

July 5–9, 2016 Toronto, Canada

ICNMD 2016

14th International Congress on Neuromuscular Diseases



➤ Onsite Program



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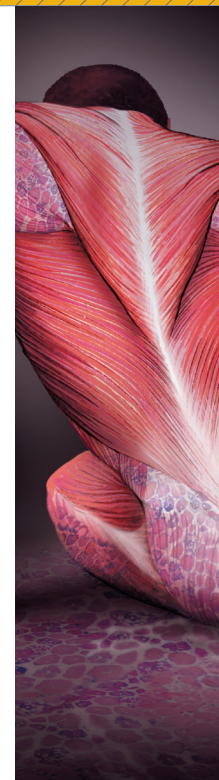


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CONGRESS SECRETARIAT



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WELCOME MESSAGE

Message from the ICNMD President



Dr. Vera Bril

► **PRESIDENT,**
14TH INTERNATIONAL CONGRESS
ON NEUROMUSCULAR DISEASES

Dear Colleagues:

It gives me the greatest pleasure to welcome you to Toronto for the **14th International Congress on Neuromuscular Diseases** from **July 5–9, 2016**. We are very excited to be hosting this important conference in Toronto as the Congress has been located in Europe for its meetings excepting the inaugural meeting in Vancouver in 2002. We hope to follow in the successful footsteps of the meetings in Vancouver, Istanbul, Naples and Nice. At the meeting in Nice in 2014, it was decided that the Congress would move to a two-year cycle instead of meeting every four years. At that meeting, Toronto won the bid to host the next International Congress in 2016.

We have developed robust committees: International, program and local organizing, that are populated by outstanding neuromuscular physicians. The program committee is planning a fabulous program ranging across the spectrum of neuromuscular disorders. We will have sessions on muscular dystrophies, other myopathies, myasthenia gravis, polyneuropathies, spinal cord disorders, genetics, ultrasound and neurofibromatosis to name some of the major themes within the Congress. We will have updates on our understanding of the genetics, pathogenesis, evaluation and treatment of neuromuscular disorders. At the end of this Congress, we hope that attendees feel that they have garnered the most up-to-date information available in neuromuscular disorders.

We will have sessions on muscular dystrophies, other myopathies, myasthenia gravis, polyneuropathies, spinal cord disorders, genetics, ultrasound and neurofibromatosis to name some of the major themes within the Congress.

The members of the local organizing committee are very proud to be hosting the ICNMD2016 and look forward to welcoming you to our city and country. Toronto and its environs are an amazing place to visit. Our multicultural city has many diverse neighborhoods of interest to visitors. Short distances away are wonderful attractions such as Niagara Falls. We also have easy access to the North Country for those who are interested in visiting the Canadian wilderness. The meeting promises to be highly rewarding on a social basis as well as on an intellectual basis.

We welcome you to an inspiring, educational and enjoyable program.

Sincerely,



Dr. Vera Bril

Ellen & Martin Prosserman Centre
for Neuromuscular Diseases
University Health Network
University of Toronto

COMMITTEES

PRESIDENT

Dr. Vera Bril

Ellen & Martin Prosserman Centre
for Neuromuscular Diseases

University Health Network
University of Toronto, CA

LOCAL COMMITTEE MEMBERS

Carolina Barnett

University of Toronto, CA

Ari Breiner

University of Toronto, CA

James Dowling

Hospital for Sick Children
(SickKids), CA

Aaron Izenberg

Sunnybrook Health Sciences
Centre, CA

Charles Kassardjian

University of Toronto, CA

Hans Katzberg

University of Toronto, CA

Mark Tarnopolsky

McMaster University Medical
Centre, CA

Jiri Vajsar

Hospital for Sick Children
(SickKids), CA

Lorne Zinman

Sunnybrook Health Sciences
Centre, CA

PROGRAM COMMITTEE MEMBERS

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University of Kansas Medical
Center, US

Michael Benatar

University of Miami Leonard M.
Miller School of Medicine, US

Timothy Benstead

Capital Health, CA

Vera Bril

University of Toronto, CA

Ted M. Burns

University of Virginia School
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Craig Campbell

London Health Sciences Centre, CA

Kristine Chapman

UBC Department of Medicine,
CA

Avneesh Chhabra

University of Texas Southwestern
Medical Center, US

David Cornblath

Johns Hopkins University School
of Medicine, US

Jeffrey Dilworth

Ottawa Hospital Research Institute,
CA

Mazen Dimachkie

University of Kansas Medical
Center, US

Peter Daniel Donofrio

Vanderbilt University, US

P. James B. Dyck

Mayo Clinic, US

Michael G. Fehlings

University of Toronto, CA

Eva L. Feldman

University of Michigan, US

Kevin Flanigan

Nationwide Children's Hospital, US

Angela GengeMontreal Neurological Institute
and Hospital, CA**Susan Iannaccone**University of Texas Southwestern
Medical Center, US**John Kissel**Ohio State University Wexner
Medical Center, US**Lawrence Korngut**

University of Calgary, CA

Richard Lewis

Cedars-Sinai Hospital, US

Ingemar Merkies

Academic Hospital Maastricht, NL

Tahseen Mozaffar

University of California, Irvine, US

Ali Naraghi

University of Toronto, CA

Richard Robitaille

Université de Montréal, CA

James RussellUniversity of Maryland School
of Medicine, US**Kathryn Selby**Children's & Women's Health
Centre of BC, CA**Zaeem Siddiqi**

University of Alberta, CA

Gordon Smith

University of Utah Health Care, US

Gil Wolfe

University of Buffalo, SUNY, US

Gelareh Zadeh

University of Toronto, CA

Douglas W. Zochodne

University of Calgary, CA

**INTERNATIONAL COMMITTEE
MEMBERS****Claude Desnuelle**Centre Hospitalier Univeritaire
de Nice, FR**John England**

LSUHSC School of Medicine, US

Wolfgang Grisold

Vienna Hospital Association, AT

Haruki KoikeNagoya University Graduate
School of Medicine, JP**Hadi Manji**

University College London, UK

Carlos Navarrete Maldonado

Clínica Davila, CL

Davide Pareyson

Istituto Neurologico Carlo Besta, IT

Mary Reilly

University College London, UK

Gerard Said

Hôpital de la Salpêtrière, FR

Andreas Steck

University of Basel, CH

REGISTRATION INFORMATION



REGISTRATION INFORMATION

Registration for all attendees (delegates, invited speakers, exhibitors, sponsors, media and accompanying persons) is located at the **Sheraton Centre, Lower Concourse Level**.

ONSITE REGISTRATION HOURS

MONDAY, JULY 4	12:30 – 17:00
TUESDAY, JULY 5	07:00 – 20:00
WEDNESDAY, JULY 6	07:00 – 17:00
THURSDAY, JULY 7	07:00 – 17:00
FRIDAY, JULY 8	07:00 – 19:00
SATURDAY, JULY 9	07:00 – 17:00

REGISTRATION MATERIALS

- ▶ Name Badge and tickets
- ▶ Delegate Bag includes:
 - Program Book
 - Congress USB Stick with Congress abstracts
 - Congress Abstract Book
 - Invitations for Industry-Supported Symposia
 - Additional Promotional Flyers



REGISTRATION COUNTERS

Pre-Registration

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

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

Onsite Registration/Outstanding Payments

- ▶ For all delegates who have 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
 - Hands-On Training Sessions and Workshop Tickets
 - Gala Dinner Tickets

Delegate Help Desk

- ▶ Tourism Toronto will be available for any information around Toronto
- ▶ Exhibitors can pick up their lead retrieval
- ▶ General Congress information



OFFICIAL NETWORKING EVENTS

OPENING CEREMONY*

TUESDAY, JULY 5 18:30 – 21:00

Plenary Hall, Grand Ballroom Centre, Lower Concourse

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

WELCOME RECEPTION*

TUESDAY, JULY 5 19:00 – 21:00

Exhibit Hall, Lower Concourse

Following the Opening Ceremony, guests are invited to the Exhibit Hall for the Welcome Reception and the opportunity to mix and mingle with exhibitors, colleagues and friends while enjoying local wines and hors d'oeuvres.

CONGRESS DINNER

FRIDAY, JULY 8 18:30 – 22:00

Willow East & Centre, Mezzanine level

(Not included in the registration fee – USD 100 per person)

Join old and new friends and enjoy a three-course dinner showcasing local products and complimented with invigorating wines. At the top of the evening, Canada's up and coming solo artist Avery Raquel will take the stage showcasing some of her favourite Jazz songs. Don't miss out on what is to be a great evening.

* These official networking events are Included in the registration fee for delegates and accompanying persons.



CLOSING CEREMONY*

SATURDAY, JULY 9 17:00 – 18:00

Grand Ballroom Centre, Lower Concourse

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 XV ICNMD Congress in 2018.

CME ACCREDITATION INFORMATION

ICNMD 2016 is now CME Accredited!

As you join us in Toronto, Canada for the 14th International Congress on Neuromuscular Diseases, you will be awarded with the following credits from the University of Toronto:

▶ **Royal College of Physicians & Surgeons of Canada**

*27.0 Section 1 credits;

▶ **The American Medical Association**

*27.0 Category 1 credits

Obtainable via a conversion process based on a reciprocal agreement with the Royal College of Physicians & Surgeons of Canada;

▶ **The European Union of Medical Specialists**

*27.0 ECMEC credits;

CME credits will be awarded to the delegate following the Congress with the completion of the Congress Evaluation Form.

* The above total credits for each accreditation category do not include the Poster Sessions.

OFFICIAL LANGUAGE

The official language of the ICNMD Congress is English.

SPEAKER AND POSTER INFORMATION

SPEAKER READY ROOM


Planner Office, located on the concourse level, is the designated Speaker Ready Room.

HOURS

MONDAY, JULY 4	12:30 – 17:00
TUESDAY, JULY 5	07:00 – 17:00
WEDNESDAY, JULY 6	07:00 – 17:00
THURSDAY, JULY 7	07:00 – 17:00
FRIDAY, JULY 8	07:00 – 17:00
SATURDAY, JULY 9	07:00 – 16:00

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, out-lines, animations, etc.

-  **All Presenters (Invited Speakers)** 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.

POSTER DISPLAY

The poster exhibition is located in the **Exhibit Hall**.

POSTER DISPLAY SCHEDULE

Group Topics 2, 4, 6, & 8

Poster Session 1 Wednesday, July 6 – Thursday, July 7

Group Topics 1, 3, 5 & 7

Poster Session 2 Friday, July 8 – Saturday, July 9

*With the exception of the Top 10 Best Posters — those will be displayed throughout the entire Congress on the Electronic Poster Screens.

POSTER DISPLAY HOURS

POSTER SESSION 1

WEDNESDAY, JULY 6 – THURSDAY, JULY 7 10:30 – 12:00

POSTER SESSION 2

FRIDAY, JULY 8 – SATURDAY, JULY 9 10:30 – 12:00

Important Information for Poster Presenters

Poster presenters are requested to be beside their poster during the poster sessions (**see Poster Session Display Hours above**).

A limited number of pins will be available at the Poster Service Desk.

POSTER DISPLAY CATEGORIES

GROUP 1

Muscle Diseases of Genetic Origin: Clinical Features, Pathophysiology, Therapy

- 1.1 Dystrophinopathy
- 1.2 Muscle Dystrophies (Non-Dystrophinopathy)
- 1.3 Congenital Muscular Dystrophy
- 1.4 Congenital Myopathies / Myopathies with Prominent Muscle Contractures
- 1.5 Distal Myopathy / Myofibrillar Myopathies
- 1.6 Myotonic Myopathies
- 1.7 Facioscapulothoracic Muscular Dystrophies / Oculopharyngeal Muscular Dystrophy
- 1.8 Metabolic Myopathies / Mitochondrial Myopathies
- 1.9 Muscle Channelopathies and Related Disorders
- 1.10 Other Myopathies Including GNE – Hereditary Inclusion Body Myopathy

GROUP 2

Acquired Myopathies: Clinical Features, Pathophysiology, Therapy

- 2.1 Inflammatory / Dysimmune Myopathies
- 2.2 Inclusion Body Myositis
- 2.3 Toxic / Endocrine / Other Acquired Myopathies

GROUP 3

Diseases of Neuromuscular Junction: Clinical Features, Pathophysiology, Therapy

- 3.1 Myasthenia Gravis
- 3.2 Myasthenic Syndromes
- 3.3 Congenital Myasthenia

GROUP 4

Peripheral Neuropathy: Clinical Features, Pathophysiology, Therapy

- 4.1 Inflammatory / Dysimmune / Associated with Monoclonal Gammopathy/Paraneoplastic
- 4.2 Hereditary Peripheral Neuropathy
- 4.3 Metabolic / Toxic
- 4.4 Infectious Peripheral Neuropathy (including Leprosy, HIV)
- 4.5 Others

GROUP 5

Motor Neuron Diseases: Clinical Features, Pathophysiology, Therapy

- 5.1 Biology, Genetics
- 5.2 Biomarkers in MND
- 5.3 Epidemiology, Clinic, Treatment
- 5.4 Spinal Muscular Atrophy / Neuronopathies

GROUP 6

Novel Diagnostic Methods in Neuromuscular Diseases

- 6.1 Ultrasound
- 6.2 MRI
- 6.3 Other Biomarkers
- 6.4 Electrodiagnosis
- 6.5 Small Nerve Fibre Evaluation
- 6.6 Biochemical and Molecular Techniques

GROUP 7

Basic Sciences in Neuromuscular Diseases

- 7.1 Muscle Homeostasis / Muscle Regeneration
- 7.2 Muscle Structure / Muscle Development / Muscle Growth
- 7.3 Muscle Atrophy / Degeneration
- 7.4 Nuclear Envelope / Nuclear Matrix of Muscle Cell
- 7.5 Ion Channel Function in Neuron and Muscle
- 7.6 Immune Mechanisms in Neuromuscular Diseases
- 7.7 Fundamental Approaches to Motor Neuron, Axon and Related Structures
- 7.8 Neuromuscular Junction: Basic Aspects
- 7.9 Others

GROUP 8

Miscellaneous

- 8.1 Outcome Measures in Clinical Trials
- 8.2 Biomarkers in Neuromuscular Disorders
- 8.3 Home Care / Social Programs in Neuromuscular Diseases
- 8.4 Psychological and Neuropsychological Approaches in Neuromuscular Diseases
- 8.5 Ethics in Neuromuscular Disorders
- 8.6 Rehabilitation in Neuromuscular Diseases
- 8.7 Others

PLENARY SPEAKERS

► WEDNESDAY JULY 6, 2016

PLENARY 1.0

GENETICS

► CHAIR: James Dowling, CA

08:00 – 09:00 PL 1.1 GENOMIC APPROACHES TO DIAGNOSIS OF RARE MUSCLE DISEASE

► **Keynote Speaker:**
Daniel MacArthur, US

09:00 – 09:30 PL 1.2 GENE DISCOVERY IN CHARCOT-MARIE-TOOTH NEUROPATHIES

► **Stephan Züchner, US**

09:30 – 10:00 PL 1.3 RNA SEQUENCE AND RNA ANALYSIS

► **James Dowling, CA**

LOCATION► All Plenary Sessions will be held in the Plenary Hall, located in the **Grand Ballroom Centre, Lower Concourse Level.**

► THURSDAY JULY 7, 2016

PLENARY 2.0

HOT TOPICS

► CHAIRS: Vera Bril, CA & Carlos Navarrete Maldonado, CL

08:00 – 08:15 PL 2.1 STEM CELL THERAPY IN ALS

► **Eva Feldman, US**

08:15 – 08:30 PL 2.2 RESULTS OF THE THYMECTOMY TRIAL IN MYASTHENIA GRAVIS

► **Gil Wolfe, US**

08:30 – 08:45 PL 2.3 REGAIN: A RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED MULTI-CENTER PHASE 3 STUDY OF THE SAFETY AND EFFICACY OF ECULIZUMAB IN SUBJECTS WITH REFRACTORY GENERALIZED MYASTHENIA GRAVIS

► **James F. Howard, Jr., US**

08:45 – 09:00 PL 2.4 APPROACH TO PATIENT-CENTERED OUTCOMES RESEARCH

► **Richard Barohn, US**

09:00 – 09:15 PL 2.5 DO WE STILL NEED MUSCLE BIOPSY IN THE ERA OF ULTRASOUND?

► **Carsten Bonnemann, US**

09:15 – 09:30 PL 2.6 THERAPEUTIC APPROACHES TO INCLUSION BODY MYOSITIS

► **Mazen Dimachkie, US**

09:30 – 09:45 PL 2.7 TREATMENT OF AMYLOID NEUROPATHY

► **David Adams, France**

09:45 – 10:00 PANEL DISCUSSION

► **FRIDAY JULY 8, 2016**

PLENARY 3.0 MUSCULAR DYSTROPHY

08:00 – 08:30 PL 3.1 GENE THERAPY FOR MUSCULAR DYSTROPHY

► **Keynote Speaker:**
Dongsheng Duan, US

08:30 – 09:00 PL 3.1 RNA THERAPEUTICS FOR DUCHENNE MUSCULAR DYSTROPHY

► **Keynote Speaker:**
Dana Martin, US

09:00 – 09:30 PL 3.2 ANTISENSE THERAPY FOR MYOTONIC DYSTROPHY

► **Charles Thornton, US**

09:30 – 10:00 PL 3.3 CRISPR BASED GENE EDITING FOR MUSCULAR DYSTROPHY

► **Ronald Cohn, CA**

► **SATURDAY JULY 9, 2016**

PLENARY 4.0 MOTOR NEURON DISEASE

► **CHAIR: John Kissel, US**

08:00 – 09:00 PL 4.1 ALS THERAPY DEVELOPMENT: CHALLENGES AND OPPORTUNITIES

► **Keynote Speaker:**
Michael Benatar, US

09:00 – 09:30 PL 4.2 BIOLOGY OF C9ORF72 DISEASE

► **Leonard Petrucelli, US**

09:30 – 10:00 PL 4.3 ANTISENSE THERAPY FOR SPINAL MUSCULAR ATROPHY

► **John Kissel, US**

PROGRAM AT A GLANCE

TUESDAY
JULY 5, 2016

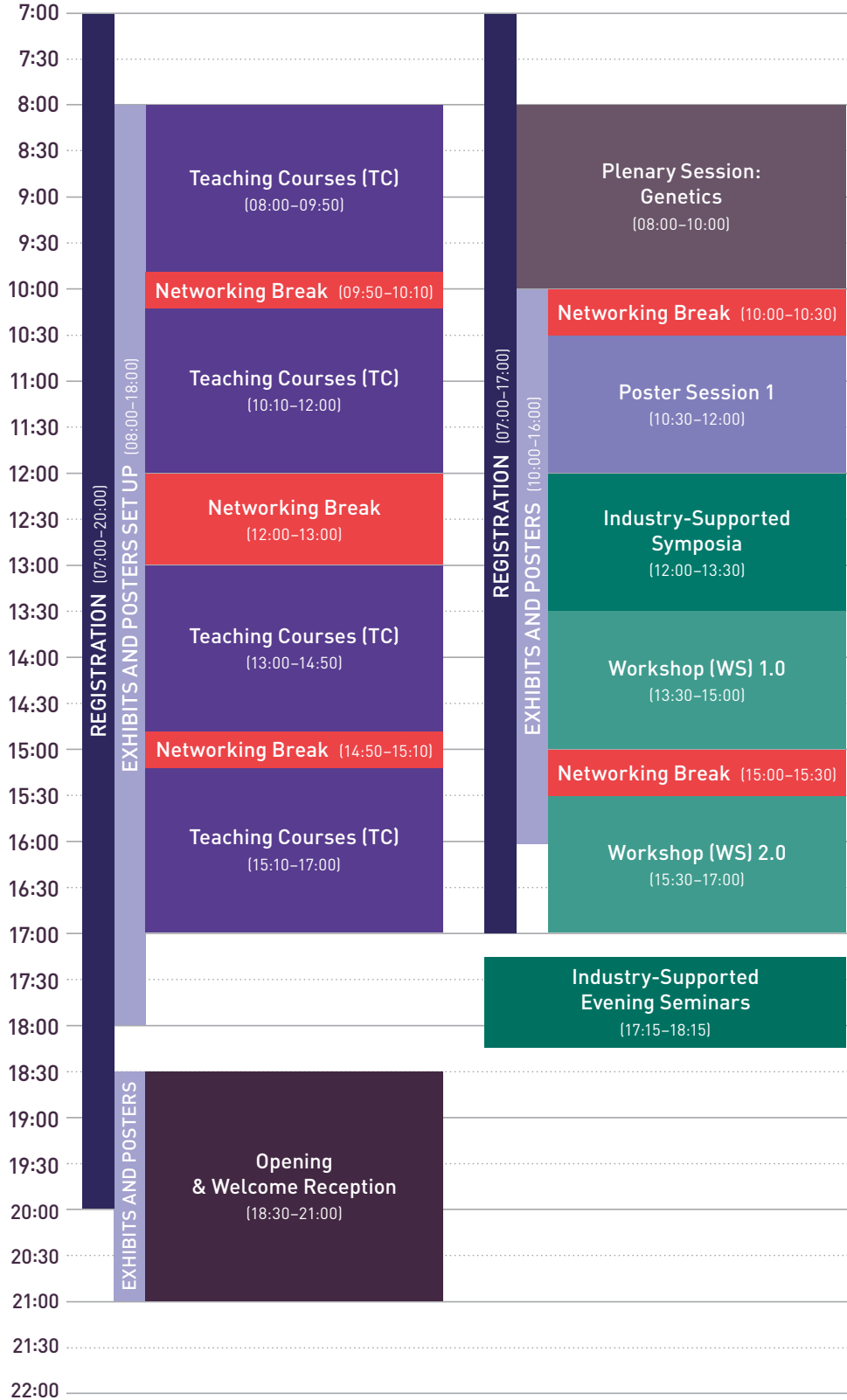
WEDNESDAY
JULY 6, 2016

REGISTRATION HOURS

- TUESDAY, JULY 5**
▶ 07:00–20:00
- WEDNESDAY, JULY 6**
▶ 07:00–17:00
- THURSDAY, JULY 7**
▶ 07:00–17:00
- FRIDAY, JULY 8**
▶ 07:00–19:00
- SATURDAY, JULY 9**
▶ 07:00–17:00

