseldonk2, H. Franssen1, L. Van Der Pol1, L. Van Den Berg1; 1Neurology/Utrecht/NL, 2Neurology/Tilburg/NL

PS2Group5-009  MOTOR-UNIT NUMBER ESTIMATION IN THE ABDUCTOR POLICIS BREVIS MUSCLE OF PATIENTS WITH CARPAL TUNNEL SYNDROME
*B. Haghiashitani*1, F. Akhondi2, Z. Mirza Asgari2, M. Almasi2, B. Zamani2, M.R. Matamed2, M. Mehr Pour2; 1Neurology//IR, 2/Tehran/IR

PS2Group5-010  HIGH RESOLUTION NEUROSONOGRAPHY IN PATIENTS WITH CARPAL TUNNEL SYNDROME AND NORMAL CONTROLS
*A. Nalini*1, S. Nashi2, V. Preethish-Kumar1, N. Yadav3, K. Bhattacharya3, K. Polavarapu1, S. Vengalil1; 1Neurology/Bengaluru/IN, 2/Bengaluru/IN, 3/Neuro Imaging and Interventional Radiology/Bengaluru/IN

PS2Group5-011  CAN WE EARLY DETECT CARDIAC DYSFUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY?
*M. Marchel*, J. Kochanowski, A. Serafin, B. Truszkowska, G. Opolski; Department of Cardiology/Warsaw/PL

PS2Group5-012  PERIPHERAL NERVOUS SYSTEM DISORDERS IN MULTIPLE MYELOMA: RABAT CLINICAL NEUROPHYSIOLOGY DEPARTMENT EXPERIENCE
*L. Belarabi*, N. Birouk, B. Kably, L. Errguig, H. Belaïdi, R. Ouazzani; Clinical Neurophysiology/Rabat/MA

PS2Group5-013  CRAMPS, FASCICULATIONS AND MUSCLE FATIGABILITY DUE TO VITAMIN D DEFICIENCY
*L. Belarabi*, N. Birouk, B. Kably, R. Ouazzani; Clinical Neurophysiology/Rabat/MA

PS2Group5-014  NEXT-GENERATION SEQUENCING FOR MOLECULAR DIAGNOSIS IN NEUROMUSCULAR DISORDERS. RESULTS FROM 151 PATIENTS

PS2Group5-015  TRANSCRANIAL MAGNETIC STIMULATION AS A DIAGNOSTIC AND PROGNOSTIC TOOL IN CHILDREN WITH SEQUELAE OF ACUTE TRANSVERSE MYELITIS
*V. Voitenkov*1, N. Skripchenko2; 1Clinical Neurophysiology/Saint-Petersburg/RU, 2/Saint-Petersburg/RU

PS2Group5-016  TARGETED METHYL-SEQ QUANTIFICATION BY NGS TECHNOLOGY FOR ROUTINE DIAGNOSTIC OF FSHD
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PS2Group5-017  FACIAL NERVE ULTRASOUND IN BELL’S PALSY
*S.-H. Baek*1, Y.-J. Kwon1, Y.H. Kim1, H.Y. Seok2, B.-J. Kim1; 1Department of Neurology/Seoul/KR, 2Department of Neurology/Daejeon/KR

PS2Group5-018  CRGP UPREGULATES MYOSIN HEAVY CHAIN TYPE I MESSENGER RNA THROUGH CALCIUM-NEURIN-IL-6-INDEPENDENT MANNER IN C2C12 CELLS
*Y. Mori*1, J. Yamaji2; 1Department of Rehabilitation Sciences/Kashiwara/JP, 2Department of Nutrition Sciences/Kashiwara/JP

PS2Group5-019  ANALYSIS OF EUROPEAN EARLY-ONSET MYASTHENIA GRAVIS GWAS SIGNALS IN AFRICAN GENOMES
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PS2Group5-020  RNASEQ IN URINARY-DERIVED STEM CELLS IDENTIFIED THE EXPRESSION OF 244 NEUROMUSCULAR GENES
*M. Salzaran1, H. Osman1, R. Rossii1, R. Selvatici1, M. Neri1, F. Gualand1, M. Fang2, Z. Lu2, A. Grilli3, S. Biccia-toti3, A. Ferlini*1; 1Medical Sciences/Ferrara/IT, 2/Shenzhen/CN, 3Dipartimento di Scienze della Vita/Modena/IT

PS2Group5-021  NEUROPATHY IN SPINOCEREBELLAR ATAXIA TYPE 2: A NERVE ULTRASOUND STUDY
*L. Pelosi*1, R. Iodice2, A. Antenora2, D. Kiffoyle1, E. Mullroy1, M. Rodrigues1, R. Roxburgh1, A. Filla2, F. Manganel-li2, L. Santoro1; 1Neurology and Clinical Neurophysiology/Auckland/NZ, 2Neurology/Sydney/NSW/AU, 3Neurology/St Leonards/AU

PS2Group5-022  NERVE ULTRASOUND IN POEMS SYNDROME: SIMILAR TO CIDP
*A.-K. Peyer Kauffmann*1, E. Wilder-Smith2; 1Neurozenology/Busan/KR, 2Neurology/Kashihara/KR