EXHIBIT HOURS

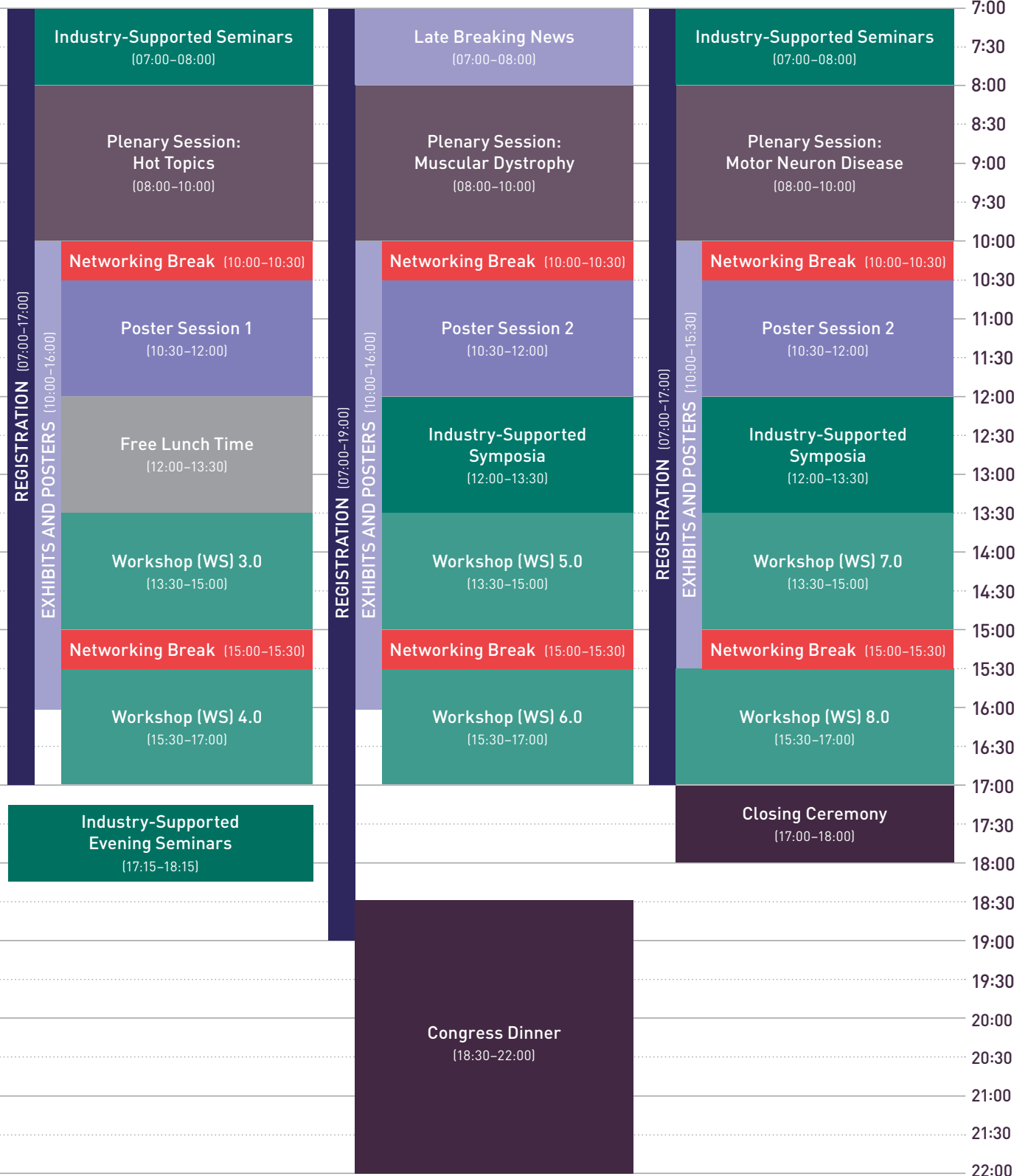
- WEDNESDAY, JULY 6**
▶ 10:00–16:00
- THURSDAY, JULY 7**
▶ 10:00–16:00
- FRIDAY, JULY 8**
▶ 10:00–16:00
- SATURDAY, JULY 9**
▶ 10:00–15:30



THURSDAY
JULY 7, 2016

FRIDAY
JULY 8, 2016

SATURDAY
JULY 9, 2016



PRE-CONGRESS TEACHING COURSES ▶ TUESDAY JULY 5, 2016

LOCATION ▶ All Teaching Course Sessions are located on the **2nd Floor of the Sheraton Centre Toronto Hotel.**

07:00–20:00 REGISTRATION OPEN

08:00–18:00 EXHIBITS AND POSTERS SET UP
LOCATION ▶ Exhibit Hall, Lower Concourse

08:00–09:50 TC 1.0 - Paediatric Muscular Dystrophy

LOCATION ▶ City Hall

Chairs: Kevin Flanigan, US
& Volker Straub, UK

08:00–08:55 1.1 CONGENITAL MYOPATHIES
James J. Dowling, CA

08:55–09:50 1.2 DYSTROPHINOPATHIES
Kevin Flanigan, US

09:50–10:10 NETWORKING BREAK

10:10–11:05 1.3 LIMB-GIRDLE DYSTROPHIES
Volker Straub, UK

11:05–12:00 1.4 AXIAL MYOPATHIES
Anthony A. Amato, US

08:00–09:50 TC 2.0 - Myasthenia Gravis

LOCATION ▶ Dominion North

Chairs: Carolina Barnett-Tapia, CA
& Gil I. Wolfe, US

08:00–08:55 2.1 CLINICAL ASSESSMENT OF MYASTHENIA GRAVIS
Carolina Barnett-Tapia, CA

08:55–09:50 2.2 ANTIBODY TESTING IN MYASTHENIA GRAVIS
Luis Querol, ES

09:50–10:10 NETWORKING BREAK

10:10–11:05 2.3 ELECTROPHYSIOLOGICAL TESTING IN MYASTHENIA GRAVIS
Hans D. Katzberg, CA

11:05–12:00 2.4 TREATMENT OF MYASTHENIA GRAVIS
Gil I. Wolfe, US

08:00–09:50 TC 3.0 - Genetics

LOCATION ▶ Churchill

Chair: Mary Reilly, UK

08:00–08:55 3.1 THE ABC'S OF GENETICS
Ronald D. Cohn, CA

08:55–09:50 3.2 EVALUATION OF VARIANTS OF UNKNOWN SIGNIFICANCE
Mary Reilly, UK

09:50–10:10 NETWORKING BREAK

10:10–11:05 3.3 THE ETHICAL IMPLEMENTATION OF GENOMIC MEDICINE
M. Stephen Meyn, US

11:05–12:00 3.4 GENETICS COUNSELLING
Jeanna McCuaig, CA

08:00–09:50 TC 4.0 - Non-immune Mediated Polyneuropathy

LOCATION ▶ Simcoe & Dufferin

Chairs: James Russell, US
& P. James Dyck, US

08:00–08:55 4.1 HEREDITARY POLYNEUROPATHY
Stephan Züchner, US

08:55–09:50 4.2 ASSESSMENT OF NEUROPATHY IN DIABETES AND PRE-DIABETES
James Russell, US

09:50–10:10 NETWORKING BREAK

10:10–11:05 4.3 TREATMENT OF POEMS
P. James Dyck, US

11:05–12:00 4.4 PARANEOPLASTIC NEUROPATHY
Kristine Chapman, CA

08:00–09:50 TC5.0 - Ultrasound in Neuromuscular Disorders

LOCATION ▶ Dominion South

Chairs: Francis O. Walker, US
& Ari Breiner, CA

08:00–08:25 5.1 INTRODUCTION TO ULTRASOUND
Ari Breiner, CA

08:25–08:50 5.2 ULTRASOUND OF FOCAL PERIPHERAL NEUROPATHIES
Francis O. Walker, US

08:50–09:15 5.3 ULTRASOUND OF DIFFUSE NEUROPATHIES & MOTOR NEURON DISEASE
Lisa Hobson-Webb, US

09:15–09:40 5.4 ULTRASOUND OF MUSCLE
Steven Shook, US

09:40–09:50 DISCUSSION PERIOD

09:50–10:10 NETWORKING BREAK

10:10–12:00 5.5 PRACTICAL DEMONSTRATIONS

- 10:10–10:45 Station 1**
Median and ulnar nerves (upper limb)
- 10:45–11:20 Station 2**
Fibular and tibial nerves (lower limb)
- 11:20–11:55 Station 3**
Muscle +/- diaphragm

Equipment for the Practical Demonstrations generously provided by GE Healthcare and Philips Healthcare

12:00–13:00 NETWORKING BREAK**13:00–17:00 TC 6.0 - Inflammatory Neuropathies co-sponsored with PNS****LOCATION**► City Hall

Chairs: Ingemar Merkies, NL
& Ivo van Schaik, NL

- 13:00–13:55 6.1 CLINICAL ASPECTS OF IMMUNE MEDIATED NEUROPATHIES**
Jean-Marc Léger, FR
- 13:55–14:50 6.2 CLINIMETRICS OF IMMUNE MEDIATED NEUROPATHIES**
Ingemar Merkies, NL

14:50–15:10 NETWORKING BREAK

- 15:10–16:05 6.3 PATHOGENESIS AND IMMUNOLOGY OF IMMUNE MEDIATED NEUROPATHIES**
Hans-Peter Hartung, DE
- 16:05–17:00 6.4 IMMUNOTHERAPY: WHAT TO DO FIRST, HOW TO START & STOP, WEAR OFF, LONG-TERM CONSIDERATIONS, LONG-TERM OUTCOME**
Ivo van Schaik, NL

13:00–17:00 TC 7.0 - ALS**LOCATION**► Churchill

Chairs: Angela Genge, CA
& Michael Benatar, US

- 13:00–13:55 7.1 EVALUATION OF PATIENTS WITH ALS**
Angela Genge, CA
- 13:55–14:50 7.2 THE ROLE OF GENETIC TESTING IN MOTOR NEURON DISEASE**
Matthew Harms, US

14:50–15:10 NETWORKING BREAK

- 15:10–16:05 7.3 BIOMARKERS RELEVANT TO ALS THERAPY DEVELOPMENT**
Michael Benatar, US
- 16:05–17:00 7.4 SYMPTOMATIC MANAGEMENT OF ALS**
Stacy Rudnicki, US

13:00–17:00 TC 8.0 - Myopathy**LOCATION**► Dominion North

Chair: Mark Tarnopolsky, CA

- 13:00–13:55 8.1 EVALUATION OF METABOLIC MYOPATHY**
Mark Tarnopolsky, CA
- 13:55–14:50 8.2 EXERCISE THERAPY IN MYOPATHY**
Ronni Haller, US

14:50–15:10 NETWORKING BREAK

- 15:10–16:05 8.3 TREATMENT OF MYOTONIC DYSTROPHY**
Charles Thornton, US
- 16:15–17:00 8.4 THE TREATMENT OF INFLAMMATORY MYOPATHY**
Anthony A. Amato, US

13:00–17:00 TC 9.0 - Autonomic Neuropathy**LOCATION**► Simcoe & Dufferin

Chairs: Eva L. Feldman, US
& Paola Sandroni, US

- 13:00–13:55 9.1 INTRODUCTION TO AUTONOMIC NEUROPATHY**
Eva L. Feldman, US
- 13:55–14:50 9.2 ASSESSMENT OF AUTONOMIC NEUROPATHY**
Pariwat Thaisethawatkul, US

14:50–15:10 NETWORKING BREAK

- 15:10–16:05 9.3 POSTURAL ORTHOSTATIC TACHYCARDIA SYNDROME**
Paola Sandroni, US
- 16:05–17:00 9.4 AUTOIMMUNE AUTONOMIC NEUROPATHY**
Steven Vernino, US

13:00–17:00 TC 10.0 - Adult Muscular Dystrophy and Myopathy**LOCATION**► Dominion South

Chairs: Jiri Vajsar, CA
& Mazen Dimachkie, US

- 13:00–13:55 10.1 UPDATE ON FASCIO-SCAPULO-HUMERAL DYSTROPHY (FSHD)**
Rabi Tawil, US
- 13:55–14:50 10.2 THE NEED FOR TRANSITION**
Jiri Vajsar, CA

14:50–15:10 NETWORKING BREAK

- 15:10–16:05 10.3 ADULT LIMB-GIRDLE DYSTROPHY**
Anthony A. Amato, US
- 16:05–17:00 10.4 INCLUSION BODY MYOSITIS**
Mazen Dimachkie, US

18:30–21:00 OPENING CEREMONY & WELCOME RECEPTION

LOCATION► Plenary Hall, Grand Ballroom Centre and Exhibit Hall, Lower Concourse

CONGRESS PROGRAM ► WEDNESDAY JULY 6, 2016

LOCATION► All Plenary & Workshop Sessions are located on the **Lower Concourse of the Sheraton Centre Toronto Hotel.**

07:00–17:00 REGISTRATION OPEN

10:00–16:00 EXHIBITS AND POSTERS OPEN
LOCATION► Exhibit Hall, Lower Concourse

08:00–10:00 Plenary Session PL 1.0
Genetics
ROOM► Plenary Hall, Grand Ballroom Centre, Lower Concourse level

Chair: James Dowling, CA

08:00–09:00 PL 1.1–GENOMIC APPROACHES TO
DIAGNOSIS OF RARE MUSCLE DISEASE
Keynote Speaker: Daniel MacArthur, US

09:00–09:30 PL 1.2–GENE DISCOVERY IN CHARCOT-
MARIE-TOOTH NEUROPATHIES
Stephan Züchner, US

09:30–10:00 PL 1.3–RNA SEQUENCE AND RNA
ANALYSIS
James Dowling, CA

10:00–10:30 NETWORKING BREAK
LOCATION► Exhibit Hall, Lower Concourse

10:30–12:00 Poster Session 1
ROOM► Exhibit Hall
See page 34 for poster information.

12:00–13:30 INDUSTRY-SUPPORTED SYMPOSIUM
See page 30 for information

13:30–15:00 Workshop WS 1.1
Imaging of Muscle
ROOM► Grand Ballroom Centre
Chair: Ari Breiner, CA

13:30–14:15 WS 1.1.1–MUSCLE ULTRASOUND
Carsten Bonnemann, US

14:15–15:00 WS 1.1.2–THE APPLICATION OF MRI IN
MUSCLE DISEASE
Volker Straub, UK

13:30–15:00 Workshop WS 1.2
Management of ALS Patients

ROOM► Osgoode Ballroom West

Chair: Hans Katzberg, CA

13:30–14:15 WS 1.2.1–SYMPTOMATIC TREATMENT
OF ALS
Stacy Rudnick, US

14:15–15:00 WS 1.2.2–END OF LIFE ISSUES IN ALS
Christen Shoesmith, US

13:30–15:00 Workshop WS 1.3
Metabolic Myopathy

ROOM► Grand Ballroom West

Chair: John Vissing, DE

13:30–14:15 WS 1.3.1–EVALUATION AND TREATMENT
OF POMPE DISEASE
Mark Tarnopolsky, US

14:15–15:00 WS 1.3.2–DIETARY AND OTHER
THERAPIES IN MUSCLE GLYCOGENOSIS
AND DISORDERS OF MUSCLE LIPID
OXIDATION
John Vissing, US

13:30–15:00 Workshop WS 1.4
Neuromuscular Databases

ROOM► Osgoode Ballroom East

Chair: Lawrence Korngut, CA

13:30–14:15 WS 1.4.1–NEUROMUSCULAR DATABASES
Lawrence Korngut, US

14:15–15:00 WS 1.4.2–TREAT NMD
Kevin Flanigan, US

**13:30–15:00 Workshop WS 1.5
Treatment of Myasthenia Gravis**

ROOM ▶ Grand Ballroom East

Chair: Susan Iannaccone, US

**13:30–14:15 WS 1.5.1–GENERAL TREATMENT
APPROACHES**

Gil Wolfe, US

**14:15–15:00 WS 1.5.2–TREATMENT OF MG IN THE
PAEDIATRIC POPULATION**

Susan Iannaccone, US

**15:00–15:30 NETWORKING BREAK
LOCATION** ▶ Exhibit Hall, Lower Concourse

**15:30–17:00 Workshop WS 2.1
Emerging Concepts in the Pathology
and Clinical Management of
Degenerative Cervical Myelopathy
(DCM)**

ROOM ▶ Osgoode Ballroom West

Chair: Michael Fehlings, CA

**15:30–15:45 WS 2.1.1–EMERGING CONCEPTS IN THE
PATHOBIOLOGY OF DEGENERATIVE
CERVICAL MYELOPATHY, EPIDEMIOLOGY
AND CLINICAL PRESENTATION**

Michael Fehlings, US

**15:45–16:00 WS 2.1.2–CLINICAL IMPLICATIONS,
OUTCOMES AND REHABILITATION
PATHWAYS**

Anthony Burns, US

**16:00–16:15 WS 2.1.3–UNDERSTANDING DISEASE
SEVERITY THROUGH NOVEL SURROGATE
MEASUREMENT APPROACHES IN NTSCI**

Sukhvinder Kalsi-Ryan, CA

**16:15–16:30 WS 2.1.4–ADVANCED TECHNIQUES IN
IMAGING SPECIFIC TO DEGENERATIVE
MYELOPATHY**

Julien Cohen-Adad, US

16:30–17:00 PANEL DISCUSSION

**15:30–17:00 Workshop WS 2.2
MRI Studies in Peripheral Nerve
Disease**

ROOM ▶ Grand Ballroom East

Chair: Ali Naraghi, CA

**15:30–16:15 WS 2.2.1–NOVEL PROCESSING METHODS
FOR PERIPHERAL NERVE IMAGING**

Jennifer Kollmer, DE

**16:15–17:00 WS 2.2.2–CHALLENGES IN MRI STUDIES
OF PERIPHERAL NERVES**

Ali Naraghi, US

**15:30–17:00 Workshop WS 2.3
Outcomes in Hereditary Neuropathy**

ROOM ▶ Grand Ballroom Centre

Chair: Mary Reilly, UK

15:30–16:15 WS 2.3.1–OUTCOMES IN CMT

Mary Reilly, UK

**16:15–17:00 WS 2.3.2–MONITORING HEREDITARY
NEUROPATHIES IN CLINICAL TRIALS**

Michael Shy, US

**15:30–17:00 Workshop WS 2.4
Role of Skin Punch Biopsy**

ROOM ▶ Osgoode Ballroom East

Chair: David Saperstein, US

**15:30–16:15 WS 2.4.1–ROLE OF SKIN PUNCH BIOPSY
IN CLINICAL PRACTICE**

David Saperstein, US

**16:15–17:00 WS 2.4.2–ROLE OF SKIN PUNCH BIOPSY
AS A RESEARCH OUTCOME MEASURE**

Michael James Polydefkis, US

**15:30–17:00 Workshop WS 2.5
Ultrasound in Peripheral Nerve
Disease, Upper Limb**

ROOM ▶ Grand Ballroom West

**15:30–17:00 WS 2.5.1–PRACTICAL DEMONSTRATION
AND DISCUSSION**

Francis O. Walker, US

Equipment for the Practical Demonstration
generously provided by GE Healthcare and
Philips Healthcare

17:15–18:15 INDUSTRY-SUPPORTED SYMPOSIUM
See page 30 for information

CONGRESS PROGRAM ▶ THURSDAY JULY 7, 2016

LOCATION ▶ All Plenary & Workshop Sessions are located on the **Lower Concourse of the Sheraton Centre Toronto Hotel.**

07:00–17:00 REGISTRATION OPEN

07:00–08:00 INDUSTRY-SUPPORTED SYMPOSIA
See page 31 for information

10:00–16:00 EXHIBITS AND POSTERS OPEN
LOCATION ▶ Exhibit Hall, Lower Concourse

10:30–12:00 Poster Session 1

ROOM ▶ Exhibit Hall

See page 34 for poster information.