PS2Group5-023  COMPARISON OF HOME-BASED VERsus HOSPITAL-BASED SPIROMETRY MEASUREMENTS IN DUCHENNE MUSCULAR DYSTROPHY

PS2Group5-024  AGE-RELATED DIFFERENCES IN MUSCLE MEMBRANE POLARIZATION AS ASSESSED BY VELOCITY RECOVERY CYCLES
J. Lee1, R. Boland-Freitas2, *K. Ng*3; 1Neurology/Sydney/NSW/AU, 2Neurology/Sydney/NSW/AU, 3Neurology/St Leonards/AU

PS2Group5-025  EFFECTIVE DIAGNOSTIC AND TREATMENT METHODS IN VASCULAR PARKINSONISM AND PARKINSON’S DISEASE: TEMPORARY SYMPTOM CORRECTION
*D.T. Akramova*; Neurology/Tashkent City/UZ

PS2Group5-026  KIF5A, MUTATION CAN LEAD TO SPASTIC PARAPLEGIA (SPG10); GABAARs-VESICLE TRANSPORT MOTOR INTERACTS WITH G3BP2 AND GABARAPS
*D.-H. Seog*1, S. Kim2; 1Biochemistry/Busan/KR, 2Neurology/Busan/KR

PS2Group5-027  VANGL2, A CORE COMPONENT OF THE WNT PCP PATHWAY CONTRIBUTES TO NEUROMUSCULAR JUNCTION FORMATION
*M. Boex*1, J. Messéant1, S. Bauché2, L. Strochlic3, B. Fontaine3; 1Team “GEN-PHYs: Neugenetics and Physiology” UMR_5112/Paris/FR, 2Equipe Neurogenetic and physiology/Paris/FR, 3/Paris/FR

PS2Group5-028  A 3D SYSTEM FOR MECHANICAL CHARACTERIZATION OF ARTIFICIAL SKELETAL MUSCLE MICROTISSUE
*M. Spörrer*1, D. Kahl1, S. Wiedenmann1, I. Thievenssen1, R. Schröder2, W.H. Goldmann1, B. Fabry1; 1Department of Physics, Biophysics Group/Erlangen/DE, 2Neuropathology/Erlangen/DE

PS2Group5-029  POTENTIALLY CONFOUNDING VARIABLES OF GDF-15: MITOCHONDRIA DISEASE AND OTHER NEUROLOGICAL DISEASES
*A. Ishii*1, S. Nohara1, Z. Miyake1, N. Tozaka1, S. Okune1, H. Takeda1, H. Tsuji1, Y. Tomidokoro1, K. Nakamago1, K. Ishii1, M. Watanabe1, A. Tamaoka1, S. Yatsuga2, Y. Koga2; 1Neurology/Tsukuba/JP, 2Pediatrics/Kurume/JP

PS2Group5-030  QUANTITATIVE ESTIMATES OF ULTRASOUND IMAGING IN MUSCLE PATHOLOGY
*D. Veltisista*, E. Chroni; Neurology/Patras/GR

PS2Group5-031  CIRCULATING KV1.3+ CELLS IN PATIENTS WITH SPORADIC INCLUSION BODY MYOSITIS

PS2Group5-032  ULTRASOUND IMAGING OF GENIOGLOSSUS MUSCLE FUNCTION WITH CONTRAST AGENT
*O.M. Semeryak*1; /Lviv/UA

PS2Group5-033  COMPUTATIONAL SPEECH ANALYSIS AS A TOOL FOR EARLY DETECTION OF BULBAR DYSFUNCTION IN ALS PATIENTS

PS2Group5-034  THE IMPORTANCE OF A NON-INVASIVE SCREENING IN PROXIMAL MYOPATHIES
*G. Bruno*, L. Allegorico, L. Lombardi, G. Di Iorio, S. Sam-polo; Second Neurology/Naples/IT

PS2Group6-001  CLINICAL DIVERSITY OF P/Q-TYPE CALCIUM CHANNEL ANTIBODY-ASSOCIATED PARANEOPLASTIC DISORDERS
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PS2Group6-002  GENOMES IDENTIFIED THE EXPRESSION OF 264 NEUROGENES
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PS2Group6-003  CELL ATLAS IDENTIFIED A CORE COMPONENT OF THE WNT PCP PATHWAY CONTRIBUTES TO NEUROMUSCULAR JUNCTION FORMATION
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PS2Group6-004  A 3D SYSTEM FOR MECHANICAL CHARACTERIZATION OF ARTIFICIAL SKELETAL MUSCLE MICROTISSUE
*M. Spörrer*1, D. Kahl1, S. Wiedenmann1, I. Thievenssen1, R. Schröder2, W.H. Goldmann1, B. Fabry1; 1Department of Physics, Biophysics Group/Erlangen/DE, 2Neuropathology/Erlangen/DE

PS2Group6-005  QUANTITATIVE ESTIMATES OF ULTRASOUND IMAGING IN MUSCLE PATHOLOGY
*O.M. Semeryak*1; /Lviv/UA
PS2Group6-002  CLINICAL FEATURES AND TREATMENT OUTCOMES OF NEUROLYMFIOMATOSIS IN KOREA  
*D.H. Sung*; Department of Physical and Rehabilitation Medicine/Seoul/KR

PS2Group6-003  PEDIATRIC EXTRADURAL COMPRESSIVE MYELOPATHY SECONDARY TO GANGLIONEUTO-BLASTOMA; NERVE ROOTS AND BRACHIAL PLEXUS BEWAE  
*A. Yaworski*1, K. R.G. Straatman, M. P. De Ceuster2, N. T. Janssens, J. N. Smeets3; 1Department of Pediatric Neurology/Brussels/BEL, 2Neurosurgery/Brussels/BEL, 3Department of Radiology/Brussels/BEL

PS2Group9-001  PSYCHOLOGICAL ASPECTS IMPACTING QUALITY OF LIFE OF PATIENTS WITH MYOPATHY  
*A. Rohmer Cohen*1, V. Noel2, M. Mane2, S. Zorgani2, D. Delorme2, J. Rangel Escribano2, C. Bungener1; 1Boulogne-Billancourt/FR, 2/Paris/FR

PS2Group9-002  AN UNCOMMON CO-EXISTENCE OF INHERITED AND CHRONIC INFLAMMATORY DEMEYLINATING POLYNEUROPATHY  
*I. Gläzere*1, S. Šetlere2, I. Kazaine2, G. Rozentāls2; 1Neurology/Riga/LV, 2Pediatric Neurology/Riga/LV

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*B.-N. Yoon*1, J.-W. Yang2; 1Neurology/Mayonge-Billancourt/FR, 2/Incheon/KR

PS2Group9-006  GLOBAL VS INDIVIDUAL MUSCLE FATTY DEGENERATIVE CHANGES TO MONITOR DISEASE PROGRESSION IN IMMUNE-MEDIATED NECROTIZING MYOPATHY  
C. Kounako1, H. Reyngoudt1, J.-M. Boisserie2, O. Landon-Cardinal3, Y. Allenbach3, *P.G. Carlier*1; 1AIM & CEA NMR Laboratory, Neuromuscular Investigation Center/Paris/FR, 2/NMR Laboratory, Neuromuscular Investigation Center/Paris/FR, 3/Department of Internal Medicine and Clinical Immunology/Paris/FR

PS2Group9-007  KCNK9 IMPRINTING SYNDROME WITH CONGENITAL HYPERTONIA, DYSMORPHISM AND DEVELOPMENTAL DELAY DUE TO NOVEL MUTATION P.A.LA237ASP  
*P. Seeman*1, J. Haberlova2, L. Sedláčková, M. Šedivá1, P. Laššuthová1; 1Child Neurology, DNA Laboratory/Praha/CZ, 26 Department of Child Neurology/Prague/CZ

PS2Group9-010  PERCEIVED QUALITY OF LIFE AND MOTOR SCALES OUTCOMES IN TYPE 2 AND 3 SMA PATIENTS: ARE THEY RELATED?  
A.L. Frongia1, *I. Zschaech*1, M.M.A. Alarcon Cornejo1, D. Natera De Benito1, L. Carrera-Garcia2, J.F. Mata1, N. Padros1, O. Moya1, J. Medina1, C. Ortez1, J. Colomer3, A. Nascimento1; 1Neuromuscular Disorders Unit/Barcelona/ES, 2/Universidad de Patologia Neuromuscular/Barcelona/ES, 3/Barcelona/ES

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*P.T. Thomas*1, M. Warrier1, A. Nalini2; 1Department of Psychiatric Social Work/Bangalore/IN, 2Department of Neurology/Bangalore/IN

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*E. Matsuura*; Neurology and Geriatrics/Kagoshima City/JP

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PS2Group9-014  OSTEOPOROSIS IN DUCHENNE MUSCULAR DYSTROPHY PATIENTS  
F.M. Sertpoyraz1, *F. Baydan*2; 1Physical Therapy and Rehabilitation clinic/Izmir/TR, 2/2Pediatric Neurology/Izmir/TR

PS2Group9-015  NEUROMUSCULAR GENETIC REGISTRY IN RUSSIA  
*D. Vlodavets*1, A. Monakhova1, D. Reshetov2, S. Artemyeva1, I. Shulyakova1, O. Shidlovskaya1, E. Belousova1; 1Russian Children Neuromuscular Center/Moscow/RU, 2/Moscow/RU
PS2Group9-016  ALTERNATIVE ANALYSES OF RESPIRATORY FUNCTION IN DUCHENNE MUSCULAR DYSTROPHY: CONSISTENT TREATMENT BENEFIT OF IDEBENONE

PS2Group9-017  MISDIAGNOSIS OF DUCHENNE MUSCULAR DYSTROPHY
*I.D.L. Kalit*, J.D.L. Fernandes, A.J. Godoy; /Alto De Pinheiros - Sao Paulo/BR