12:00–13:30 INDUSTRY-SUPPORTED SYMPOSIUM
See page 31 for information

08:00–10:00 Plenary Session PL 2.0
Hot Topics
ROOM ▶ Plenary Hall, Grand Ballroom
Centre, Lower Concourse level

Chairs: Vera Bril, CA
& Carlos Navarrete Maldonado, CL

08:00–08:15 PL 2.1–STEM CELL THERAPY IN ALS
Eva Feldman, US

**08:15–08:30 PL 2.2–RESULTS OF THE THYMECTOMY
TRIAL IN MYASTHENIA GRAVIS**
Gil Wolfe, US

**08:30–08:45 PL 2.3–REGAIN: A RANDOMIZED,
DOUBLE-BLIND, PLACEBO-CONTROLLED
MULTI-CENTER PHASE 3 STUDY OF THE
SAFETY AND EFFICACY OF ECULIZUMAB
IN SUBJECTS WITH REFRACTORY
GENERALIZED MYASTHENIA GRAVIS**
James Howard, Jr., US

**08:45–09:00 PL 2.4–APPROACH TO PATIENT-
CENTERED OUTCOMES RESEARCH**
Richard Barohn, US

**09:00–09:15 PL 2.5–DO WE STILL NEED MUSCLE
BIOPSY IN THE ERA OF ULTRASOUND?**
Carsten Bonnemann, US

**09:15–09:30 PL 2.6–THERAPEUTIC APPROACHES TO
INCLUSION BODY MYOSITIS**
Mazen Dimachkie, US

**09:30–09:45 PL 2.7–TREATMENT OF AMYLOID
NEUROPATHY**
David Adams, FR

09:45–10:00 PANEL DISCUSSION

10:00–10:30 NETWORKING BREAK
LOCATION ▶ Exhibit Hall, Lower Concourse

13:30–15:00 Workshop WS 3.1
**Challenges in Design of Investigator-
Initiated Research**

ROOM ▶ Osgoode Ballroom West

Chair: Richard Barohn, US

**13:30–14:15 WS 3.1.1–CHALLENGES FOR
INVESTIGATOR INITIATED TRIALS AND
FOR CONDUCTING MULTICENTER TRIALS**
Richard Barohn, US

14:15–15:00 WS 3.1.2–TRANSATLANTIC CHALLENGES
Richard Barohn, US

13:30–15:00 Workshop WS 3.2
**Diagnosis and Treatment of Myotonic
Dystrophy**

ROOM ▶ Grand Ballroom East

Chair: Charles Thornton, US

**13:30–14:15 WS 3.2.1–HOW TO TREAT MYOTONIC
DYSTROPHY**
Charles Thornton, US

**14:15–15:00 WS 3.2.2–CURRENT KNOWLEDGE OF
DISEASE PROGRESSION IN MYOTONIC
DYSTROPHY**
Richard Moxley, III, US

13:30–15:00 Workshop WS 3.3
Modern Concepts in Genetics

ROOM ▶ Grand Ballroom Centre

Chair: Kevin Flanigan, US

**13:30–14:15 WS 3.3.1–MOLECULAR DIAGNOSTICS IN
THE NEUROMUSCULAR CLINIC**
Grace Yoon, CA

14:15–15:00 WS 3.3.2
Kevin Flanigan, US

13:30–15:00 **Workshop**
WS 3.4 – Small Fibre Neuropathy

ROOM ▶ Osgoode Ballroom East

Chair: Giuseppe Lauria, IT

13:30–14:15 **WS 3.4.1–AMYLOID NEUROPATHY AS A MODEL OF SMALL FIBER NEUROPATHY**

David Adams, FR

14:15–15:00 **WS 3.4.2–DIAGNOSIS OF SMALL FIBRE NEUROPATHY**

Giuseppe Lauria, IT

13:30–15:00 **Workshop**
WS 3.5 – Ultrasound in Peripheral Nerve Disease, Lower Limb

ROOM ▶ Grand Ballroom West

Chair: Francis O. Walker, US

13:30–15:00 **WS 3.5.1–PRACTICAL DEMONSTRATION AND DISCUSSION**

Francis O. Walker, USA

Equipment for the Practical Demonstration generously provided by GE Healthcare and Philips Healthcare

15:00–15:30 **NETWORKING BREAK**
LOCATION ▶ Exhibit Hall, Lower Concourse

15:30–17:00 **Workshop WS 4.1**
Bioinformatics and Clinical Research

ROOM ▶ Grand Ballroom West

Chair: Jon Katz, US

15:30–16:15 **WS 4.1.1–TECHNOLOGY PLATFORMS FOR COLLABORATIONS IN CLINICAL RESEARCH**

Alexander Sherman, US

16:15–17:00 **WS 4.1.2–USING THE ELECTRONIC MEDICAL RECORD FOR RESEARCH**

Jon Katz, US

15:30–17:00 **Workshop WS 4.2**
Controversies Over Large Nerve Biopsy

ROOM ▶ Osgoode Ballroom West

Chair: Anthony A. Amato, US

15:30–16:15 **WS 4.2.1–NERVE BIOPSY ARE RARELY NEEDED**

Anthony A. Amato, US

16:15–17:00 **WS 4.2.2–IT IS VALUABLE**

P James Dyck, US

15:30–17:00 **Workshop WS 4.3**
Genetics of Hereditary Polyneuropathy

ROOM ▶ Grand Ballroom East

Chair: Stephan Züchner, US

15:30–16:15 **WS 4.3.1–OVERVIEW OF GENETICS OF HEREDITARY POLYNEUROPATHY**

Stephan Züchner, US

16:15–17:00 **WS 4.3.2–CELLULAR REPROGRAMMING AND INHERITED PERIPHERAL NEUROPATHIES: PERSPECTIVES AND CHALLENGES**

Mario Saporta, US

15:30–17:00 **Workshop WS 4.4**
Outcome Measures in Inflammatory Neuropathy

ROOM ▶ Grand Ballroom Centre

Chair: Jean-Marc Léger, FR

15:30–15:42 **WS 4.4.1–WHAT THE PERINOMS STUDY TAUGHT US**

Ingemar Merkies, NL

15:42–15:54 **WS 4.4.2–HOW WE SHOULD ASSESS INFLAMMATORY NEUROPATHY**

Jean-Marc Léger, FR

15:30–17:00 **Workshop WS 4.5**
Primer for Genetic Testing

ROOM ▶ Osgoode Ballroom East

Chair: James Dowling, CA

15:30–16:15 **WS 4.5.1–EVALUATION OF VARIANTS OF UNKNOWN SIGNIFICANCE**

Mary Reilly, UK and James Dowling, CA

16:15–16:25 **WS 4.5.2–CLINICAL WHOLE EXOME SEQUENCING**

Livija Medne, US

16:25–16:45 **WS 4.5.2–GENE PANELS**

Kimberly Amburgey, CA

17:15–18:15 **INDUSTRY-SUPPORTED SYMPOSIUM**
See page 31 for information

CONGRESS PROGRAM ► FRIDAY JULY 8, 2016

LOCATION► All Plenary & Workshop Sessions are located on the **Lower Concourse of the Sheraton Centre Toronto Hotel.**

07:00–20:00 REGISTRATION OPEN

10:00–16:00 EXHIBITS AND POSTERS OPEN
LOCATION► Exhibit Hall, Lower Concourse

12:00–13:30 INDUSTRY-SUPPORTED SYMPOSIUM
See page 32 for more information

- 07:00–08:00 Late Breaking News LB 1.0**
ROOM► Plenary Hall, Grand Ballroom Centre, Lower Concourse level
Chairs: Vera Bril, CA & John England, US
- 07:00–07:20 LB 1.1–A PHASE 2 TRIAL OF RITUXIMAB IN MYASTHENIA GRAVIS: STUDY UPDATE**
Richard J. Nowak, US
- 07:20–07:40 LB 1.2–NEUROLOGICAL COMPLICATIONS ON ZIKA VIRUS**
John England, US
- 07:40–08:00 LB 1.3–GUILLAIN-BARRE SYNDROME AND VARIANTS ASSOCIATED WITH ZIKA VIRUS OUTBREAKS**
Osvaldo Nascimento, Brazil

- 08:00–10:00 Plenary Session PL 3.0 Muscular Dystrophy**
ROOM► Plenary Hall, Grand Ballroom Centre, Lower Concourse level
- 08:00–08:30 PL 3.1–GENE THERAPY FOR MUSCULAR DYSTROPHY**
Keynote Speaker: Dongsheng Duan, US
- 08:30–09:00 PL 3.1–RNA THERAPEUTICS FOR DUCHENNE MUSCULAR DYSTROPHY**
Keynote Speaker: Dana Martin, US
- 09:00–09:30 PL 3.2–ANTISENSE THERAPY FOR MYOTONIC DYSTROPHY**
Charles Thornton, US
- 09:30–10:00 PL 3.3–CRISPR BASED GENE EDITING FOR MUSCULAR DYSTROPHY**
Ronald Cohn, CA

10:00–10:30 NETWORKING BREAK
LOCATION► Exhibit Hall, Lower Concourse

- 10:30–12:00 Poster Session 2**
ROOM► Exhibit Hall
See page 34 for poster information.

13:30–15:00 Workshop WS 5.1 Approach to Muscular Dystrophies

ROOM► Grand Ballroom Centre

Chair: Carsten Bonnemann, US

13:30–14:15 WS 5.1.1–THE CLINICIANS APPROACH TO LIMB GIRDLE MUSCULAR DYSTROPHY (LGMD)
Volker Straub, UK

14:15–15:00 WS 5.1.2–CONGENITAL MUSCULAR DYSTROPHIES
Carsten Bonnemann, US

13:30–15:00 Workshop WS 5.2 Interesting Neuromuscular Cases

ROOM► Grand Ballroom East

Chairs: Aaron Izenberg, CA & Hans Katzberg, CA

13:30–13:45 WS 5.2.1–A 47-YEAR-OLD FEMALE PATIENT WITH SLOW PROGRESSIVE DISTAL AND ASYMMETRIC WEAKNESS
Renata Andrade, BR

13:45–14:00 WS 5.2.2–A PATIENT WITH DISTAL WEAKNESS, CRAMPS AND FAINTING, OCULAR MOVEMENT ABNORMALITY
Corrado Angelini, IT

14:00–14:15 WS 5.2.3–IN THE ERA OF EXON SEQUENCING HOW DO WE MANAGE THE PROGRESSIVE PROXIMAL, AXIAL AND FACIAL WEAKNESS LEADING TO COMPLETE BULBAR PALSY AND TONGUE FASCICULATIONS IN CHILDREN?
Elena Gargaun, FR

14:15–14:30 WS 5.2.4–MYOPATHY WITH HYPERCKEMIA AND GLOBAL DEVELOPMENTAL DELAYS: THINK BEYOND THE ALPHA-DYTROGLYCONOPATHIES
Livija Medne, US

14:30–14:45 WS 5.2.5–CRAMP-FASCICULATION SYNDROME - AN UNEXPECTED ETIOLOGY
Peter Y.K. Van den Bergh, BY

14:45–15:00 WS 5.2.6–A CASE OF VELOPHARYNGEAL INSUFFICIENCY
Veena Vasi, UK

13:30–15:00 **Workshop WS 5.3**
Diagnosis and Treatment of Diabetic Neuropathy

ROOM ▶ Osgoode Ballroom West

Chair: Bruce Perkins, CA

13:30–14:15 **WS 5.3.1–DIAGNOSIS AND TREATMENT OF DIABETIC NEUROPATHY**

Bruce Perkins, CA

14:15–15:00 **WS 5.3.2–THE USE OF OMEGA-3 SUPPLEMENTATION FOR MANAGING DIABETIC NEUROPATHY: RESULTS FROM A CLINICAL PILOT TRIAL**

Evan Lewis, CA

13:30–15:00 **Workshop WS 5.4**
Neuropathic Pain

ROOM ▶ Grand Ballroom West

Chair: John England, US

13:30–14:15 **WS 5.4.1–THE ROLE OF GUIDELINES IN DECISIONS ON TREATMENT**

John England, US

14:15–15:00 **WS 5.4.2–UPDATE ON TREATMENT OF NEUROPATHIC PAIN**

Jaya Trivedi, US

13:30–15:00 **Workshop WS 5.5**
Outcome Scales in Myasthenia Gravis

ROOM ▶ Osgoode Ballroom East

Chair: Ted Burns, US

13:30–14:15 **WS 5.5.1–MYASTHENIA GRAVIS IMPAIRMENT INDEX**

Carolina Barnett Tapia, CA

14:15–15:00 **WS 5.5.2–REVIEW OF CURRENT MG SCALES**

Ted Burns, US

15:00–15:30 **NETWORKING BREAK**
LOCATION ▶ Exhibit Hall, Lower Concourse

15:30–17:00 **Workshop WS 6.1**
ALS Overlap Syndromes

ROOM ▶ Grand Ballroom East

Chair: Maria Carmela Tartaglia, CA

15:30–16:15 **WS 6.1.1–GENETIC ASPECTS OF ALS OVERLAP SYNDROMES**

Ekaterina Rogaeva, CA

16:15–17:00 **WS 6.1.2–CLINICAL ASPECTS OF ALS OVERLAP SYNDROMES**

Maria Carmela Tartaglia, CA

15:30–17:00 **Workshop**
WS 6.2 – Cramps in Neuromuscular Disease

ROOM ▶ Grand Ballroom Centre

Chair: Nicholas Silvestri, US

15:30–16:15 **WS 6.2.1–TREATMENT OF MUSCLE CRAMPS**

Hans Katzberg, CA

16:15–17:00 **WS 6.2.2–ASSESSMENT OF MUSCLE CRAMPS**

Nicholas Silvestri, US

15:30–17:00 **Workshop WS 6.3**
Diabetic Neuropathy

ROOM ▶ Osgoode Ballroom East

Chair: James Russell, US

15:30–16:15 **WS 6.3.1–EPIDEMIOLOGY AND PATHOPHYSIOLOGY OF DIABETIC NEUROPATHY**

James Russell, US

16:15–17:00 **WS 6.3.2–TREATMENT OF DIABETIC NEUROPATHY**

Vera Bril, CA

15:30–17:00 **Workshop WS 6.4**
Peripheral Nerve Tumors

ROOM ▶ Osgoode Ballroom West

Chair: Wolfgang Grisold, AT

15:30–16:15 **WS 6.4.1–LYMPHOMA AND OTHER PERIPHERAL NERVE TUMORS**

Wolfgang Grisold, AT

16:15–17:00 **WS 6.4.2–NEUROFIBROMATOSIS 1 AND MALIGNANT TRANSFORMATION OF PERIPHERAL NERVE SHEATH TUMORS**

Gelareh Zadeh, US

15:30–17:00 **Workshop WS 6.5**
Ultrasound of Muscle and Nerve

ROOM ▶ Grand Ballroom West

Chair: Ari Breiner, CA

15:30–16:15 **WS 6.5.1–NEUROMUSCULAR PHYSICIANS SHOULD PERFORM NM ULTRASOUND**

Steven Shook, US

16:15–17:00 **WS 6.5.2–RADIOLOGISTS SHOULD PERFORM NM ULTRASOUND**

Linda Probyn, US

17:00–22:00 **CONGRESS DINNER**

LOCATION ▶ Willow East & Centre located at the Sheraton on the Mezzanine level

CONGRESS PROGRAM ▶ SATURDAY JULY 9, 2016

LOCATION ▶ All Plenary & Workshop Sessions are located on the **Lower Concourse of the Sheraton Centre Toronto Hotel.**

07:00–19:00	REGISTRATION OPEN	13:30–14:15	WS 7.1.1–IGG4 AUTOANTIBODIES RELATED TO NEUROMUSCULAR DISEASES: THERAPEUTIC IMPLICATIONS Luis Querol, ES
07:00–08:00	INDUSTRY-SUPPORTED SYMPOSIA See page 33 for information	14:15–15:00	WS 7.1.2–IS IT ALL ABOUT THE ANTIBODIES Andrew Mammen, US
10:00–15:30	EXHIBITS AND POSTERS OPEN LOCATION ▶ Exhibit Hall, Lower Concourse		
08:00–10:00	Plenary Session PL 4.0 Motor Neuron Disease ROOM ▶ Plenary Hall, Grand Ballroom Centre, Lower Concourse level Chair: John Kissel, US	13:30–15:00	Workshop WS 7.2 Evaluation of Variants of Unknown Significance ROOM ▶ Osgoode Ballroom East Chair: Mary Reilly, UK
08:00–09:00	PL 4.1–ALS THERAPY DEVELOPMENT: CHALLENGES AND OPPORTUNITIES Keynote Speaker: Michael Benatar, US	13:30–14:15	WS 7.2.1–NERVE Mary Reilly, UK
09:00–09:30	PL 4.2–BIOLOGY OF C9ORF72 DISEASE Leonard Petrucelli, US	14:15–15:00	WS 7.2.2–MUSCLE Raveen Basran, CA
09:30–10:00	PL 4.3–ANTISENSE THERAPY FOR SPINAL MUSCULAR ATROPHY John Kissel, US	13:30–15:00	Workshop WS 7.3 Inclusion Body Myopathy ROOM ▶ Grand Ballroom East Chair: Mazen Dimachkie, US
10:00–10:30	NETWORKING BREAK LOCATION ▶ Exhibit Hall, Lower Concourse	13:30–14:15	WS 7.3.1–GENERAL TREATMENT APPROACHES Mazen Dimachkie, US
10:30–12:00	Poster Session 2 ROOM ▶ Exhibit Hall See page 34 for poster information.	14:15–15:00	WS 7.3.2–ONGOING DEVELOPMENTS IN IBM Anthony A. Amato, US
12:00–13:30	INDUSTRY-SUPPORTED SYMPOSIUM See page 33 for information	13:30–15:00	Workshop WS 7.4 Modern Concepts in Spinal Muscular Atrophy ROOM ▶ Grand Ballroom West Chair: Susan Iannaccone, US
13:30–15:00	Workshop WS 7.1 Autoantibodies in Neuromuscular Disease ROOM ▶ Grand Ballroom Centre Chair: Luis Querol, ES	13:30–14:15	WS 7.4.1–SMA TODAY Susan Iannaccone, US
		14:15–15:00	WS 7.4.2–UPDATE ON SPINAL MUSCULAR ATROPHY John Kissel, US