PS2Group9-018  ENSURING HIGH QUALITY MUSCLE BIOPSIES AND MAGNETIC RESONANCE BIOMARKERS IN THE PHASEOUT DMD STUDY

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A. Oyewole1, *J. Lee*1, R. Leary1, K. Bushby1, A. Aartsma-Rus2, J. Kirschner3, K. Flanigan4; 1/The John Walton Muscular Dystrophy Research Centre/Newcastle/GB, 2/Human Genetics/Leiden/NL, 3/Freiburg/DE, 4/Columbia/US, 5/London/GB

PS3Group2-001  THYMECTOMY LOWERS THE MYASTHENIA GRAVIS BIOMARKER MIR-150-5P

PS3Group2-002  EFFICACY OF SUBCUTANEOUS IMMUNOGLOBULIN IN MYASTHENIA GRAVIS EXACERBATION

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*Z. A. Siddiqi*1, G. Beecher2, D. Anderson3; 1/Neurology/Medicine/Edmonton, /CA, 2/Neurology/Medicine/Edmonton, AB/CA, 3/Edmonton, AB/CA

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PS3Group2-010  MYASTHENIA GRAVIS: THYMECTOMY IN CHILE (MGTC)
*M. Fuenteaiba*1, R. Gonzalez2, A. Riquelme2; 1/Medicine/Concepcion/CL, 2/Concepcion/CL

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PS3Group2-014  THE FIRST KOREAN CASE OF COLQ-MUTANT MYASTHENIC SYNDROME
*S.-H. Jung*, J.-H. Shin, D.-S. Kim; Neurology/Kyung Hee University, South Korea

PS3Group2-005  WHEN IS BETTER GOOD ENOUGH? PATIENT ACCEPTABLE STATES IN MYASTHENIA GRAVIS
*C. Barnett*1, M. Mendoza2, H. Katzberg1, V. Bri2; 1/Toronto, ON/CA, 2/Toronto/CA

PS3Group2-006  BIOMARKER DISCOVERY IN CHILDHOOD REFRACTORY OCULAR MYASTHENIA GRAVIS
*C. Khongkhathithum*, K. Srisuwan, S. Chutipongtane, L. Thampratankul, A. Visudthibhan; Pediatrics/Bangkok/TH

PS3Group2-007  DIFFERENCES BETWEEN THYMOMA-TOUS AND NON-THYMOMATOUS MYASTHENIA GRAVIS
*F. Aguirre*1, A. Manin2, A. Vilai1; 1/Neurology/Buenos Aires/AR, 2/Neurology/Buenos Aires City/AR

PS3Group2-008  THYMOMA-ASSOCIATED MYASTHENIA GRAVIS IN ARGENTINA
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PS3Group2-009  EPIDEMIOLOGICAL FEATURE OF MYASTHENIA GRAVIS IN SOUTHERN CHILEAN POPULATION
*M. Fuenteaiba*1, M. Padilla2, F. Maturana2; 1/Medicine/Concepcion/CL, 2/Concepcion/CL

PS3Group2-010  MYASTHENIA GRAVIS: THYMECTOMY IN CHILE (MGTC)
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PS3Group2-011  RESPONSE TO ECLULIZUMAB IN PATIENTS WITH ACHR+ REFRACTORY MYASTHENIA GRAVIS RECENTLY TREATED WITH CHRONIC IVIG

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*S.-H. Jung*, J.-H. Shin, D.-S. Kim; Neurology/Kyung Hee University, South Korea
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PS3Group2-017 SOCIAL CONSEQUENCES AND QUALITY OF LIFE OF PATIENTS WITH MYASTHENIA GRAVIS  
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PS3Group2-018 HIGH EFFICACY AND SAFETY OF RITUXIMAB FOR MYASTHENIA GRAVIS: A NATIONWIDE STUDY BY AUSTRIAN ADULT NEUROLOGISTS  
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PS3Group2-019 DIFFERENTIAL RESPONSE TO RITUXIMAB IN ACHR AND MUSK ANTIBODY POSITIVE MYASTHENIA GRAVIS: A SINGLE-CENTER RETROSPECTIVE STUDY  
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PS3Group2-020 OUTCOMES IN GENERALIZED MYASTHENIA GRAVIS PATIENTS IN THE NEW MILLENIUM: DATA FROM A SINGLE-CENTER RETROSPECTIVE STUDY  

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PS3Group2-022 CONGENITAL MYASTHENIC SYNDROME: AN INDIAN SCENARIO  
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PS3Group2-023 ‘MINIMAL SYMPTOM EXPRESSION’ IN ACETYLCHOLINE RECEPTOR-POSITIVE REFRACTORY MYASTHENIA GRAVIS PATIENTS TREATED WITH ECULIZUMAB  

PS3Group2-024 THE PLACEBO EFFECT IN MYASTHENIA GRAVIS: A META-ANALYSIS  
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PS3Group2-025 CONGENITAL MYASTHENIC SYNDROMES IN A SUBPOPULATION OF THE NORTH OF PORTUGAL  
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PS3Group2-026 FETAL ACETYLCHOLINE RECEPTOR INACTIVATION SYNDROME: A RARE, BUT POTENTIALLY TREATABLE CAUSE OF FAMILIAL MYOPATHY  
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PS3Group2-027 CLINICAL AND SEROLOGICAL PREDICTORS OF THYMOMA RECURRENCES IN MYASTHENIA GRAVIS  
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PS3Group2-028 THE EFFECTS OF NEUROTROPHIC FACTORS ON HUMAN MUSCLE CELLS: A COMPARISON WITH MURINE MUSCLE CELLS  
*A. Barbeau*, F. Semprez, C. Legay; /Paris Cedex/FR

PS3Group2-029 A PILOT STUDY OF ENGINEERED AGRIN MURINE MUSCLE CELLS  
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PS3Group2-030 THE EFFECTS OF ECULIZUMAB ON HUMAN MUSCLE CELLS  
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PS3Group2-033 THE IMPACT OF REFRAC'TORY MYAS-THENIA GRAVIS (MG) ON PATIENT HEALTH-RELATED QUALITY-OF-LIFE (QOL)

PS3Group2-034 VOCAL CORD PARALYSIS: A RARE PRE-SENTATION OF MYASTHENIA GRAVIS
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PS3Group2-035 SERONEGATIVE MYASTHENIA GRAVIS WITH ANTI-LRP4 ANTIBODIES

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PS3Group4-002 PD AND SAFETY DATA FROM JEW-ELFISH, A STUDY OF R07916 IN SMA PATIENTS PREVIOUSLY ENROLLED IN A SMN2-SPlicing MODIFIER STUDY

PS3Group4-003 MLBPA BASED SMN1 DELETION ANALYSIS: CLINICAL CORRELATION IN INDIAN vPATIENTS WITH SPINAL MUSCULAR ATROPHY
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PS3Group4-004 ACUTE MOTOR NEURONOPATHY ASSOCIATED TO AN INFECTION BY TICK-BORNE ENCEPHALITIS VIRUS

PS3Group4-005 ASSOCIATION OF AMYOTROPHIC LATERAL SCLEROSIS AND MULTIPLE SCLEROSIS: A CASE REPORT
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PS3Group4-006 EXPLORATION OF THE REPOSITIONING POTENTIAL OF MARKETED DRUGS FOR NEUROPATHIES
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PS3Group4-007 DISSECTING UBAS DISFUNCTION IN NERVOUS SYSTEM DISORDERS USING THE ZEBRAFISH
*R. Serrano*1, V. Oorschot2, G. Ramm2, R. Bryson-Rich-
PS3Group4-008 BRANAPLAM IN TYPE 1 SPINAL MUSCULAR ATROPHY: RESPIRATORY SUPPORT AND FEEDING

PS3Group4-009 NUSINERSEN TREATMENT IN LONG-STANDING ADULT SMA TYPE 3 PATIENTS
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PS3Group4-010 DIAGNOSIS AND DIFFERENTIAL DIAGNOSIS OF SPINAL MUSCULAR ATROPHY
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PS3Group4-011 A CASE OF HEXANUCLEOTIDE REPEAT EXPANSION CAUSING AMYOTROPHIC LATERAL SCLEROSIS
*P.D. Shanmugarajah*, J.O.S. Archer; Neurology/Wakefield/GB

PS3Group4-012 A DE NOVO HETEROZYGOUS POINT MUTATION IN THE DYN1CH1 GENE CAUSING SPINAL MUSCULAR ATROPHY WITH LOWER EXTREMITY DOMINANT
*K. J. Thusang*, H. Jiang; Neurology/Detroit, MI/US

PS3Group4-013 SMARD1: A RARE CAUSE OF HYPOTONIA AND RESPIRATORY FAILURE IN INFANCY
*C. Garrido1, J. Oliveira2, A. Sousa3, M. Cardoso4, R. Santos2, M. Santos5; 1/Neuropediatria/011/PT, 2/Genetics Service/-/PT, 3/Laboratory of Autoimmune Diagnostics, Center for Molecular Medicine/St-Petersburg/RU

PS3Group4-014 NUSINERSEN EXPERIENCE IN INDIVIDUALS WITH SPINAL MUSCULAR ATROPHY TYPE III: A CASE SERIES
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PS3Group4-015 RG7916 SIGNIFICANTLY INCREASES SMN PROTEIN IN SMA TYPE 1 BABIES

PS3Group4-016 PATHOGENIC VARIANT OF THE REEP1 GENE IN A KOREAN FAMILY WITH Autosomal Dominant Hereditary Spastic Paraplegia

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PS3Group4-018 ENHANCEMENT OF BULBAR FUNCTION IN ALS: LESSONS LEARNED FROM THE NUDEXTA TREATMENT TRIAL
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PS3Group4-019 SALBUTAMOL TREATMENT IN TYPE 2 SMA PATIENTS: 18 MONTHS ASSESSMENT
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PS3Group4-020 VERY LATE-ONSET AMYOTROPHIC LATERAL SCLEROSIS IN A PORTUGUESE COHORT: WHICH DIFFERENCES?
*M. Oliveira Santos*, M. Gromicho, S. Pinto, M. De Carvalho; /Lisbon/PT