13:30–15:00 **Workshop WS 7.5**
Update on FSHD

ROOM ▶ Osgoode Ballroom West

Chair: Rabi Tawil, US

13:30–14:15 **WS 7.5.1–RECENT CONCEPTS IN FSHD**
Rabi Tawil, US

14:15–15:00 **WS 7.5.2–CLINICAL PRESENTATION IN FSHD**

Jeffrey Statland, US

15:00–15:30 **NETWORKING BREAK**
LOCATION ▶ Exhibit Hall, Lower Concourse

15:30–17:00 **Workshop WS 8.1**
Exercise Therapy for Metabolic Myopathies

ROOM ▶ Osgoode Ballroom East

Chair: John Vissing, DE

15:30–16:15 **WS 8.1.1–EXERCISE THERAPY IN MITOCHONDRIAL DISORDERS**

Ronni Haller, US

16:15–17:00 **WS 8.1.2–EXERCISE TRAINING AND PATHOPHYSIOLOGY OF EXERCISE IN METABOLIC MYOPATHIES**

John Vissing, DE

15:30–17:00 **Workshop WS 8.2**
How To Do Investigator-Initiated Trials; PCORI

ROOM ▶ Osgoode Ballroom West

Chair: Richard Barohn, US

15:30–16:15 **WS 8.2.1–WHY PATIENT-CENTERED OUTCOMES RESEARCH?**

Richard Barohn, US

16:15–17:00 **WS 8.2.2–INVESTIGATOR-INITIATED CLINICAL TRIALS**

Richard Barohn, US

15:30–17:00 **Workshop WS 8.3**
Metabolic Neuropathies

ROOM ▶ Grand Ballroom West

Chair: A. Gordon Smith, US

15:30–16:15 **WS 8.3.1–NEUROPATHY IN PRE-DIABETES & THE METABOLIC SYNDROME**

A. Gordon Smith, US

16:15–17:00 **WS 8.3.2–NEUROPATHY DUE TO SYSTEMIC DISEASE**

Mamatha Pasnoor, US

15:30–17:00 **Workshop WS 8.4**
Outcome Measures in Neuromuscular Disorders

ROOM ▶ Grand Ballroom Centre

Chair: Linda Lowes, US

15:30–16:15 **WS 8.4.1–BEST OUTCOME MEASURES TO USE FOR NM PATIENTS**

Linda Lowes, US

16:15–17:00 **WS 8.4.2–OUTCOME MEASURES IN MUSCULAR DYSTROPHY**

Craig McDonald, US

15:30–17:00 **Workshop WS 8.5**
Treatment of Muscular Dystrophy

ROOM ▶ Grand Ballroom East

Chair: Kevin Flanigan, US

15:30–15:42 **WS 8.5.1–GENE-DIRECTED TREATMENT OF MUSCULAR DYSTROPHY**

Kevin Flanigan, US

15:42–15:54 **WS 8.5.2–NON-GENE DIRECTED**

Craig Campbell, CA

17:00–19:00 **CLOSING CEREMONY**
LOCATION ▶ Plenary Hall,
Grand Ballroom Centre,
Lower Concourse



ICNMD

14th International Congress on Neuromuscular Diseases

**➤ Congress
Information**

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DELEGATE HELP DESK / TORONTO TOURISM INFORMATION DESK

Located in the registration area on the [lower concourse level](#).

HOURS

MONDAY, JULY 4	12:30 – 17:00
TUESDAY, JULY 5	07:00 – 20:00
WEDNESDAY, JULY 6	07:00 – 17:00
THURSDAY, JULY 7	07:00 – 17:00
FRIDAY, JULY 8	07:00 – 19:00
SATURDAY, JULY 9	07:00 – 17:00

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DELEGATE LOUNGE

Located in the [Exhibit Hall](#).

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EMERGENCY SERVICES AND FIRST AID

Emergency Number: 911 The hotel also has a direct emergency line: 4977 which is accessible from any house phone. The hotel security team is equipped with first aid and is CPR-certified.



LUNCH

Lunch is not provided by the congress, however there are many nearby shops and restaurants within walking distance and within the Sheraton Centre. See 'Restaurants' for more information. There is also access to the food courts via the path located on the [concourse level](#).



MOBILE APP

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- Check into a session (1 pt) and post a comment (1 pt)
- Comment on the Activity Wall (2pts)
- Like a post you see in the Activity Wall (1pt)

At the Closing Ceremony on Saturday, twenty people with the highest score will be entered into a draw to win an iPad mini.

For attendees using the HTML5 versions, you can access your notifications by navigating to your profile, where you will see a number in the top right that indicates your current notifications. Tap that number and you will be taken to all your notifications. Please also use the Tweet! and Facebook tab within your menu to connect with Twitter and Facebook on your HTML5 version.



NETWORKING BREAKS

Tea, coffee and a light snack will be provided at the following times in the **Exhibit Hall**:

HOURS

TUESDAY, JULY 5	09:50 – 10:10 and 14:50 – 15:10
WEDNESDAY, JULY 6	10:00 – 10:30 and 15:00 – 15:30
THURSDAY, JULY 7	10:00 – 10:30 and 15:00 – 15:30
FRIDAY, JULY 8	10:00 – 10:30 and 15:00 – 15:30
SATURDAY, JULY 9	10:00 – 10:30 and 15:00 – 15:30



OFFICIAL SOCIAL MEDIA ACCOUNTS

Join the Conversation @ICNMD2016

Share your experience and images from ICNMD 2016 with colleagues and friends via Twitter and Facebook. Our official social media feeds throughout the Congress can be followed @ICNMD2016. We encourage you to tag your posts with #ICNMD2016.



RESTAURANTS

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

Restaurants within the hotel include:

Shopsy's Deli

Located on the lobby level in the Sheraton Centre Toronto and open seven days a week, Shopsy's Deli has been a favourite Toronto deli since 1921.

Link Café

The perfect spot to relax over a cup of coffee while planning the day's activities or checking email with complimentary high-speed Internet, or grab one to go.

BnB Restaurant

Sheraton Centre's signature restaurant BnB is a contemporary bistro and bar that features the best burgers in the market alongside classic comfort foods.

Quinn's Steakhouse and Irish Bar

Located on the lobby level of the hotel, and open seven days a week, Quinn's Steakhouse and Irish Bar is a Toronto steak restaurant featuring excellent sirloins, prime rib, seafood and classic pub dishes.



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 Sheraton Centre. Smoking is prohibited within six (6) metres of any entryway, openable window or air intake of a building.



STAFF AND VOLUNTEERS

Volunteers are happy to assist with any questions delegates may have regarding the Congress or the Sheraton Centre. Delegates can easily locate them by their staff/volunteer shirts.

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TRAVEL AWARD

The ICNMD 2016 Travel Award is presented to **Dr. Seena Vengalil from the National Institute of Mental Health and Neurosciences in India.**

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INDUSTRY-SUPPORTED SYMPOSIA (ISS)

WEDNESDAY JULY 6

12:00-13:30

ROOM► Grand Ballroom Centre

LUNCHEON SYMPOSIUM SUPPORTED BY

SANOFI GENZYME 

Pompe Medical Expert Symposium

Chair & Moderator: Dr. Vera Bril, University of Toronto, ON

- Introduction of Late Onset Pompe Disease:
Dr. Mark Tarnopolsky, McMaster University, Hamilton, ON
- Diagnostic Strategies of LGMW Including Next Generation Sequencing:
Dr. Matthew Harms, Columbia University, New York, NY

17:15-18:15

ROOM► Osgoode Ballroom West

EVENING SYMPOSIUM SUPPORTED BY


bridge to a stronger tomorrow

Multi-pathway Approach to DMD Treatment

Chair: Diana Escolar

- Antifibrotics: HT-100 For The Treatment Of DMD:
Diana Escolar, MD, Chief Medical Officer,
Akashi Therapeutics, Inc.
- Modulating Calcium Homeostasis: AT-300 Effects In MDX Model Of DMD:
Ernest D. Bush, Ph.D, Chief Scientific Officer,
Akashi Therapeutics, Inc.
- A Novel Approach To Building Muscle In DMD: DT-200, Selective Androgen Receptor Modulator:
Diana Escolar, MD, Chief Medical Officer,
Akashi Therapeutics, Inc.

07:00-8:00

ROOM► Grand Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY



The immune-modulatory role of plasma exchange in PNS & Neuromuscular diseases: A Presentation & clinical debate

- Plasma exchange immune-modulation & mechanism of action:
Prof. Hans-Peter Hartung, Heinrich-Heine-Universität, Düsseldorf, Germany
- Clinical debate on the place of plasma exchange in PNS & neuromuscular diseases (acute & chronic), addressing current evidence & unmet medical needs:
 - **Prof. Jean-Marc Leger**, MD, FAAN, University Hospital Pitié-Salpêtrière, Paris, France
 - **Prof. Mazen Dimachkie**, University of Kansas Medical Center, Kansas City, KS

07:00-8:00

ROOM► Osgoode Ballroom East

BREAKFAST SYMPOSIUM SUPPORTED BY



Updates in Myasthenia Gravis

Chair: Dr. Vera Bril, University Health Network/
Mount Sinai Hospital, ON

- Novel Clinical Assessment Methods in Myasthenia Gravis:
Dr. Carolina Barnett, University Health Network and University of Toronto, ON
- Recent Updates in the Treatment of Myasthenia Gravis:
Dr. Zaeem A. Siddiqi, University of Alberta Hospital, Edmonton, AB

07:00-8:00

ROOM► Osgoode Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY



Advances in Myositis: Immune Mechanisms of Disease, Current Clinical Practice and Future Directions

Moderator: Kirsten Gruis, MD, Senior Medical Director, Rare Diseases, Idera Pharmaceuticals

- Toll-Like Receptors and Innate Immune Mechanisms of Muscle Disease:
Kanneboyina Nagaraju, DVM, PhD, George Washington University School of Medicine and Health Sciences; Children's National Medical Center, Washington, DC
- Current Understanding of Standards of Care and Management of Inflammatory Myopathies:
Anthony A. Amato, MD, Brigham and Women's Hospital; Harvard Medical School, Boston, MA

17:15-18:15

ROOM► Osgoode Ballroom West

EVENING SYMPOSIUM SUPPORTED BY



The Patient Journey: From symptom onset to new treatment horizons in hereditary ATTR amyloidosis with polyneuropathy (hATTR-PN)

Chair: Giuseppe Vita, MD, Italy

- Patient journey and unmet medical needs in hATTR-PN
- Natural history, treatment options and clinical assessment tools in hATTR-PN
- Development of RNAi therapeutics as a potential treatment option for patients with hATTR-PN

Presenters:

- **Michelle Mezei**, MD (Canada)
- **P. James Dyck**, MD (USA)
- **Alejandra Gonzalez Duarte**, MD (Mexico)

FRIDAY JULY 8

12:00-13:30

ROOM► Osgoode Ballroom West

LUNCHEON SYMPOSIUM SUPPORTED BY



The Evolving Management of Duchenne Muscular Dystrophy

Chair: Jean K. Mah

- The Evolving Understanding of Clinical End Points and Meaningfulness:
Jean K. Mah, MD, MSc, FRCPC, University of Calgary;
Alberta Children's Hospital, Calgary, AB
- The Future of Duchenne Treatments:
Perry Shieh, MD, PhD, David Geffen School of Medicine
at UCLA, Los Angeles, CA

07:00-8:00

ROOM► Grand Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY



Recognizing the Patient with Refractory Myasthenia Gravis: An Interactive Workshop

Moderator: Karen Gondek

- Refractory MG
- Assessment tools and MG-ADL

Presenters:

- **Srikanth Muppidi**, MD, Stanford School of Medicine, Stanford, CA
- **Richard J. Nowak**, MD, MS, Yale School of Medicine, New Haven, CT
- **Nicholas J. Silvestri**, MD, University at Buffalo School of Medicine and Biomedical Sciences, Buffalo, NY
- **Gil I. Wolfe**, MD, FAAN, University at Buffalo School of Medicine and Biomedical Sciences, Buffalo, NY

07:00-8:00

ROOM► Osgoode Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY



Measuring response and need to continue treatment in CIDP: Moving from ICE trial outcome measures to future clinical biomarker

Hans Katzberg, MD, University of Toronto, Toronto, ON

- Residual conduction block after IVIG as a predictor of relapse in CIDP:
Norman Latov, MD, Weill Cornell Medical Center, New York, NY

- Real-time ultrasound monitoring of same lesions as a biomarker of therapeutic efficacy in CIDP:
Francis Walker, MD, Wake Forest University, Winston-Salem, NC

12:00-13:30

ROOM► Grand Ballroom West

LUNCHEON SYMPOSIUM SUPPORTED BY



Advances in pulmonary care in DMD

Chair: Oscar H Mayer

- Overview of respiratory function measures:
Oscar H. Mayer, The Children's Hospital of Philadelphia, PA
- The natural history of respiratory functional decline in DMD:
Erik Henricson, Physical Medicine and Rehabilitation Neuromuscular Research Center, Sacramento, CA
- Current standards of care and future perspectives:
Oscar H. Mayer
- Treatment strategies to slow decline in respiratory function in DMD - Report of the DELOS Phase III trial:
Gunnar Buyse, University of Leuven, Belgium

POSTER SESSIONS

BEST POSTERS

Top 10 Best Posters will be displayed on the Electronic Poster Screens during the entire Congress:

SCREEN 1

PS2Group1-055 ANTISENSE TARGETING OF 3' END ELEMENTS INVOLVED IN DUX4 MRNA PROCESSING IS AN EFFICIENT THERAPEUTIC STRATEGY FOR FACIOSCAPULOHUMERAL DYSTROPHY: A NEW GENE SILENCING APPROACH
Anne-Charlotte Marsollier¹, Lucasz Ciszewski², Virginie Mariot¹, Linda Popplewell², Thomas Voit², George Dickson², Julie Dumonceaux¹; ¹Paris, FR, ²London, UK

PS1Group8-002 REDUCTION OF ISOAGGLUTININS IN IVIG BY ANTI-A DONOR SCREENING REDUCES THE RISK OF HEMOLYTIC EVENTS
Ayman Kafal, Montreal, QC, CA

SCREEN 2

PS2Group1-006 ETEPLIRSEN FOR DUCHENNE MUSCULAR DYSTROPHY (DMD): CLINICAL AND BIOCHEMICAL RESULTS WITH LONGITUDINAL COMPARISON TO EXTERNAL CONTROLS ON SIX-MINUTE WALK TEST (6MWT)
J Mendell¹, Nathalie Goemans², Louise Rodino-Klapac¹, Z Sahenk¹, Linda Lowes¹, Lindsay Alfano¹, Katherine Berry¹, E Peterson¹, S Lewis¹, K Shontz¹, P Duda³, C Donoghue⁴, J Dworzak³, B Wentworth⁴, E Kaye⁴, Eugenio Mercuri⁵, DMD Italian Network⁶; ¹Columbus, OH, US, ²Leuven, BE, ³Cambridge, US, ⁴Cambridge, MA, US, ⁵Rome, IT, ⁶Milano, IT

PS2Group1-063 ENZYME REPLACEMENT THERAPY IS BENEFICIAL AFTER 5 YEARS OF TREATMENT IN A LARGE GROUP OF ADULT POMPE PATIENTS
Esther Kuperus, Michelle Kruijshaar, Stephan Wens, Juna de Vries, Marein Favejee, Chris van der Meijden, Dimitris Rizopoulos, Esther Brusse, Pieter van Doorn, Ans van der Ploeg, Nadine van der Beek; Rotterdam, NL

SCREEN 3

PS2Group1-059 FAT OXIDATION IS LIMITED IN MADD DURING EXERCISE, BUT GLUCOSE INFUSION IMPROVES EXERCISE CAPACITY
Karen Madsen¹, Nicolai Preisler¹, Astrid Emilie Buch¹, Mads Stemmerik¹, Pascal Laforêt², John Vissing¹; ¹Copenhagen, DK, ²Paris, FR

PS2Group3-004 SFEMG FINDINGS IN OCULAR COMPLICATIONS OF COSMETIC BOTOX INJECTIONS
Daniela Navarrete¹, Carlos Navarrete¹, Raul Muñoz¹, Vera Bril², Mireya Balart¹; ¹Santiago, CL, ²Toronto, ON, CA

SCREEN 4

PS2Group1-068 THE ANTI-CONVULSANTS LACOSAMIDE, LAMOTRIGINE AND RUFINAMIDE REDUCE MYOTONIA IN ISOLATED HUMAN AND RAT SKELETAL MUSCLE
Thomas Pedersen¹, Martin Skov², Ole Nielsen²; ¹Aarhus C, DK, ²Aarhus, DK

PS2Group1-064 SUGAR INFUSION IMPROVES EXERCISE CAPACITY IN PATIENTS WITH GLYCOGENIN-1 DEFICIENCY
Mads Stemmerik¹, Pascal Laforêt², Astrid Emilie Buch¹, Karen Madsen¹, John Vissing¹; ¹Copenhagen, DK, ²Paris, FR

SCREEN 5

PS2Group1-056 TREATMENT RELATED EFFECTS OF ANTI-GAA ANTIBODIES IN LATE ONSET POMPE DISEASE
Marie Wencil, Claudia Shambaugh, Namita Goyal, Virginia Kimonis, Tahseen Mozaffar; Orange, CA, US

PS2Group1-061 FATTY ACID OXIDATION DEFECTS PRESENTING AS PRIMARY MYOPATHY AND PROMINENT DROPPED HEAD SYNDROME
Seena Vengalil, Veeramani Preethish-Kumar, Kiran Polavarapu, Atchayaram Nalini, Narayanappa Gayathri, Rita Christopher, Manjunath Mahadevappa, Chandrajit Prasad; Bangalore, IN

POSTER SESSION 1 WEDNESDAY, JULY 6 &
THURSDAY, JULY 7, 2016

10:30-12:00

ROOM ▶ Exhibit Hall

WEDNESDAY AND THURSDAY SESSIONS SUPPORTED BY



PS1 Group 2

PS1Group2-001 STATIN-INDUCED NECROTIZING AUTOIMMUNE MYOPATHY. RECURRENCE WITH FIBRATE USE

Mario Fuentealba¹, Jorge Bevilacqua²;
¹Concepcion, CL, ²Santiago, CL

PS1Group2-002 STUDY OF HYALURONIDASE-FACILITATED SCIG IN CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP)

Claudia Sommer¹, John England²,
Johannes Jakobsen³, Russell Reeve⁴,
David Gelmont⁵; ¹Wurzburg, DE, ²US,
³Copenhagen, DK, ⁴Durham, NC, US,
⁵Westlake Village, CA, US

PS1Group2-003 MECHANISM OF HYALURONIDASE-FACILITATED SCIG ALLOWING INVESTIGATIONS IN NEUROMUSCULAR DISEASE

Christopher Rabbat¹, Tobin Chettiath²,
Martin Noel³, Robert Peterman⁴, Todd
Berner⁵; ¹Kansas, AL, US, ²Westlake
Village, CA, US, ³Mississauga, ON, CA,
⁴Vienna, AT, ⁵Bannockburn, US

PS1Group2-004 RATIONALE FOR TOLL-LIKE RECEPTOR ANTAGONISM AS A POTENTIAL NOVEL THERAPEUTIC APPROACH FOR DERMATOMYOSITIS

Kirsten Gruis¹, Kanneboyina Nagaraju²,
Tahseen Mozaffar³, Anthony A. Amato⁴,
Julie Brevard¹, Lindsey Granlund¹,
Joanna Horobin¹; ¹Cambridge, MA, US,
²Washington, DC, US, ³Orange, CA, US,
⁴Boston, US

PS1Group2-005 INFLAMMATORY MYOPATHIES: NEEDLE ELECTROMYOGRAPHY CHARACTERISTICS IN A SERIE OF CASES

Rosana Scola, Paulo Lorenzoni, Claudia
Kay, Renata Ducci, Paula Rodrigues, Lineu
Werneck; Curitiba, BR

PS1Group2-006 NECROTIZING MYOPATHY ASSOCIATED TO HIV: CASE REPORT

Renata Ducci, Francisco Magalhães, Daniel
Collares, Monica Gomes-da-Silva, Paulo
Lorenzoni, Claudia Kay, Mauricio Carvalho,
Lineu Werneck, Rosana Scola; Curitiba, BR

PS1Group2-007 SRP ANTIBODY ASSOCIATED NECROTIZING MYOPATHY MIMICKED LGMD: A CASE REPORT

Pariwat Thaisethawatkul¹, Rodney
McComb²; ¹Omaha, US, ²Omaha, NE, US

PS1Group2-008 MYASTHENIA GRAVIS AND POLYMYOSITIS PRESENTED SIMULTANEOUSLY

Florentina Berianu, 22, FL, US

PS1Group2-009 FOLLISTATIN GENE THERAPY IMPROVES SIX MINUTE WALK DISTANCE IN SPORADIC INCLUSION BODY MYOSITIS (SIBM)

Jerry Mendell¹, Z Sahenk¹, Mark Hogan¹,
Samiah Al-Zaidy¹, Kevin Flanigan²,
Louise Rodino-Klapac¹, Markus McColly³,
Kathleen Church¹, S Lewis¹, Linda Lowes¹,
Lindsay Alfano¹, Katherine Berry¹, Natalie
Miller¹, Igor Dvorchik¹, Melissa Moore-
Clingenpeel¹, Brian Kaspar¹; ¹Columbus,
OH, US, ²US, ³Columbus, US

PS1Group2-010 WHOLE-BODY MRI IN AMYOPLASIA CONGENITA

Cam-Tu Emilie Nguyen¹, Sharan Goobie¹,
Craig Campbell²; ¹London, ON, CA, ²London,
ON, CA

PS1 Group 4

PS1Group4-001 RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY TO INVESTIGATE THE EFFICACY, SAFETY AND TOLERABILITY OF TWO DIFFERENT DOSES OF IGPRO20 (SUBCUTANEOUS IMMUNOGLOBULIN) FOR THE TREATMENT OF CIDP-IGG DEPENDENCY AND RESTABILIZATION PHASE

Ivo van Schaik¹, Vera Bril², Nan van
Geloven³, Hans-Peter Hartung⁴, Richard
Lewis⁵, G Sobue⁶, Billie Durn⁷, John-Philip
Lawo⁴, Orell Mielke⁷, David Cornblath⁸,
Ingemar Merkies⁹, On on behalf of the
PATH study group¹; ¹Amsterdam, NL,
²Toronto, ON, CA, ³Leiden, NL, ⁴Dusseldorf,
DE, ⁵Los Angeles, CA, US, ⁶Nagoya,
JP, ⁷Marburg, DE, ⁸Baltimore, MD, US,
⁹Maastricht, NL

PS1Group4-002 SWITCHING PATTERNS IN PATIENTS WITH ICD-9 DIAGNOSED CIDP INITIATING IVIG TREATMENT

Jeffrey Guptil¹, Jeffrey Allen², Micheal
Runken³, Josh Noone⁴, Emily Zacherle⁵,
Chris Blanchette⁵; ¹Durham, NC, US,
²Minneapolis, MN, US, ³Raleigh, NC, US,
⁴Charlotte, NC, US, ⁵Davidson, NC, US

- PS1Group4-003 SYSTEMIC LUPUS ERYTHEMATOSUS PRESENTING WITH AUTONOMIC AND SOMATIC SMALL FIBER NEUROPATHY**
Oscar Trujillo, Juan Idiaquez, Ricardo Fadic; Santiago, CL
- PS1Group4-004 INCIDENCE OF GUILLAIN-BARRE SYNDROME IN IRANIAN CHILDREN UNDER FIFTEEN YEARS OLD; NATIONAL AFP SURVEILLANCE REPORT (2008-2014)**
Seyed Hassan Tonekaboni, Habibeh Nejad Biglari; Tehran, IR
- PS1Group4-005 POLYNEUROPATHY IN THE LIMELIGHT: A CASE**
Sandya Tirupathi¹, Matthew Sayers², John McConville¹, K Pang¹, Marie-Louise Kane¹; ¹Btba, UK, ²Bt126ba, UK
- PS1Group4-006 VASCULITIC NEUROPATHY COMPLICATED BY ANTERIOR SPINAL ARTERY SYNDROME**
Michael Ackerl¹, Wolfgang Grisold²; ¹Vienna, AT, ²US
- PS1Group4-007 CLINICAL AND ELECTROPHYSIOLOGICAL CHARACTERISTICS OF CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY IN KOREA**
Seol-Hee Baek, Jun-Soon Kim, BongJe Kim, So Hyun Ahn, Kyomin Choi, Seok-Jin Choi, Jung-Joon Sung, Yoon-Ho Hong; Seoul, KR
- PS1Group4-008 NON-TRAUMATIC PLEXOPATHIES AND RADICULOPATHIES IN CHILDREN**
Cam-Tu Emilie Nguyen¹, Craig Campbell², Hugh McMillan³, Chantal Poulin⁴, Michel Vanasse⁴, Jiri Vajsar⁵; ¹London, ON, CA, ²London, ON, CA, ³Ottawa, ON, CA, ⁴Montreal, QC, CA, ⁵Toronto, ON, CA
- PS1Group4-009 PERIPHERAL T CELL LYMPHOMA PRESENTING AS MILLER FISHER'S SYNDROME**
So Hyun Ahn, Seol-Hee Baek, Jun-Soon Kim, Kyomin Choi, Seok-Jin Choi, Yoon-Ho Hong, Jung-Joon Sung; Seoul, KR
- PS1Group4-010 A COMPARATIVE, DOUBLE-BLIND, RANDOMIZED, MULTICENTRE CLINICAL TRIAL TO ACCESS THE EFFICACY AND SAFETY OF CLAIRYG VS TEGELINE IN MAINTENANCE TREATMENT OF CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP)**
Claude Desnuelle¹, Jean Pouget², Jean Christophe Antoine³, Arnaud Lacour⁴, Jerome De Seze⁵, Christophe Vial⁶, Anne-Laure Bedat Millet⁷, Julien Cassereau⁸, David Adams⁹, Guilhem Sole¹⁰, Yann Pereon¹¹, Philippe Corcia¹², Thibault Moreau¹³, Steve Genestet¹⁴, Rabye Ouaja¹⁵, Anne Hufschmitt¹⁵, Chrystelle Mercier¹⁵; ¹Nice, FR, ²Marseille, FR, ³St Etienne, FR, ⁴Lille, FR, ⁵Strasbourg, FR, ⁶Lyon, FR, ⁷Rouen, FR, ⁸Angers, FR, ⁹Le Kremlin Bicetre, FR, ¹⁰Bordeaux, FR, ¹¹Nantes, FR, ¹²Tours, FR, ¹³Dijon, FR, ¹⁴Brest, FR, ¹⁵Les Ulis, FR
- PS1Group4-011 AN INTERNATIONAL, MULTICENTRE, EFFICACY AND SAFETY STUDY OF I10E, IQYMUNE IN INITIAL AND MAINTENANCE TREATMENT OF PATIENTS WITH CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP)**
Eduardo Nobile-Orazio¹, Richard Hughes², Isabel Illa³, Jean Marc Leger⁴, S J Ingemar Merckies⁵, Luca Padua⁶, Rabye Ouaja⁷, Witold Malyszczak⁷, Sophie Puget⁸; ¹Milan, IT, ²London, UK, ³Barcelona, ES, ⁴Paris, FR, ⁵Hoofddorg, NL, ⁶Roma, IT, ⁷Les Ulis, FR, ⁸Les Luis, FR
- PS1Group4-012 NEUROMUSCULAR COMPLICATIONS ARE NOT RARE IN MIDDLE EAST RESPIRATORY SYNDROME**
Jee-Eun Kim, Su-yeon Park, Jae-Hyeok Heo, Hye-ok Kim, Sook-hee Song, Sang-Soon Park, Tai-Hwan Park, Jin-Young Ahn, Min-Ky Kim, Jae-Phil Choi; Seoul, KR
- PS1Group4-013 NOVEL ANTIGEN-SPECIFIC TREATMENT FOR ANTI-MYELIN-ASSOCIATED GLYCOPROTEIN NEUROPATHY**
Ruben Herrendorff, Pascal Haenggi, H el ene Pfister, Andreas Steck, Beat Ernst; Basel, CH
- PS1Group4-014 A EUROPEAN, RANDOMISED, DOUBLE-BLIND, CROSS-OVER STUDY OF A NEW 10% HUMAN INTRAVENOUS IMMUNOGLOBULIN VERSUS OTHER IVIG IN PATIENTS WITH MULTIFOCAL MOTOR NEUROPATHY-LIME STUDY**
Jean Marc Leger¹, Richard Hugues², S J Ingemar Merckies³, Eduardo Nobile-Orazio⁴, Rabye Ouaja⁵, Witold Malyszczak⁵, Sophie Puget⁶; ¹Paris, FR, ²London, UK, ³Hoofddorg, NL, ⁴Milan, IT, ⁵Les Ulis, FR, ⁶Les Luis, FR
- PS1Group4-015 CORRELATION BETWEEN IGM PARAPROTEINEMIA AND MORPHOMETRIC PARAMETERS OF SURAL NERVE IN ANTI-MAG, SGGL NEUROPATHY**
Kon Ping Lin, Hua Chuan Chao, Cheng Ta Chou, Yi-Chung Lee; Taipei, TW
- PS1Group4-016 INFLAMMATORY DIABETIC NEUROPATHY: HELPFUL DIAGNOSTIC PARAMETERS**
Pariwat Thaisethhawatkul¹, J Americo Fernandes, Jr², Ezequiel Piccione², Laetitia Truong², P James Dyck³; ¹Omaha, US, ²Omaha, NE, US, ³Rochester, MN, US

- PS1Group4-017 EGR2 MUTATION ENHANCE PHENOTYPE SPECTRUM OF DEJERINE-SOTTAS SYNDROME**
Elena Gargaun¹, Andreea Seferian¹, Ruxanda Cardas¹, Anne Gaelle Lemoing¹, Juliette Nectoux¹, Catherine Delanoe¹, Gisèle Bonne¹, Anne Boland¹, Jean-Francois Deleuze², Cécile Masson¹, Laurent Servais¹, Teresa Gidaro¹; ¹Paris, FR, ²Evry, FR
- PS1Group4-018 DEMYELINATING FEATURES IN NEUROPHYSIOLOGICAL STUDY OF TRANSTHYRETIN FAMILIAL AMYLOID POLYNEUROPATHY DUE TO VAL30MET MUTATION IN A PORTUGUESE POPULATION**
Marcio Cardoso, Ana Sousa, Katia Valdez, Teresa Coelho; Porto, PT
- PS1Group4-019 TRANSTHYRETIN-RELATED HEREDITARY AMYLOIDOSIS IN AN ARGENTINE FAMILY WITH TTR TYR114CYS MUTATION**
Marcelo Rugiero, Marcelo Chaves, Mariela Bettini, Maria Ines Araoz, Maria Saez, Patricia Sorroche, Edgardo Cristiano; Buenos Aires, AR
- PS1Group4-020 NOVEL INF2 GENE MUTATIONS IN CZECH PATIENTS WITH SPORADIC HMSN**
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- PS1Group4-021 PREDICTION OF NERVE CONDUCTION STUDIES OUTCOMES IN PATIENTS WITH FAMILIAL AMYLOIDOTIC POLYNEUROPATHY RECEIVING TAFAMIDIS THERAPY**
Sandra Sousa¹, Katia Valdez², Isabel Fonseca², Teresa Coelho²; ¹Cascais, PT, ²Porto, PT
- PS1Group4-022 HEREDITARY MOTOR AND SENSORY NEUROPATHY WITH PYRAMIDAL SIGNS CAUSED BY NEFL GENE MUTATION**
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- PS1Group4-023 ATYPICAL CIDP OR CMT IN THE ELDERLY? A CASE REPORT**
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- PS1Group4-024 ANTI-GRAVITY AEROBIC TRAINING IN PATIENTS WITH CHARCOT-MARIE-TOOTH DISEASE TYPES 1A AND 1X**
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- PS1Group4-025 COEXISTENCE OF CHARCOT MARIE TOOTH DISEASE TYPE 1A AND DIABETES: A CLINICOPATHOLOGICAL STUDY**
Kon Ping Lin, Hua Chuan Chao; Taipei, TW
- PS1Group4-026 IN VIVO FUNCTIONAL ANALYSIS OF THE NOVEL BSCL2 P.R96H MUTATION RESULTING IN HEREDITARY MOTOR NEUROPATHY**
Cheng-Tsung Hsiao, Pei-Chien Tsai, Yi-Chu Liao, Kon Ping Lin, Yi-Chung Lee; Taipei, TW
- PS1Group4-027 TWO NOVEL DE NOVO GARS MUTATIONS CAUSE EARLY-ONSET AXONAL CHARCOT-MARIE-TOOTH DISEASE**
Yi-Chu Liao, Yo-Tsen Liu, Pei-Chien Tsai, Bing-Wen Soong, Yi-Chung Lee; Taipei, TW
- PS1Group4-028 BIOPHYSICAL CHARACTERISTICS AND CLINICAL CORRELATION OF GJB1 MUTATIONS IN CHARCOT-MARIE-TOOTH DISEASE TYPE X1**
Pei-Chien Tsai, Yi-Chu Liao, Kon Ping Lin, Yo-Tsen Liu, Yi-Chung Lee; Taipei, TW
- PS1Group4-029 A NICOTINAMIDE ADENINE NUCLEOTIDE (NAD+) PRECURSOR IS A POTENTIAL THERAPY FOR DIABETIC NEUROPATHY**
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- PS1Group4-030 FREQUENT LABORATORY TESTS ABNORMALITIES IN PERIPHERAL NEUROPATHY**
Alon Abraham, Majed Majed Alabdali, Abdulla Alsulaiman, Hana Albulaihe, Ari Breiner, Carolon Barnett, Hans Katzberg, Danah Aljaafari, Leif Lovblom, Bruce Perkins, Vera Bril; Toronto, ON, CA
- PS1Group4-031 CLINICAL AND LABORATORY FEATURES OF SMALL FIBER NEUROPATHIES (SFN) WITH IGM VS TS-HDS**
Jafar Kafaie, Minsoo Kim; Saint Louis, MO, US
- PS1Group4-032 AN INTERESTING CASE OF SCIATIC NEUROPATHY**
Jason Lazarou; Toronto, ON, CA
- PS1Group4-033 SUBACUTE BRACHIAL PLEXOPATHY ASSOCIATED WITH CYSTIC SUBCORACOID BURSTITIS**
Suk-Won Ahn, Dae-Woong Kang, Myung-Jin Kim, Jung-Joon Sung, Yoon-Ho Hong, Chang-Seop Kim; Seoul, KR
- PS1Group4-034 A CASE OF NEUROMYOTONIA ASSOCIATED WITH A CHRONIC POLYRADICULONEUROPATHY**
Anna Paula Covaleski¹, Vanessa Mota¹, Otávio Lins¹, Wilson Marques²; ¹Recife, BR, ²Ribeirão Preto, BR

- PS1Group4-035 QUALITY OF LIFE IN PATIENTS WITH DIABETIC PERIPHERAL NEUROPATHY: A LITERATURE REVIEW**
Semra Aciksoz, Ankara, TR
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Byung-Nam Yoon¹, Jung-Joon Sung², Suk-Won Ahn², Ji-Eun Kim², Yoon-Ho Hong²;
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- PS1Group4-037 THE USE OF OMEGA-3 SUPPLEMENTATION FOR MANAGING DIABETIC NEUROPATHY: RESULTS FROM A CLINICAL PILOT TRIAL**
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- PS1Group4-038 B12 DEFICIENCY IS A CAUSE OF REVERSIBLE AUTONOMIC FAILURE: A CASE REPORT**
Pariwat Thaisethhawatkul, Omaha, US
- PS1Group6-007 POTENTIALLY CONFOUNDING VARIABLES OF GDF-15: NEW BIOMARKER OF MITOCHONDRIA DISEASES**
Akiko Ishii¹, Seitaro Nohara¹, Fumiko Yamamoto¹, Shuichi Yatsuga², Makoto Terada¹, Tetushi Aizawa¹, Tetsuto Yamaguchi¹, Kumi Yanagihara¹, Tetsuya Moriyama¹, Naoki Touzaka¹, Zenshi Miyake¹, Hiroshi Tsuji¹, Yasushi Tomidokoro¹, Kiyotaka Nakamagoe¹, Kazuhiro ISHII¹, Masahiko Watanabe¹, Yasutoshi Koga², Akira Tamaoka¹; ¹Tsukuba, JP, ²Kurume, JP
- PS1Group6-008 A NOVEL ASSESSMENT OF BAROREFLEX ACTIVITY BY PHOTOPLETHYSMOGRAPHY AND TERNARY ARITHMETIC CODING IN A RAT MODEL**
An-Bang Liu¹, Hsien-Tsai Wu², Chun-Keng Lin²; ¹Hualien, TW, ²Shoufeng, TW
- PS1Group6-009 AGREEMENT BETWEEN AUTOMATED AND MANUAL QUANTIFICATION OF CORNEAL NERVE FIBER LENGTH: IMPLICATIONS FOR DIABETIC NEUROPATHY RESEARCH**
Daniel Scarr, Cesar Falappa, Ilia Ostrovski, Leif Lovblom, Mohammed Farooqi, Dylan Kelly, Tong Wu, Elise Halpern, Mylan Ngo, Eduardo Ng, Andrej Orszag, Vera Bril, Bruce Perkins; Toronto, ON, CA

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- PS1Group6-002 UNUSUAL CAUSE OF NOCTURNAL HAND PAIN-3 CASES DIAGNOSED BY POCUS AFTER NORMAL EMG**
Abraham Chaiton, Toronto, ON, CA
- PS1Group6-003 IMPACT OF DRISAPERSEN ON APPARENT FAT FRACTION IN DUCHENNE MUSCULAR DYSTROPHY**
Courtney Bishop¹, Rexford Newbould¹, Zhengning Lin², Robert Janiczek³, Susanne Wang²; ¹London, ON, CA, US, ²Novato, CA, US, ³Middlesex, UK
- PS1Group6-004 USEFULNESS OF MRI IN CASES OF HYPERCKEMIA**
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- PS1Group6-005 LOWER LIMB MUSCLE VOLUME TEST, RE-TEST VARIABILITY USING MRI**
Hui Jing Yu¹, Thomas Fuerst², Randall Stoltz³, Juan Chavez⁴; ¹Princeton, NJ, US, ²Newark, CA, US, ³Indianapolis, IN, US, ⁴Cambridge, MA, US
- PS1Group6-006 VITAMIN DEFICIENCIES IN PATIENTS WITH VARIOUS MYOPATHIES AND OTHER NEUROMUSCULAR CONDITIONS-PILOT STUDY**
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- PS1Group6-010 USE OF CORNEAL NERVE FIBRE LENGTH (CNFL) FOR DIABETIC NEUROPATHY IDENTIFICATION IN OLDER PATIENTS WITH LONGSTANDING TYPE 1 DIABETES**
Mohammed Farooqi¹, Leif Lovblom¹, Daniel Scarr¹, Julie Lovshin¹, Yuliya Lytvyn¹, Genevieve Boulet¹, Alanna Weisman¹, Hillary Keenan², Michael Brent¹, Narinder Paul¹, Ilia Ostrovski¹, Vera Bril¹, David Cherney¹, Bruce Perkins¹; ¹Toronto, ON, CA, ²Boston, US
- PS1Group6-011 VALIDITY OF AN AUTOMATED PROTOCOL OF IN VIVO CORNEAL CONFOCAL MICROSCOPY FOR DIABETIC SENSORIMOTOR POLYNEUROPATHY DETECTION IN TYPE 1 DIABETES**
Daniel Scarr, Nancy Cardinez, Ilia Ostrovski, Tong Wu, Mohammed Farooqi, Leif Lovblom, Elise Halpern, Ausma Ahmed, Mylan Ngo, Eduardo Ng, Andrej Orszag, Vera Bril, Bruce Perkins; Toronto, ON, CA
- PS1Group6-012 VALIDATION OF COOLING DETECTION THRESHOLD AS A MARKER OF SENSORIMOTOR POLYNEUROPATHY IN TYPE 2 DIABETES**
Mohammed Farooqi, Andrej Orszag, Zoe Lysy, Leif Lovblom, Elise Halpern, Mylan Ngo, Eduardo Ng, Ari Breiner, Vera Bril, Bruce Perkins; Toronto, ON, CA