PS3Group4-021 ATAXIN-2 IN RUSSIAN ALS PATIENTS
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PS3Group4-022 AVXS-101 PHASE 1 GENE THERAPY CLINICAL TRIAL IN SMA TYPE 1: EVENT-FREE SURVIVAL AND ACHIEVEMENT OF DEVELOPMENTAL MILESTONES
*J.R. Mendell*,1, S. Al-Zaidy1, R. Shell1, W.D. Arnold1, L. Redino-Klapac1, T.W. Prior1, L. Lowes1, L. Alfano1, K. Berry1, K. Church1, J.T. Kissel1, S. Nagendra2, J. L'italien2, D.M. Sproule2, C. Wells2, A.H.M. Burghes1, K. Foust2, K. Meyer1, S. Likhit1, B. Kaspar1; 1/Columbus, OH/US, 2/Bannockburn, IL/US

PS3Group4-023 AN AUTOMATED ANALYSIS OF REPEAT EXPANSIONS IN ALS: LESSONS LEARNED FROM THE NUDEXTA TREATMENT TRIAL

PS3Group4-024 THE OLEOSOXINE-TREATED TYPE 2 AND NON-AMALATORY TYPE 3 SMA PATIENTS

PS3Group4-025 PATHOGENIC VARIANT OF THE REEP1 GENE IN A KOREAN FAMILY WITH Autosomal Dominant Hereditary Spastic Paraplegia

PS3Group4-026 NEUROFILAMENT LIGHT CHAIN AS A POTENTIAL BIOMARKER IN SPINAL MUSCULAR ATROPHY

PS3Group4-027 ENHANCEMENT OF BULBAR FUNCTION IN ALS: LESSONS LEARNED FROM THE NUDEXTA TREATMENT TRIAL
*R. Smith*,1, K. Myers1, E. Macklin2; 1/La Jolla, CA/US, 2/Massachusetts General Hospital/Boston, MA/US

PS3Group4-028 SALBUTAMOL TREATMENT IN TYPE 2 SMA PATIENTS: 18 MONTHS ASSESSMENT
A.L. Frongia1, *D. Natera De Benito*,1, M.M.A. Alarcon-Cornejo1, L. Carrera-Garcia2, A. Borras1, N. Padros1, O. Moya1, J. Armas1, J. Medina1, M. Vigo3, C. Jou1, C. Jimenez Mallebrera1, C. Ortez1, J. Colomer1, A. Nascimento1; 1/Neuromuscular Disorders Unit/Barcelona/ES, 2/Unidad de Patología Neuromuscular./Barcelona/ES, 3/Boston, MA/US

PS3Group4-029 VERY LATE-ONSET AMYOTROPHIC LATERAL SCLEROSIS IN A PORTUGUESE COHORT: WHICH DIFFERENCES?
*M. Oliveira Santos*, M. Gromicho, S. Pinto, M. De Carvalho; /Lisbon/PT
PS3Group4-025 SMA-RTER? COGNITIVE ASSESSMENT IN SPINAL MUSCULAR ATROPHY TYPE 1-2 USING EYE TRACKING
*L. Paternoster*1, N. Deconinck2, S. Baijot1, G. Deliens3; 1Department of Pediatric Neurology/Brussels/BE, 2Department Pediatric Neurology/Brussels/BE, 3ULB Neurosciences Institute/Brussels/BE

PS3Group4-026 LONGER-TERM ASSESSMENT OF NUSINERSEN SAFETY/EFFICACY IN INFANTILE-ONSET SPINAL MUSCULAR ATROPHY: INTERIM ANALYSIS OF SHINE

PS3Group4-027 A CASE OF KENNEDY’S DISEASE IN A PATIENT INITIALLY PRESENTED WITH RECURRENT PERIODONTITIS AND OROMANDIBULAR PAIN

PS3Group4-028 UPDATED PHARMACODYNAMIC AND SAFETY DATA FROM SUNFISH PART 1, A STUDY OF ORAL RG7916 IN PATIENTS WITH TYPE 2 OR TYPE 3 SMA

PS3Group4-029 MORPHINE FOR DYSPNEA IN ALS
*L. Brylev*, V. Parshikov, Y. Krasnaya, E. Dikhter, E. Larin, Y. Batmanova, V. Shtabnitskiy, S. Avdeykin, A. Ataulina, D. Puzanok, A. Kasianova; /Moscow/RU

PS3Group4-030 EXTRACELLULAR VESICLES FROM ALS SPINAL CORD AND BRAIN CONTAIN DYSREGULATED MIRNAS
*E. Feldman*; /Ann Arbor/US
EMERGING STRATEGIES IN THE DIAGNOSIS AND MANAGEMENT OF MUSCLE DISEASES

MONDAY 9 JULY, 12:15–13:45
PARK CONGRESS 1, GROUND FLOOR, HILTON VIENNA AM STADTPARK, VIENNA

AGENDA

Symposium introduction

MYO-SEQ – experience from a large-scale next generation sequencing project of unclassified myopathies

Prof John Vissing (Chair)