- PS1Group6-013 HEREDITARY NEUROPATHIES: THE ROLE OF COPY NUMBER VARIATIONS (CNVS) IN THE NGS TARGETED GENE PANEL DIAGNOSTIC TESTING**
Petra Lassuthova, Jana Neupauerová, Simona Marková, Marcela Krůtová, Radim Mazanec, Dana Brožková, Pavel Seeman; Prague, CZ
- PS1Group6-014 HOW MULTI-GENE PANELS CAN CHANGE THE LANDSCAPE OF DIAGNOSING NEUROMUSCULAR DISORDERS**
Margaret Bradbury¹, Amanda Lindy², Amy Decker², Deborah Copenheaver², Sharon Suchy²; ¹Olney, MD, US, ²Gaithersburg, MD, US
- PS1Group6-015 SENSITIVITY AND SPECIFICITY OF DR1 BISULFITE SEQUENCING IN DETECTING SMCHD1 MUTATION IN A COHORT OF FSHD1 AND FSHD-LIKE PATIENTS**
Audrey Briand¹, Christian Baudoin², Nadira Lagha², Pilvi Nigumann², Françoise Chapon³, Tania Stojkovic¹, Christophe Vial⁴, Françoise Bouhour⁴, Elena Pegoraro⁵, Philippe Petiot⁴, Antony Behin¹, Bruno Eymard¹, Pascal Laforêt¹, Leonardo Salviati⁵, Marc Jeanpierre¹, Michel Vidaud¹, Claude Desnuelle², Gael Cristofari², Sabrina Sacconi²; ¹Paris, FR, ²Nice, FR, ³Caen, FR, ⁴Lyon, FR, ⁵Padova, IT
- PS1Group6-017 ROBUST GENOTYPING IN THE DIAGNOSTICS OF LIMB GIRDLE MUSCULAR DYSTROPHIES**
Baiba Lace¹, Jurgis Strautmanis², Ieva Micule², Maruta Naudina², Loreta Cimbalištie³, Algirdas Utkus³, Birute Burnyte³, Janis Stavusis², Inna Inashkina²; ¹Quebec, QC, CA, ²Riga, LV, ³Vilnius, LT
- PS1Group6-018 PROFLECT: A USER-FRIENDLY TOOL TO DETECT COPY NUMBER VARIATION (CNV) AMONG AMPLICON SEQUENCING DATA**
Paco Derouault, Claire-Cécile Barrot, Rémi Moulinas, Franck Sturtz, Stéphane Merillou, Anne-Sophie LIA; Limoges, FR
- PS1Group6-019 GENETIC SEQUENCING OF PATIENTS WITH LIMB GIRDLE MUSCLE WEAKNESS USING AN NGS PANEL**
Elaine Lee¹, Madhuri Hegde², Hillarie Windish¹, Babi Nallamilli², Laura Rufibach¹; ¹Seattle, WA, US, ²Decatur, US
- PS1 Group 8**
- PS1Group8-001 A PHASE III DOUBLE-BLIND, RANDOMIZED, PLACEBO-CONTROLLED STUDY (SIDEROS) ASSESSING THE EFFICACY OF IDEBENONE IN SLOWING THE RATE OF RESPIRATORY FUNCTION LOSS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY RECEIVING GLUCOCORTICOID STEROIDS**
Gunnar Buyse¹, Oscar Mayer², R Donisa-Dregheci³, F Couttet⁴, Jodi Wolff⁴, Nick Coppard⁴; ¹Leuven, BE, ²Philadelphia, PA, US, ³Liestal, CH, ⁴Liestal, SE
- PS1Group8-003 DEVELOPMENT OF A NOVEL TOOL FOR ASSESSMENT OF CRAMP SEVERITY: THE TORONTO CLINICAL CRAMP INDEX (TCCI)**
Hans Katzberg, Vera Bril, Carolina Barnett-Tapia; Toronto, ON, CA
- PS1Group8-004 RELIABILITY AND VALIDITY OF THE 100 METER TIMED TEST AS AN OUTCOME MEASURE IN DUCHENNE MUSCULAR DYSTROPHY**
Lindsay Alfano¹, Natalie Miller¹, Katherine Berry¹, Kevin Flanigan², Linda Cripe¹, Jerry Mendell¹, Linda Lowes¹; ¹Columbus, OH, US, ²US
- PS1Group8-005 INTRAVENOUS IMMUNOGLOBULIN "WEAR-OFF EFFECT" IN CIDP: STUDY DESIGN AND PROGRESS UPDATE**
Jeffrey Allen¹, Jeffrey Allen², Mamatha Pasnoor³, Ted Burns⁴, Senda Ajroud-Driss², John Ney⁵, Albert Cook⁶, Thomas Brannagan, III⁷, John Kissel⁸, Kenneth Gorson⁹, Richard Lewis¹⁰, Melvin Berger¹¹, Patty Riley¹¹, David Schaefer¹², Timothy Walton¹²; ¹Minneapolis, MN, US, ²Chicago, IL, US, ³Kansas City, KS, US, ⁴US, ⁵Seattle, WA, US, ⁶Johns Creek, GA, US, ⁷New York, NY, US, ⁸Columbus, OH, US, ⁹Boston, MA, US, ¹⁰Los Angeles, CA, US, ¹¹King Of Prussia, PA, US, ¹²Lenexa, KS, US
- PS1Group8-006 IDEBENONE REDUCES RESPIRATORY COMPLICATIONS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
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- PS1Group8-007 TREATMENT EFFECT OF IDEBENONE ON INSPIRATORY FUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
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- PS1Group8-008 REFERENCE VALUES FOR THE THREE-MINUTE WALK TEST, NORTH STAR AMBULATORY ASSESSMENT AND TIMED TESTS IN TYPICALLY DEVELOPING BOYS AGED 2.5-5 YEARS**
Katrijn Klingels, Jasmine Hoskens, Lise Van Verdegem, Marleen Van den Hauwe, Gunnar Buyse, Nathalie Goemans; Leuven, BE
- PS1Group8-009 NEUROMUSCULAR JUNCTION IN EXPERIMENTAL AUTOIMMUNE ENCEPHALOMYELITIS: A HISTOPATHOLOGICAL ANALYSIS**
Thalita Rocha¹, Jetro Sguarezi¹, Sara Ferreira¹, Rodolfo Thomé², Liana Verinaud², Catarina Rapôso²; ¹Bragança Paulista, BR, ²Campinas, BR
- PS1Group8-010 WHEN SHOULD WE TREAT HYPERCKEMIA?**
Astrid Emilie Buch, Karen Pedersen, Sofie Ostergaard, Jesper Thomassen, Ruth Frikke-Schmidt, Nanna Witting, John Vissing; Copenhagen E, DK
- PS1Group8-011 VALIDATION OF PROTEIN BIOMARKERS FOR DUCHENNE MUSCULAR DYSTROPHY**
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- PS1Group8-012 SURVEY ON USAGE OF TELECOMMUNICATION TERMINALS IN JAPANESE PATIENTS WITH NEUROMUSCULAR DISEASES**
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- PS1Group8-013 IS HOME TREATMENT IN AUTO-IMMUNE DISEASE PATIENTS TREATED BY IVIG SAFE?**
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- PS1Group8-014 AT HOME VERSUS HOSPITAL IVIG FOR THE TREATMENT OF MULTIFOCAL MOTOR NEUROPATHY (MMN), CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP) AND LEWIS SUMNER SYNDROME (LSS): A COST OF ILLNESS STUDY**
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- PS1Group8-015 IMPACT OF DIABETIC NEUROPATHY ON DIABETES DISTRESS AND DEPRESSION IN LONGSTANDING T1DM: RESULTS FROM THE CANADIAN STUDY OF LONGEVITY IN TYPE 1 DIABETES**
Johnny-Wei Bai¹, Alanna Weisman¹, Mohammed Farooqi¹, Leif Lovblom¹, Elise Halpern¹, Genevieve Boulet¹, Devrim Eldelekli¹, Julie Lovshin¹, Yuliya Lytvyn¹, Hillary Keenan², Michael Brent¹, Narinder Paul¹, Vera Brill¹, David Cherney¹, Bruce Perkins¹; ¹Toronto, ON, CA, ²Boston, US
- PS1Group8-016 ORAL MOTOR COMMUNICATION INVENTORY FOR ALS: CONTENT VALIDATION**
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- PS1Group8-017 REHABILITATION NURSING IN NEUROMUSCULAR DISEASES**
Tulay Basak, Ankara, TR
- PS1Group8-018 PROBLEMS OF FAMILIES LIVING WITH BOYS WITH DUCHENNE MUSCULAR DYSTROPHY (DMD) IN A DEVELOPING COUNTRY**
Yakup Sayin, Coşkun Özdemir; İstanbul, TR
- PS1Group8-019 REAL WORLD USE OF PRIVIGEN IN THE TREATMENT OF GBS AND CIDP: RESULTS OF A RETROSPECTIVE OBSERVATIONAL STUDY**
Ayman Kafal, Montreal, QC, CA

- PS1Group8-020 REFRACTORY POSTURAL ORTHOSTATIC TACHYCARDIA SYNDROME: EFFICACY AND SAFETY OF WEEKLY ALBUMIN INFUSIONS**
Zaeem Siddiqi, Aimee Soloway, Derrick Blackmore; Edmonton, AB, CA
- PS1Group8-021 RD-CONNECT: DATA SHARING AND ANALYSIS FOR RARE DISEASE RESEARCH WITHIN THE INTEGRATED PLATFORM AND THROUGH GA4GH BEACON AND MATCHMAKER EXCHANGE**
Andreas Roos¹, Sergi Beltran², Davide Piscia², Steven Laurie², Joan Protasio², Anastasios Papakonstantinou², Andrés Cañada³, Jose Maria Fernández³, Mark Thompson⁴, Rajaram Kaliyaperumal⁴, Séverine Lair⁵, Pedro Sernadela⁶, Marta Girdea⁷, Michael Brudno⁷, André Blavier⁵, Rachel Thompson¹, Volker Straub¹, Matthew Bellgard⁸, Justin Paschall⁹, Marco Roos⁴, Peter A C 't Hoen⁴, Alfonso Valencia³, David Salgado¹⁰, Christophe Bérourd¹⁰, Ivo Glynne Gut², Hanns Lochmüller¹; ¹Newcastle, UK, ²Barcelona, ES, ³Madrid, ES, ⁴Leiden, NL, ⁵Rouen, FR, ⁶Aveiro, PT, ⁷Toronto, ON, CA, ⁸Perth, ACT, AU, ⁹Cambridge, UK, ¹⁰Marseille, FR
- PS1Group8-022 CASE REPORT OF RECURRENT MENINGITIS SECONDARY TO CSF RHINORRHEA**
Aleena Soomro, Karachi, PK
- PS1Group8-023 CLINICAL PRESENTATION OF ANTI-NMDA ENCEPHALITIS**
Rabail Karim, Bashir Soomro; Karachi, PK
- PS1Group8-024 NUDT15 VARIANT IS THE MOST COMMON VARIANT ASSOCIATED WITH THIOPURINE-INDUCED EARLY LEUKOPENIA AND ALOPECIA IN KOREAN PATIENTS WITH VARIOUS NEUROLOGICAL DISEASES**
Sun-Young Kim¹, Dae-Seong Kim², Jin-Hong Shin², Jin-Sung Park³, Sa-Yoon Kang⁴, Ki-Jong Park³, Tai-Seung Nam⁶, So-Young Huh⁷, Jong-kuk Kim⁷; ¹44033, KR, ²-, KR, ³Daegu, KR, ⁴Jeju, KR, ⁵Jinju, KR, ⁶Gwangju, KR, ⁷Pusan, KR
- PS1Group8-025 DATABASE OF NEUROMUSCULAR DISEASES IN REGION OF CONCEPCION IN CHILE**
Mario Fuentealba, Concepcion, CL
- PS1Group8-026 SEROPREVALENCE OF HUMAN T-LYMPHOTROPIC VIRUS TYPE 1 IN PATIENTS WITH SURGICAL HISTORY IN KAGOSHIMA, SOUTHERN JAPAN**
Yuichi Tashiro¹, Eiji Matsuura¹, Satoshi Nozuma¹, Akihiro Hashiguchi², Osamu Watanabe², Hiroshi Takashima¹; ¹Kagoshima City, JP, ²Kagoshima, JP
- PS1Group8-027 HIGH RISK BREAST CANCER SCREENING IN WOMEN WITH NEUROFIBROMATOSIS TYPE 1**
Jeanna McCuaig, Shelley Westergard, Catherine Maurice, Paul Kongkham, Galareh Zadeh, Carolina Barnett-Tapia, Vera Bril, Raymond Kim; Toronto, ON, CA
- PS1Group8-029 UNDERSTANDING THE CANADIAN NEUROMUSCULAR DISEASE RESEARCH LANDSCAPE**
Megan Johnston¹, Christopher MacDonald¹, Jeff Dilworth², Hans Katzberg³, Jean Mah¹, Lawrence Korngut¹; ¹Calgary, AB, CA, ²Ottawa, ON, CA, ³Toronto, ON, CA
- PS1Group8-030 CANADIAN NEUROMUSCULAR DISEASES NETWORK (CAN-NMD) THE DEVELOPMENT & IMPLEMENTATION OF A WEB-BASED KNOWLEDGE SHARING AND EXCHANGE PLATFORM**
Gracia Mabaya¹, Craig Campbell¹, Cynthia Gagnon², Megan Johnston³, Laura McAdam⁴, Jeremy Dixon³, Kelvin Jones⁵, Charles Kassardjian⁴, Aneal Khan³, Jane Mitchell⁴, Annie Plourde², Maryam Oskoui⁴, Lawrence Korngut³; ¹London, ON, CA, ²Jonquière, QC, CA, ³Calgary, AB, CA, ⁴Toronto, ON, CA, ⁵Edmonton, AB, CA, ⁶Montreal, QC, CA
- PS1Group8-031 UNDERSTANDING DECISION NEEDS FOR RESPIRATORY INTERVENTIONS IN PAEDIATRIC NEUROMUSCULAR DISORDERS FROM THE PERSPECTIVE OF HEALTHCARE PROVIDERS**
Gracia Mabaya¹, Sherri Katz², Margaret Lawson², April Price¹, Dhenuka Radhakrishnan², Jean Mah³, Lawrence Korngut³, Hugh McMillan², Cheryl Scholtes¹, Allyson Shephard², Melissa Heletea², Craig Campbell¹, Lynda Hoey²; ¹London, ON, CA, ²Ottawa, ON, CA, ³Calgary, AB, CA
- PS1Group8-032 A PROPOSAL: ISAACS SYNDROME (ACQUIRED NEUROMYOTONIA) DIAGNOSTIC CRITERIA**
Osamu Watanabe¹, Kimiyoshi Arimura¹, Hiroshi Takashima²; ¹Kagoshima, JP, ²Kagoshima City, JP
- PS1Group8-033 UNDERSTANDING THE PERSPECTIVES OF YOUNG ADULTS WITH DUCHENNE MUSCULAR DYSTROPHY AS THEY TRANSITION TO ADULTHOOD AND ADULT HEALTH CARE**
Sally Lindsay, Laura McAdam, Tania Mahenderin; Toronto, ON, CA

PS1Group8-034 CHARACTERISTICS DEVELOPMENT OF A NEW IVIG (I10) THE QUALITY BY DESIGN APPROACH (QBD)
Philippe Paolantonacci, Catherine Decoupade, Philippe Appourchaux, Catherine Michalski, Rabye Ouaja, Ousmane Alfa Cisse, Ludovic Burlot; Les Ulis, FR

PS1Group8-035 PURIFICATION OF IVIG (INTRAVENOUS IMMUNOGLOBULIN) FROM IGNG MANUFACTURING PROCESS TO OPTIMIZE PRODUCT TOLERABILITY PROFILE: EXAMPLE OF HUMAN NORMAL IMMUNOGLOBULIN (IQYMUNE® 100 MG, ML, SOLUTION FOR INFUSION)
Philippe Paolantonacci¹, Catherine Decoupade¹, Philippe Appourchaux¹, Catherine Michalski¹, Rabye Ouaja¹, Sophie Puget², Ludovic Burlot¹; ¹Les Ulis, FR, ²Les Luis, FR

PS1Group8-036 HEREDITARY MUSCLE DISORDERS IN MIDDLE EUROPE: DATA FROM HOSPITAL REGISTRY
Stan Vohanka, Josef Bednarik, Olesja Parmova, Magda Chmelikova, Lenka Fajkusova; Brno, CZ

PS1Group8-037 ANTINOCICEPTIVE AND ANTI-INFLAMMATORY EFFECTS OF COMBINED ADMINISTRATION OF VITAMIN B12 AND KETOROLAC IN RATS
MD Mizanur Rahman, Dhaka, BD

PS1Group8-038 MEASURING PRIORITIES AND GOALS OF CHILDREN WITH DUCHENNE MUSCULAR DYSTROPHY TO DEVELOP A MEANINGFUL PATIENT REPORTED OUTCOME MEASURE
Roni Propp¹, Sarah Buttle², Shannon Weir¹, Clarissa Encisa¹, Aileen Davis¹, Laura McAdam¹, Nancy Salbach¹, Unni Narayanan¹; ¹Toronto, ON, CA, ²Ottawa, ON, CA

POSTER SESSION 2

FRIDAY, JULY 8 &
SATURDAY, JULY 9, 2016

10:30-12:00

ROOM► Exhibit Hall

FRIDAY SESSION SUPPORTED BY



SATURDAY SESSION SUPPORTED BY



PS2 Group 1

PS2Group1-001 DYSFELINOPATHIES IN BURKINA FASO: A CASE REPORT
Anselme Dabilgou, Christian Napon, Julie M A Kyelem, Alassane Drave, Anila Bhunnoo, Jean Kabore; Ouagadougou, BF

PS2Group1-002 THE QUALITY OF LIFE IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY, IRANIAN EXPERIENCE
Gholamreza Zamani, Morteza Heidari, Mahshid Mehdizadeh; Tehran, IR

PS2Group1-003 CURRENT STATUS OF DYSTROPHINOPATHY NATIONAL REGISTRY IN JAPAN
En Kimura¹, Madoka Mori-Yoshimura², Satomi Mitsuhashi², Fumi Takeuchi³, Harumasa Nakamura³, Hirohumi Komaki², Ichizo Nishino², Mitsuru Kawai⁴, Shin'ichi Takeda²; ¹Kodaira, JP, ²Tokyo, JP, ³Kodaira, Tokyo, JP, ⁴Hasuda, Saitama, JP

PS2Group1-004 CHARACTERISTICS OF JAPANESE PATIENTS WITH BECKER MUSCULAR DYSTROPHY IN A JAPANESE NATIONAL REGISTRY OF MUSCULAR DYSTROPHY (REMUDY): HETEROGENEITY AND CLINICAL VARIATION
Madoka Mori-Yoshimura¹, Satomi Mitsuhashi¹, Hirohumi Komaki¹, Naohiro Yonemoto², Harumasa Nakamura³, Fumi Takeuchi³, Yukiko Hayashi¹, Miho Murata¹, Ichizo Nishino¹, Shin'ichi Takeda¹, En Kimura²; ¹Tokyo, JP, ²Kodaira, JP, ³Kodaira, Tokyo, JP

PS2Group1-005 META-ANALYSES OF ATALUREN IN PATIENTS WITH NONSENSE MUTATION DUCHENNE MUSCULAR DYSTROPHY
Craig Campbell¹, Francesco Muntoni², Eugenio Mercuri³, Xiaohui Luo⁴, Gary Elfring⁴, Hans Kroger⁵, Peter Riebling⁵, Tuyen Ong⁵, Robert Spiegel⁵, Stuart W Peltz⁵, Craig McDonald⁶; ¹London, ON, CA,

- ²London, UK, ³Rome, IT, ⁴South Plainfield, AL, US, ⁵South Plainfield, NJ, US, ⁶Sacramento, CA, US
- PS2Group1-007 LONGITUDINAL EFFECT OF DRISAPERSEN VERSUS HISTORICAL CONTROLS ON AMBULATION IN DUCHENNE MUSCULAR DYSTROPHY**
Nathalie Goemans¹, Már Tulinius², Anna-Karin Kroksmark², Marleen Van den Hauwe¹, Zhengning Lin³, Susanne Wang³, Giles Champion⁴; ¹Leuven, BE, ²Gothenburg, SE, ³Novato, CA, US, ⁴Leiden, NL
- PS2Group1-008 IMPACT OF MUSCLE FUNCTION, NUTRITIONAL STATE AND SYSTEMIC INFLAMMATION, ON BONE MINERAL DENSITY IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
Oriana Cruz¹, Maricela Rodriguez-Cruz², Carlos Wong-Baeza³, Salvador Atilano-Miguel², Tomas Almeida-Becerril²; ¹Mexico,MX, ²D.f,MX, ³Mexico, D.f,MX
- PS2Group1-009 MIBG THERAPY FOR AN INOPERABLE PARAGANGLIOMA IN DUCHENNE MUSCULAR DYSTROPHY**
Denis Duboc, Marine Paul, Laurie Fanon, Karim Wahbi, Marco Alifano, Florence Tenenbaum, Laurence Guignat; Paris, FR
- PS2Group1-010 EFFECT OF METFORMIN ON IN VIVO AND EX VIVO PATHOLOGY SIGNS IN EXERCISED DYSTROPHIC MDX MICE**
Roberta Capogrosso, Anna Cozzoli, Arcangela Giustino, Paola Mantuano, Francesca Sanarica, Michela De Bellis, Annamaria De Luca; Bari, IT
- PS2Group1-011 DEVELOPMENT OF A PATIENT-REPORTED OUTCOME MEASURE FOR UPPER LIMB FUNCTION IN DUCHENNE MUSCULAR DYSTROPHY (DMD-UPPER LIMB PROM)**
Katrijn Klingels¹, Anna Mayhew², Elena Mazzone³, Michelle Eagle², Tina Duong⁴, Valérie Decostre⁵, Marion Main⁶, Marleen Van den Hauwe¹, Ulla Werlauff⁷, Imelda De Groot⁸, Sonia Messina⁹, Valeria Ricotti⁶, Giles Champion¹⁰, Laurent Servais⁵, Elizabeth Vroom¹¹, Eugenio Mercuri³, Nathalie Goemans¹; ¹Leuven, BE, ²Newcastle Upon Tyne, UK, ³Rome, IT, ⁴Stanford, US, ⁵Paris, FR, ⁶London, UK, ⁷Aarhus, DK, ⁸Nijmegen, NL, ⁹Messina, IT, ¹⁰Leiden, NL, ¹¹Veenendaal, NL
- PS2Group1-012 GENOTYPE PHENOTYPE ANALYSIS OF MULTIPLEX LIGATION DEPENDENT PROBE AMPLIFICATION (MLPA) POSITIVE DUCHENNE, BECKER MUSCULAR DYSTROPHY (DMD, BMD) PATIENTS**
Seena Vengalil, Kiran Polavarapu, Veeramani Preethish-Kumar, Atchayaram Nalini, Meera Purushottam, Deepha Sekar; Bangalore, IN
- PS2Group1-013 CLINICAL AND MUSCLE BIOPSY CHARACTERISTICS OF A COHORT OF CHILDREN UNDER TWO YEARS OF AGE WITH DUCHENNE MUSCULAR DYSTROPHY**
Ana Sousa, Elisa Costa, Ricardo Taipa, Melo Pires, Manuela Santos; Porto, PT
- PS2Group1-014 NOVEL MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY WITH DELETION OF EXONS 834**
Tatiana Dimitrieva, Alexey Deikin, Denis Reshetov, Dmitry Vlodayets, Eugeniia Zotova; Moscow, RU
- PS2Group1-015 THE NEED FOR TRANSITION THE NEED FOR TRANSITION. WORKSHOP TC10.2**
Jiri Vajsar, Toronto, ON, CA
- PS2Group1-016 A CASE REPORT OF A 10 YEAR OLD BOY WITH COMBINATION OF DMD AND DOWN SYNDROME**
Dmitry Vlodayets, Marina Komarova, Denis Reshetov; Moscow, RU
- PS2Group1-017 PROGNOSTIC MODEL FOR 1-YEAR CHANGE IN 6-MINUTE WALK DISTANCE (6MWD) IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY (DMD)**
Nathalie Goemans¹, James Signorovitch², Elyse Swallow², Jinlin Song², Susan Ward³; ¹Leuven, BE, ²Boston, MA, US, ³Cambridge, MA, US
- PS2Group1-018 DIAGNOSIS OF DUCHENNE MUSCULAR DYSTROPHY IN ITALY: CRITICAL ISSUES AND AREAS FOR IMPROVEMENTS**
Adele D'Amico¹, Michela Catteruccia¹, Marika Pane¹, Giovanni Baranello², Alessandra Govoni², Sonia Messina³, Maria Grazia D'angelo⁴, Luisa Politano⁵, Ksenjia Gorni², Stefano Carlo Previtali², Antonella Pini⁶, Roberta Battini⁷, Angela Berardinelli⁸, Federica Ricci⁹, Elena Pegoraro¹⁰, Claudio Bruno¹¹, Federica Trucco¹¹, Barbara Panasisi², Giuseppe Vita³, Tiziana Mongini⁹, Maurizio Moggio², Giacomo Pietro Comi², Eugenio Mercuri¹, Enrico Bertini¹; ¹Rome, IT, ²Milan, IT, ³Messina, IT, ⁴Bosiso Parini, IT, ⁵Naples, IT, ⁶Bologna, IT, ⁷Pisa, IT, ⁸Pavia, IT, ⁹Turin, IT, ¹⁰Padua, IT, ¹¹Genoa, IT
- PS2Group1-019 THE PROPHYLACTIC USE OF PAMIDRONATE ON GLUCOCORTICOID-INDUCED BONE LOSS IN THE MDX MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY**
Sung-Hee Yoon, Jinghan Chen, Marc Grynepas, Jane Mitchell; Toronto, ON, CA
- PS2Group1-020-TRPV2 INHIBITION THERAPY CAN BE EFFECTIVE FOR CARDIOMYOPATHY OF MUSCULAR DYSTROPHY**
Tsuyoshi Matsumura¹, Misa Matsui¹, Yuko Iwata², Masanori Asakura², Toshio Saito¹, Harutoshi Fujimura¹, Saburo Sakoda¹; ¹Toyonaka, JP, ²Suita, JP

- PS2Group1-021 A MISSENSE MUTATION IN THE PUTATIVE SARCOPLASMIC RETICULUM TRANSMEMBRANE PROTEIN DCST2 CAUSES THE STRONGMAN SYNDROME**
Talita Conte¹, Martine Tétreault^{1,2}, Marie-Josée Dicaire³, Sylvie Provost⁴, Najwa Al-Bustani⁵, Marie-Pierre Dubé⁵, Véronique Bolduc⁵, Myriam Srour⁶, Erin O'Ferrall⁵, Jean-Pierre Bouchard⁷, Gina Ravenscroft⁸, Russell Hepple⁵, Tanja Taivassalo⁵, Nigel Laing⁹, Phillipa Lamont¹⁰, Jean Mathieu¹¹, Bernard Brais⁵; ¹2b4, QC, CA, ²B, QC, CA, ³B, CA, ⁴C, QC, CA, ⁵Montreal, QC, CA, ⁶Montreal, QC, CA, ⁷Quebec, QC, CA, ⁸Nedlands, AU, ⁹Nedlands, WA, AU, ¹⁰Perth, WA, AU, ¹¹Jonquiere, QC, CA
- PS2Group1-022 HEREDITARY MYOPATHY WITH EARLY RESPIRATORY FAILURE**
Sandra Sousa¹, Jorge Oliveira², Emília Vieira², Teresa Coelho², Manuela Santos², Marcio Cardoso², Ricardo Taipa², Melo Pires², Rosário Santos²; ¹Cascais, PT, ²Porto, PT
- PS2Group1-023 GNE MYOPATHY: MILESTONES AND DISEASE PROGRESSION BASED ON PATIENT SELF-REPORTED DATA COLLECTED THROUGH THE GLOBAL PATIENT REGISTRY**
Oksana Pogoryelova¹, Phillip Cammish², Supriya Rao³, Ed Conner³, Alison Skrinar³, Hanns Lochmüller⁴; ¹Newcastle Upon Tyne, UK, ²Newcastle Upon Tyne, UK, ³Novato, US, ⁴Newcastle, UK
- PS2Group1-024 MULTISYSTEM PROTEINOPATHY WITH MOTOR NERVE CONDUCTION BLOCKS**
Oscar Trujillo, Juan Casar, Roger Gejman, Ricardo Fadic; Santiago, CL
- PS2Group1-025 PHENOTYPIC CHARACTERIZATION AND PATTERN OF MUSCLE INVOLVEMENT IN GNE MYOPATHY**
Veeramani Preethish-Kumar¹, Oksana Pogoryelova², Kiran Polavarapu¹, Narayanappa Gayathri¹, Seena Vengalil¹, Judith Hudson², Chandrajit Prasad¹, Hanns Lochmüller³, Atchayaram Nalini¹; ¹Bangalore, IN, ²Newcastle Upon Tyne, UK, ³Newcastle, UK
- PS2Group1-026 CALPAINOPATHIES IN CHILE**
Jorge Bevilacqua¹, Yves Mathieu², Martin Krahn², Marc Bartoli², Claudia Castiglioni¹, Karin Kleinsteuber¹, Jorge Díaz¹, Francesca Puppo², Mathieu Cerino², Sebastien Courrier², Svetlana Gorokhova², Alejandra Tringulao¹, Natalia Miranda¹, Patricio Gonzalez-Hormazabal¹, María De Los Ángeles Avaria¹, J Urtizberea³, Pablo Caviedes¹, Lilian Jara¹, Nicolas Levy²; ¹Santiago, CL, ²Marseille, FR, ³Hendaye, FR
- PS2Group1-027 THE FIRST FUNCTIONALLY MATURE HUMAN PRIMARY IN VITRO MUSCLE MODEL: A NEW PARADIGM TO EXPLORE MUSCLE PHYSIOPATHOLOGY AND ACCELERATE DRUG DISCOVERY FOR MUSCLE DISORDERS**
Joris Michaud¹, Mathieu Fernandes¹, Eve Duchemin-Pelletier¹, Pauline Poydenot¹, Pauline Menager²; ¹Grenoble, FR, ²Bethesda, US
- PS2Group1-028 DOMINANT TRUNCATING MUTATIONS IN THE A-BAND OF TTN ARE A CAUSE OF LIMB-GIRDLE MUSCULAR DYSTROPHY WITH CARDIOMYOPATHY**
Jennifer Roggenbuck, Ana Morales, Ray Hershberger, John Kissel; Columbus, OH, US
- PS2Group1-029 RESCUE OF FOLDING DEFECTIVE ALPHA-SARCOGLYCAN MUTANTS BY MEANS OF PROTEIN FOLDING CORRECTORS**
Chiara Fecchio¹, Marcello Carotti¹, Elisa Bianchini¹, Romeo Betto¹, Roberta Sacchetto², Dorianna Sandona¹; ¹Padova, IT, ²Legnaro (pd), IT
- PS2Group1-030 CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: ONE-YEAR FOLLOW-UP**
Meredith James¹, Ursula Moore¹, Anna Mayhew¹, Michelle Eagle¹, Karen Bettinson¹, Elena Pegoraro², Kate Bushby¹; ¹Newcastle Upon Tyne, UK, ²Padova, IT
- PS2Group1-031 HISTOPATHOLOGICAL AND CLINICAL CHARACTERIZATION OF A SPORADIC TNPO3-MUTATED PATIENT**
Alessandra Ruggieri¹, Sara Gibertini¹, Barbara Pasanisi¹, Vincenzo Nigro², Marco Savarese², Maurizio Moggio¹, Corrado Angelini³, Renato Mantegazza¹, Lorenzo Maggi⁴, Lucia Morandi¹, Marina Mora¹; ¹Milan, IT, ²Naples, IT, ³Venezia Lido, IT, ⁴Milano, IT
- PS2Group1-032 ACE-083, A LOCALLY-ACTING MUSCLE AGENT, INCREASES MUSCLE VOLUME IN HEALTHY VOLUNTEERS**
Kenneth Attie¹, Chad Glasser¹, Michael Gartner², Brian Boes², R Pearsall¹, Xiaosha Zhang¹, Jade Sun¹, Brian Vidal¹, Ashley Bellevue¹, Monty Hankin¹, Matthew Sherman¹; ¹Cambridge, MA, US, ²Lincoln, NE, US
- PS2Group1-033 CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: CLINICAL DATA FROM BASELINE ASSESSMENTS**
Elizabeth Harris¹, Ursula Moore¹, Catherine Bladen¹, Anna Mayhew¹, Meredith James¹, Karen Bettinson¹, Heather Hilsden¹, Hillarie Windish², The Jain Foundation COS Consortium¹, Kate Bushby¹; ¹Newcastle Upon Tyne, UK, ²Seattle, WA, US

- PS2Group1-034 MOLECULAR PATHOGENESIS OF CAVEOLIN-3-RELATED LIMB-GIRDLE-MUSCULAR-DYSTROPHY**
José Andrés González Coraspe¹, Denisa Hathazi², Hanns Lochmüller³, René Zahedi², Joachim Weis¹, Andreas Roos³; ¹Aachen, DE, ²Dortmund, DE, ³Newcastle, UK
- PS2Group1-035 TRIM32 GENE MUTATIONS DETECTED BY NEXT GENERATION SEQUENCING**
Judith Hudson¹, Eileen Graham¹, Chiara Marini Bettolo¹, Teresinha Evangelista¹, Volker Straub¹, Fiona Norwood², Kate Bushby¹, Rita Barresi¹; ¹Newcastle Upon Tyne, UK, ²London, UK
- PS2Group1-036 MUSCLE INVOLVEMENT IN LIMB GIRDLE MUSCULAR DYSTROPHY WITH GMPPB DEFICIENCY (LGMD2T)**
Sofie Østergaard, Copenhagen, DK
- PS2Group1-037 MUSCLE MRI CAN BE A POWERFUL TOOL TO DIAGNOSE LIMB GIRDLE MUSCULAR DYSTROPHY 2L**
Maria Elena Farrugia¹, Cheryl Longman¹, William Stewart¹, Volker Straub², Richard Petty¹; ¹Glasgow, UK, ²Newcastle Upon Tyne, UK
- PS2Group1-038 MOLECULAR DIAGNOSIS OF BETA-SARCOGLYCANOPATHY: REPORTING TWO NOVEL MUTATIONS IN IRAN**
Marzieh Mojbafan¹, Sirous Zeinali¹, Seyed Hasan Tonekaboni¹, Abdolazim Nejati Zadeh², Yalda Nili Pour¹; ¹Tehran, IR, ²Bandar Abbas, IR
- PS2Group1-040 A NOVEL PATHOGENIC MUTATION IN TPM3 GENE IN A 5 YEARS OLD IRANIAN PATIENT WITH AUTOSOMAL RECESSIVE NEMALINE MYOPATHY-1**
Saeid Morovvati, Yashar Morovvati; Tehran, IR
- PS2Group1-041 MTM1-RELATED MYOPATHY CARRIER FEMALES MANIFEST SIGNIFICANT ASYMMETRIES AND A SPECTRUM OF MUSCLE INVOLVEMENT**
Benjamin Cocanougher¹, Pomi Yun², Lauren Flynn³, Mina Jain², Melissa Waite², Ruhi Vasavada², Jason Wittenbach⁴, Sabine de Chastonay⁵, Sandra Donkervoort⁶, A Foley², Carsten Bonnemann²; ¹Ashburn, US, ²Bethesda, US, ³Bethesda, MD, US, ⁴Ashburn, VA, US, ⁵Torrance, CA, US, ⁶1477, MD, US
- PS2Group1-042 P4HA1 MUTATIONS CAUSE A UNIQUE CONGENITAL DISORDER OF CONNECTIVE TISSUE INVOLVING TENDON, BONE, MUSCLE AND THE EYE**
Sandra Donkervoort¹, Yaqun Zou², Antti Salo³, Aileen Barnes⁴, Ying Hu², A Foley², Elena Makareeva², Meganne Leach², Wendy DiNonno⁴, Jahannaz Dastgir², Ronald Cohn⁵, Sergey Leikin², Joan Marini², Johanna Myllyharju³, Carsten Bonnemann²; ¹1477, MD, US, ²Bethesda, MD, US, ³Oulu, FI, ⁴Newport News, VA, US, ⁵Toronto, ON, CA
- PS2Group1-043 DE NOVO DOMINANT MOSAIC MUTATIONS IN COLLAGEN 6 GENES: UNCOMMON CAUSE OF BETHLEM AND ULLRICH MYOPATHIES THAT MAY BE MISSED BY SANGER SEQUENCING**
Adele D'Amico¹, Fabiana Fattori¹, Francesca Gualandi², Stefania Petrini¹, Valentina Doria¹, Giorgio Tasca¹, Michela Catteruccia¹, Marcello Niceta¹, Marco Tartaglia¹, Alessandra Ferlini², Enrico Bertini¹; ¹Rome, IT, ²Ferrara, IT
- PS2Group1-044 A PROSPECTIVE STUDY OF HISTOLOGY, PHENOTYPES AND GENETICS IN CONGENITAL MYOPATHY PATIENTS ABOVE 5 YEARS OF AGE IN DENMARK**
Nanna Witting¹, Ulla Werlauff², Morten Duno¹, John Vissing¹; ¹Copenhagen, DK, ²Aarhus, DK
- PS2Group1-045 IDENTIFYING AND CHARACTERIZING NOVEL MUTATIONS CAUSING ARTHROGRYPOSIS**
Forough Noohi, Martine Tétreault, Jacek Majewski, Bernard Brais; Montreal, QC, CA
- PS2Group1-046 DESMINOPATHY IN CHILE, TWO FIRST CASES REPORTED**
Jorge Bevilacqua¹, Lidia González-Quereda², Ivonne Zamorano³, Claudia Castiglioni¹, Lorena Acevedo¹, Jorge Díaz¹, María José Rodríguez², Alejandra Trangulao¹, Mario Rivera¹, Pia Gallano²; ¹Santiago, CL, ²Barcelona, ES, ³Puerto Montt, CL
- PS2Group1-047 MULTIPLE DELETIONS IN MITOCHONDRIAL DNA IN MYOFIBRILLAR MYOPATHY AND CENTRONUCLEAR MYOPATHY**
Jochen Schaefer, Heinz Reichmann, Sandra Jackson; Dresden, DE
- PS2Group1-048 THE UK MYOTONIC DYSTROPHY PATIENT REGISTRY**
Nikoletta Nikolenko¹, Libby Wood², Chris Turner³, David Hilton-Jones⁴, Antonio Atalaia¹, Chiara Marini-Bettolo¹, Paul Maddison⁵, Margaret Philips⁶, Mark Roberts⁷, Mark Rogers⁸, Volker Straub¹, Simon Hammans⁹, Hanns Lochmüller¹; ¹Newcastle Upon Tyne, UK, ²Bz, UK, ³London, UK, ⁴Oxford, UK, ⁵Nottingham, UK, ⁶Derby, UK, ⁷Salford, UK, ⁸Cardiff, UK, ⁹Southampton, UK
- PS2Group1-049 18F-FDG-PET STUDY IN PATIENTS WITH MYOTONIC DYSTROPHY TYPE 1 AND 2**
Vidosava Rakocevic Stojanovic, Stojan Peric, Vera Ilic, Aleksandra Parojcic, Jovan Pesovic, Dusanka Savic-Pavicevic, Lepasava Brajkovic; Belgrade, RS