Prof Volker Straub

Dr Jordi Diaz-Manera

Unravelling patterns of muscle change in myopathies using MRI

Prof John Vissing

Treatment of muscle glycogenoses

Summary and closing remarks
INDUSTRY-SUPPORTED SEMINARS

SATURDAY, JULY 7, 2018

12:15-13:45 Luncheon Symposium supported by Sarepta
Room ➔ Park Congress 2

Precision-genetic therapies for DMD

- Precision-genetic Therapy Approaches: An Overview – Perry Shieh
- Exon-Skipping Therapies: Summary of Clinical Data – Eugenio Mercuri
- RNA & Gene Therapy: Combination Approaches – Thomas Voit

12:15-13:45 Luncheon Symposium supported by CSL Behring
Room ➔ Park Congress 1

Subcutaneous Immunoglobulin in CIDP: Getting Under the Skin

Chair: Vera Bril

- Expanding the Evidence for Subcutaneous Immunoglobulin in CIDP: PATH and Beyond – Hans Katzberg
- Administering Subcutaneous Immunoglobulin: A Nurse’s Perspective – Dorothea Grosse-Kreul
- Patient Experiences with Intravenous and Subcutaneous Immunoglobulin Therapy – James Babington Smith

SUNDAY, JULY 8, 2018

07:00-08:00 Breakfast Symposium supported by LUPIN
Room ➔ Berg

A clinical approach in non-dystrophic and dystrophic myotonia

Chair: Giovanni Meola

- The spectrum of myotonic dystrophies (DM) and non-dystrophic myotonias – Christiane Schneider-Gold
- Outcome measure of myotonia – Giovanni Meola

12:15-13:45 Luncheon Symposium supported by Santhera Pharmaceuticals
Room ➔ Park Congress 2

Respiratory function decline in Duchenne muscular dystrophy (DMD) – Insights and evolving treatment strategies

Chair: Thomas Voit

- New insights into the natural history of respiratory function decline in patients with DMD – Craig McDonald
- Establishing clinically relevant thresholds of respiratory function decline and respiratory complications in DMD – Oscar H Mayer
- Treatment strategies to slow respiratory function decline in DMD and address unmet needs – Thomas Voit
**INDUSTRY SUPPORTED SEMINARS**

12:15-13:45 Luncheon Symposium supported by Biogen
Room ➤ Park Congress 1

**Spinal Muscular Atrophy: Challenges Beyond Childhood**
Chair: Janbernd Kirschner
- SMA in teens and adults: our current understanding – Janbernd Kirschner
- Considerations for optimizing care for teen and adult patients with SMA – Maggie Walter
- Living a normal life in an extraordinary way – Valeria Sansone

**MONDAY, JULY 9, 2018**

07:00-08:00 Breakfast Symposium supported by Alnylam Pharmaceuticals
Room ➤ Klimt 1

**Diagnosing and Assessing Burden of hATTR Amyloidosis: A Case Series**
Chair: Isabel Conceição
- A Case of Late Onset V30M: Delays in Diagnosis – Isabel Conceição
- Challenges of hATTR Amyloidosis Diagnosis – Claudia Sommer
- Recognizing Cardiac Manifestations of hATTR Amyloidosis – Pablo Garcia-Pavia

07:00-08:00 Breakfast Symposium supported by F. Hoffmann-La Roche Ltd.
Room ➤ Berg

**SMA: balancing the emotional and clinical journey from molecule to medicine**
Chair: Eugenio Mercuri
- Challenges in the management of SMA: the patient and carer perspective (the emotional journey) – Nicole Gusset
- From molecule to medicine (the clinical journey) – Laurent Servais & Lutz Mueller

12:15-13:45 Luncheon Symposium supported by Alexion
Room ➤ Park Congress 2

**A Novel, Targeted Treatment Option in Refractory Generalized Myasthenia Gravis**
Chair: Renato Mantegazza
- Disease Overview and the Patient Burden in Generalized Myasthenia Gravis - Renato Mantegazza
- Role of Complement Inhibition in Patients with Anti-AChR+ gMG – Gil Wolfe
- Patient Case Discussion Panel – Renato Mantegazza and Gil Wolfe
To those who say “impossible, impractical, unrealistic,” we say:

**CHALLENGE ACCEPTED**

We are relentless in our pursuit of new treatments. Because people living with rare diseases shouldn’t have to wait for hope.

Visit us at booth #5 to learn more or go to alnylam.com

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12:15-13:45 Luncheon Symposium supported by Sanofi Genzyme
Room ➤ Park Congress 1
Emerging Strategies in the diagnosis and management of muscle diseases
Chair: John Vissing

➤ MYO-SEQ: experience from a large-scale next generation sequencing project of unclassified myopathies – Volker Straub
➤ Unravelling patterns of muscle change in myopathies using MRI – Jordi Díaz-Manera
➤ Treatment of muscle glycogenosis – John Vissing

TUESDAY, JULY 10, 2018

12:15-13:45 Luncheon Symposium provided by The France Foundation and supported by AveXis
Room ➤ Park Congress 2
Gene Replacement Therapy in Spinal Muscular Atrophy
Chair: Eugenio Mercuri
Speakers:
➤ Richard S. Finkel
➤ Gyula Acsadi
Topics:
➤ Genetics and Diagnostic Criteria of SMA
➤ Emerging Data in SMA: Gene Replacement and Gene Modifying Therapies
➤ Durability of New and Emerging Treatments
➤ Implications for Standards of Care
➤ Potential Applications to Other Neuromuscular Diseases

12:15-13:45 Luncheon Symposium supported by PTC Therapeutics
Room ➤ Park Congress 1
Duchenne muscular dystrophy*: working together to reduce the time to diagnosis and improve patient outcomes
Chair: Günther Bernert

➤ Striving for an earlier diagnosis of DMD: impact for patients and their families – Janbernd Kirschner
➤ A look at the 2018 standards of care guidelines for DMD: is there a need for change in the current diagnostic and therapeutic procedures? – Günther Bernert
➤ Ataluren* treatment for patients with nonsense mutation DMD: what do the latest data tell us? – Yann Péréon

* Ataluren is only indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older.
### DIRECTORY OF EXHIBITORS

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IOS Press (Journal of Neuromuscular Diseases) | Table #D
IOS Press, established in 1987, publishes around 100 international journals and approximately 75 book titles annually, in a broad range of subjects. IOS Press has a strong neurosciences package that includes Journal of Neuromuscular Diseases, along with Journal of Alzheimer’s Disease, Journal of Parkinson’s Disease and Journal of Huntington’s Disease. (iospress.com/journal-of-neuromuscular-diseases)

MGZ – Medizinisch Genetisches Zentrum | Booth #13
The MGZ – Medical Genetics Center has been providing diagnostic genetic testing and counseling services for over 15 years. Analyses are performed in our accredited medical genetics lab and interpreted by both our clinical geneticists and scientific team, supporting clinicians worldwide in the diagnosis and care of their patients. (mgz-muenchen.de)

Österreichische Muskelforschung – Austrian Muscle Research Organization | Table #E
Österreichische Muskelforschung focuses on the research of muscular diseases. This non-profit organization supports research projects in Austria and works closely with the Kaiser-Franz-Josef and Gottfried von Preyer’s Kinderspital (Children’s Hospital) in Vienna. The Organization is in constant contact with patient organizations to know their needs and provide updates of the progress of research. (muskelforschung.at)

PHARNEXT | Booth #2
Pharnext is an advanced-clinical-stage biopharmaceutical company, leader in combinatorial medicine developing novel therapeutics for diseases that currently lack curative and/or disease-modifying treatments. Pharnext is pioneering a new paradigm in drug development: PLEOTHERAPY™ and has two lead products in clinical development. (pharnext.com)

Philips Healthcare | Booth #14
At Philips, we look beyond technology to the experiences of patients, providers and caregivers across the health continuum from healthy living to prevention, diagnosis, treatment, recovery and home care. We unlock insights leading to innovative solutions that help deliver better care at lower costs. It’s a unique perspective empowering us all to create a healthier future. (philips.com/healthcare)

PTC Therapeutics | Booth #1
PTC Therapeutics is a global biopharmaceutical company focused on the discovery, development and commercialisation of novel orally administered drugs that target RNA mechanisms affecting protein production. It is PTC’s mission to bring new therapies to patients affected by rare and neglected diseases. (ptcbio.com)
<p>| Booth #15 | Sanofi Genzyme | Sanofi Genzyme is the specialty care global business unit of Sanofi, focused on rare diseases, multiple sclerosis, immunology, and oncology. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world. (sanofigenzyme.com) |
| Booth #9 | Santhera Pharmaceuticals | Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company committed to developing medicines to meet the needs of patients living with mitochondrial disorders and other rare diseases. Our focus is on developing treatments for neuromuscular and neuro-ophthalmological diseases that currently lack treatment options, and have a severe impact on patients’ lives. (santhera.com) |
| Booth #18 | Sarepta | Sarepta Therapeutics is a U.S. commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Sarepta is working to rapidly advance its exon-skipping platform for the development of treatments for Duchenne muscular dystrophy and is proud to support the 15th International Congress on Neuromuscular Diseases. (sarepta.com) |
| Table #F | TREAT-NMD | TREAT-NMD is a global neuromuscular network that provides an infrastructure to ensure promising therapies reach patients quickly. The network’s focus is on developing tools that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development and into the clinic, and on establishing best-practice care for patients worldwide. (treat-nmd.eu) |
| Table #A | University of Florida Health Shands Hospital | The University of Florida Neuromuscular Division is a group of faculty and staff in the Department of Neurology at UF dedicated to improving the condition of those patients suffering from neuromuscular disorders while conducting a wide array of research projects aimed at better understanding the mysteries behind neuromuscular action and dysfunction. (UFHealth.org) |</p>
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Is homework exhausting him…

…or is it **Respiratory Function Decline** in DMD?

Respiratory complications are one of the leading causes of mortality in DMD

Come find out more at the Santhera booth (#9) at ICNMD in Vienna, Austria from 6th-10th July
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