- PS2Group1-050 ELEVATED PLASMA LEVELS OF CARDIAC TROPONIN-I PREDICT LEFT VENTRICULAR SYSTOLIC DYSFUNCTION IN PATIENTS WITH MYOTONIC DYSTROPHY TYPE 1: A COHORT FOLLOW-UP STUDY**
Mark Hamilton¹, Yvonne Robb², Helen Gregory³, Sarah Cumming¹, Berit Adam¹, Josephine McGhie¹, Anneli Cooper¹, Jillian Couto¹, Alexis Duncan¹, Monika Rahman¹, Anne McKeown¹, Alison Wilcox¹, Catherine McWilliam⁴, Maria Elena Farrugia¹, Richard Petty¹, Cheryl Longman¹, Iain Findlay¹, Alan Japp², Darren Monckton¹, Martin Denvir²; ¹Glasgow, UK, ²Edinburgh, UK, ³Aberdeen, UK, ⁴Dundee, UK
- PS2Group1-051 ONE-YEAR MRI-FOLLOW-UP IN 45 PATIENTS WITH FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY**
Grete Andersen¹, Julia Dahlqvist¹, Christoffer Vissing¹, Karen Pedersen², Carsten Thomsen¹, John Vissing¹; ¹Copenhagen, DK, ²Copenhagen E, DK
- PS2Group1-052 OCULOPHARYNGODISTAL MYOPATHY, A CASE REPORT FROM INDIA**
Seena Vengalil, Atchayaram Nalini, Veeramani Preethish-Kumar, Kiran Polavarapu, Narayanappa Gayathri, Anita Mahadevan; Bangalore, IN
- PS2Group1-053 NEUROMUSCULAR ELECTRICAL STIMULATION TRAINING OF THE TIBIALIS ANTERIOR MUSCLE IN FSHD1 PATIENTS**
Jeremy Garcia, Aude Doix, Pauline Lahaut, Véronique Tanant, Manuella Fournier-Mehouas, Serge Colson, Claude Desnuelle, Sabrina Sacconi; Nice, FR
- PS2Group1-054 SPEECH IMPAIRMENT IS COMMON IN EARLY ONSET FACIOSCAPULOHUMERAL DYSTROPHY**
Megan Hodge¹, Jia Feng², Jean Mah³, Cooperative International Neuromuscular Research Group Investigators²; ¹Edmonton, AB, CA, ²Washington, DC, US, ³Calgary, AB, CA
- PS2Group1-057 POMPE DISEASE IN AUSTRIA**
Wolfgang Löscher¹, Thomas Stulnig², Phillip Simschitz³, Michaela Brunner-Krainz⁴, Martina Huemer⁵, Stephan Iglseider⁶, Florian Lagler⁷, Herman Moser⁸, Stefan Quasthoff⁴, Julia Wanschitz¹, Wolfgang Grisold⁹; ¹Innsbruck, AT, ²Vienna, AT, ³Klagenfurt, AT, ⁴Graz, AT, ⁵Bregenz, AT, ⁶Linz, AT, ⁷Salzburg, AT, ⁸Altmünster Am Traunsee, AT, ⁹US
- PS2Group1-058 PHASE 1 SAFETY, PHARMACOKINETICS, AND EXPLORATORY EFFICACY OF NEOGAA, A NOVEL ENZYME REPLACEMENT THERAPY, IN TREATMENT-NAIVE AND ALGLUCOSIDASE ALFA-TREATED LATE-ONSET POMPE DISEASE PATIENTS**
Loren Pena¹, Richard Barohn², Barry Byrne³, Claude Desnuelle⁴, Ozlem Goker-Alpan⁵, Shafeeq Ladha⁶, Pascal Laforêt⁷, Eugen Mengel⁸, Alan Pestronk⁹, Benedikt Schoser¹⁰, Volker Straub¹¹, Jaya Trivedi¹², Philip Van Damme¹³, John Vissing¹⁴, Peter Young¹⁵, Beth Thurberg¹⁶, Raheel Shafi¹⁶, Kerry Culm-Merdek¹⁶, Gerard Short¹⁶, Ans van der Ploeg; for the NEO1 Investigator Group¹⁷; ¹Durham, NC, US, ²Kansas City, KS, US, ³Jacksonville, FL, US, ⁴Nice, FR, ⁵Fairfax, VA, US, ⁶Phoenix, AZ, US, ⁷Paris, FR, ⁸Mainz, DE, ⁹St Louis, MO, US, ¹⁰München, DE, ¹¹Newcastle Upon Tyne, UK, ¹²Dallas, TX, US, ¹³Leuven, BE, ¹⁴Copenhagen, DK, ¹⁵Münster, DE, ¹⁶Cambridge, MA, US, ¹⁷Rotterdam, NL
- PS2Group1-060 REGULATION OF LIPOPHAGY AND LIPOLYSIS IN LIPID STORAGE MYOPATHIES AND CPT DEFICIENCY**
Corrado Angelini¹, Elisabetta Tasca², Anna Nascimbeni³; ¹Venezia Lido, IT, ²Venice, IT, ³Paris, FR
- PS2Group1-062 ENERGY DEFICIT IN THE MCA RDLE MOUSE MODEL AFFECTS CALCIUM HOMEOSTASIS AND FORCE GENERATION**
Thomas Krag¹, Tomas Pinos², John Vissing¹; ¹Copenhagen, DK, ²Barcelona, ES
- PS2Group1-065 EFFECT OF SENSORY INTEGRATION THERAPY ON FINE MOTOR FUNCTION IN MITOCHONDRIAL MYOPATHY**
Berkant Torpil, Semin Akel, Sedef Şahin; Ankara, TR
- PS2Group1-066 PREVALENCE STUDY OF MUSCLE CHANNELOPATHIES IN ITALY**
Lorenzo Maggi¹, Mauro Lo Monaco², Simona Portaro³, Giovanni Meola¹, Jean Francois Desaphy⁴, Sabrina Lucchiarini¹, Serena Pagliarini¹, Raffaele Dubbioso⁵, Pia Bernasconi¹, Raffaella Brugnoli¹, Paola Imbrici¹, Giacomo Pietro Comi⁶, Renato Mantegazza⁶, Maria Trojano⁴, Adele D'amico⁷, Lucio Santoro⁵, Elena Pegoraro⁸, Luisa Politano⁹, Tiziana Mongini¹⁰, Liliana Vercelli¹¹, Gabriele Siciliano¹², Giulia Ricci¹², Diana Conte-Camerino⁴, Antonio Toscano³, Valeria Sansone¹; ¹Milano, IT, ²Roma, IT, ³Messina, IT, ⁴Bari, IT, ⁵Napoli, IT, ⁶Milan, IT, ⁷Rome, IT, ⁸Padua, IT, ⁹Naples, IT, ¹⁰Turin, IT, ¹¹Torino, IT, ¹²Pisa, IT
- PS2Group1-067 A COHORT OF PEDIATRIC AGE PATIENTS WITH NON-DYSTROPHIC MYOTONIA**
Joel Freitas, Ana Sousa, Teresa Coelho, Manuela Santos; Porto, PT

- PS2Group1-069** **THYROTOXIC PERIODIC PARALYSIS: DOES MUSCLE MEMBRANE DYSFUNCTION UNDERLIE DISEASE PATHOGENESIS?**
Nam-Hee Kim¹, Joong-Yang Cho¹, Kyung Seok Park²; ¹Goyangsi, KR, ²Seongnamsi, KR
- PS2Group1-070** **THE CULLIN 4A, B-DDB1-CEREBLON E3 UBIQUITIN LIGASE COMPLEX MEDIATES THE DEGRADATION OF CLC-1 CHANNELS RESULTING IN MYOTONIA CONGENITA**
Ssu-Ju Fu, Yi-An Chen, Yi-Jheng Peng, Chih-Yung Tang; Taipei, TW
- PS2Group1-071** **ELECTROMYOGRAPHY AND THE RISK OF PNEUMOTHORAX**
Charles Kassardjian¹, Cullen O’Gorman², Eric Sorenson³; ¹Toronto, ON, CA, ²Brisbane, QLD, AU, ³Rochester, MN, US
- PS2Group3-009** **LATE ONSET MYASTHENIA GRAVIS (LOMG): WHEN DOES IT START?**
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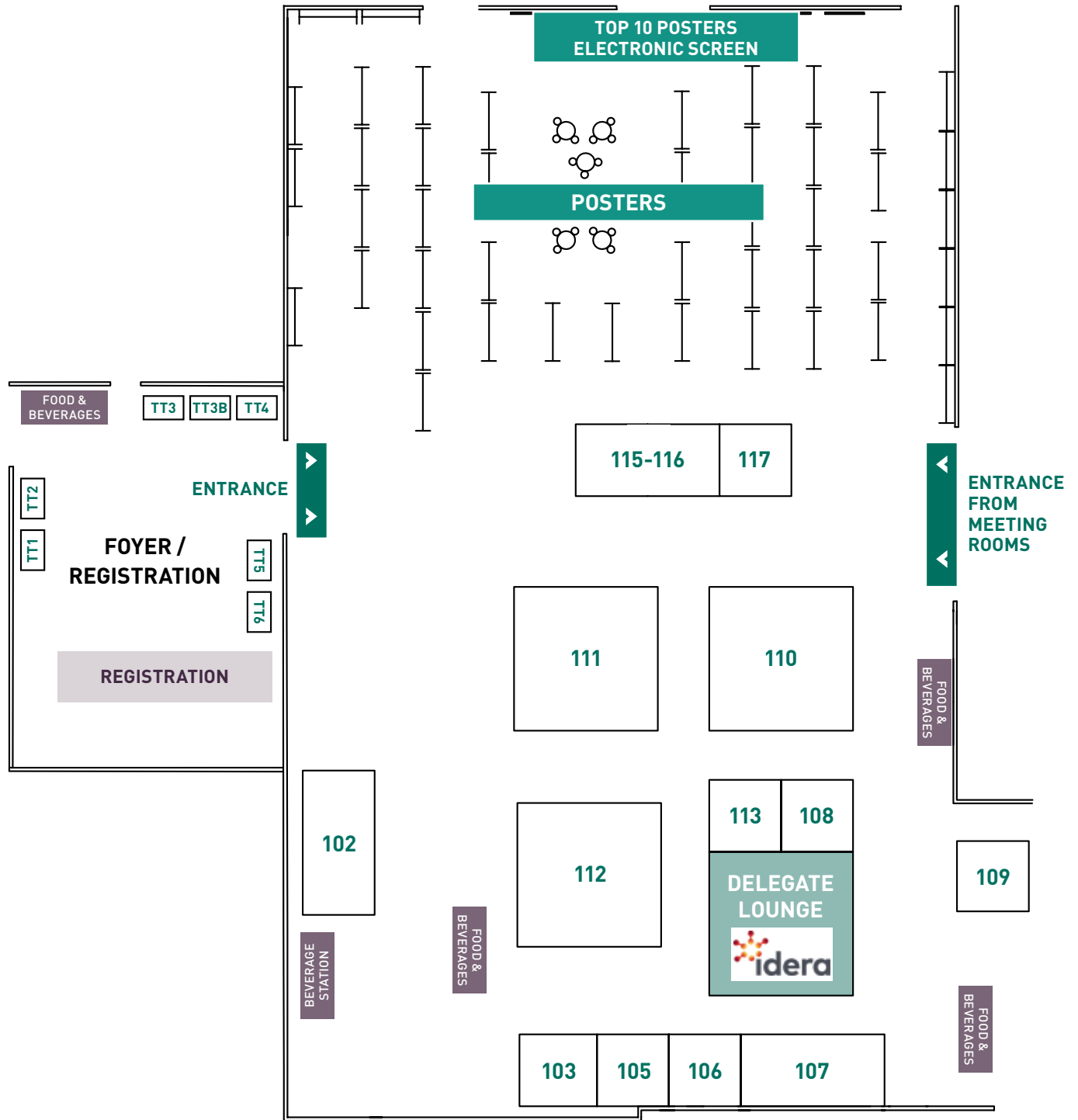
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BOOTH #: 110



Alexion is a global biopharmaceutical company focused on life-transforming therapies for patients with devastating, rare diseases. Alexion developed the first and only approved complement inhibitor for PNH & aHUS. The Company is strengthening and broadening its complement portfolio, including evaluating complement inhibition in additional ultra-rare disorders. Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for two ultra-rare, life-threatening disorders. (www.alexion.com)

BOOTH #: 115-116



Baxalta Incorporated (NYSE: BXL) is a global biopharmaceutical company developing, manufacturing and commercializing therapies for orphan diseases and underserved conditions in hematology, immunology and oncology. Launched in 2015, following its separation from Baxter, Baxalta's therapies are available in more than 100 countries. Baxalta is headquartered in Northern Illinois. (www.baxalta.com)

BOOTH #: 108



Since 1979, Cadwell has been achieving growth by responding to the needs of our end users and focusing on our core competencies. We continually develop our main solutions: EMG, IONM, EEG, Sleep, Consumables, and CadLink Data Enterprise. We are a globally focused company with worldwide business partners and clinical specialists. (www.cadwell.com)

BOOTH #: 113



CSL Behring is a global leader in the plasma protein biotherapeutics industry. We research, develop, manufacture and market biotherapies that are used to treat serious and rare conditions. Users of our therapies rely on them for their quality of life and, in many cases, for life itself. (www.cslbehring.ca)

BOOTH #: TT5



Improves the quality of life for individuals and families affected with GBS, CIDP, MMN, and variants. Serving patients with support, education, research, and advocacy. GBS is an inflammatory disorder of the peripheral nerves that affects 2 in 100,000 individuals, while CIDP, a chronic condition, can affect as high as 9 in 100,000 individuals. (www.gbs-cidp.org)

BOOTH #: 105



GeneDx is a highly respected laboratory, specializing in genetic testing for inherited disorders. GeneDx offers next-generation sequencing and deletion/duplication testing for inherited cardiac disorders, mitochondrial disorders, neurological disorders, inherited cancer disorders, prenatal disorders and other rare genetic disorders. GeneDx also offers whole exome sequencing and microarray-based testing. (www.GeneDx.com)

BOOTH #: 107**GRIFOLS**

Grifols is a global healthcare company founded in 1940, currently present in more than 100 countries worldwide and headquartered in Barcelona, Spain. In 2015 Grifols celebrates its 75th Anniversary of improving people's health and well-being through the development of life-saving plasma medicines, diagnostics systems, and hospital pharmacy products. (www.grifols.com)

BOOTH #: TT4

IOS Press, established in 1987, publishes around 100 international journals and approximately 90 book titles a year, in a broad range of subjects. IOS Press has a strong neurosciences package, which includes Journal of Alzheimer's Disease (Impact Factor 4.151, according to JCR 2014), Journal of Neuromuscular Diseases, Journal of Parkinson's Disease and Journal of Huntington's Disease. (www.iospress.com)

BOOTH #: 102

LFB is a leading European pharmaceutical group specializing in biological medicines, under hospital prescription, for serious and often rare diseases. LFB's products are currently marketed in more than 40 countries. (www.lfb.fr/en/home.html)

BOOTH #: 106**LifeLabs Genetics™**

LifeLabs is a Canadian-owned company with over 50 years of experience providing laboratory testing services to help healthcare providers diagnose, treat, monitor and prevent disease. LifeLabs Genetics is the largest privately owned genetics laboratory in Canada, offering more than 2,000 genetic and biochemical tests. (www.lifelabs.com)

BOOTH #: TT2**Muscular Dystrophy Canada**

Muscular Dystrophy Canada is at the heart of an active community of people across Canada who are affected by neuromuscular disorders. Our goal is to build a world without neuromuscular disorders and enhance the lives of Canadians with neuromuscular disorders by giving them the information, tools and support they need while we relentlessly search for new therapies and a cure. (www.muscle.ca)

BOOTH #: TT3

Myasthenia Gravis Society of Canada (MG Canada) seeks universal access for MG diagnosis and treatment for all Canadians. MG Canada is committed to advancing education and support for MG patients, MG caregivers and MG Health Professionals, and to encourage research for MG patient quality lifestyle and the quest for cure. (www.mgcanada.org)

BOOTH #: TT3B

Myositis/Myosite Canada is a non-profit, volunteer organization dedicated to improving the lives of people affected by myositis. It is governed by a Board that brings diverse strengths and perspectives. Mission: To support people with Myositis, caregivers, researchers and health care providers in diagnosing, treating, and rehabilitating Myositis people. (www.myositis.ca)

BOOTH #: 112

PTC Therapeutics is a global biopharmaceutical company focused on the discovery, development and commercialization 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 such as cystic fibrosis and Duchenne muscular dystrophy. (www.ptcbio.com)

BOOTH #: TT1

Founded in 2014, the RYR-1 Foundation supports research to find an effective treatment or a cure for RYR-1 related myopathy. The Foundation also provides resources for physician education, patient education, and patient advocacy. To learn more, please visit www.ryr1.org.

BOOTH #: 111

Sanofi Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. Visit www.genzyme.com.

BOOTH #: 117

Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company focused on the development and commercialization of prescription drugs to treat mitochondrial and neuromuscular diseases. The company is passionate about improving patients' quality of life and is well underway to become an industry leader in the treatment of rare diseases. (www.santhera.com)

BOOTH #: 109

Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We believe in the potential of blood to do even more for patients than it does today. This belief inspires our innovation and strengthens our collaboration with customers. (www.terumobct.com)

BOOTH #: TT6

TREAT-NMD is a network for the neuromuscular field that provides an infrastructure to ensure that the most promising new therapies reach patients quickly. Since its launch in January 2007 the network's focus has been on the development of 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 neuromuscular patients worldwide. (www.treat-nmd.eu)

BOOTH #: 103

Ultragenyx is a development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases. Ultragenyx has licensed rights to aceneuramic acid extended release and its potential use in treating GNE Myopathy from Nobelpharma, AAI Pharma, and the HIBM Research Group. (www.ultragenyx.com)



A PROUD SUPPORTER OF THE 14TH INTERNATIONAL CONGRESS ON NEUROMUSCULAR DISEASES

Idera Pharmaceuticals is a biopharmaceutical company developing novel nucleic acid therapeutics for people with rare diseases and cancer. With our proprietary Toll-like receptor (TLR) targeting technology, we design oligonucleotide-based therapies to modulate the immune activity of specific TLRs. Our rapidly advancing clinical programs now aim to translate this pioneering science into advances in patient care.

» **Please join us for a symposium
on Advances in Myositis** on
Thursday, July 7, at 7:00 AM in
Osgoode Ballroom West. See our
delegate bag flier for more details.

IderaPharma.com

This symposium was approved by the Program Committee as an independent activity held in conjunction with the 14th International Congress on Neuromuscular Diseases. This symposium is not sponsored or endorsed by ICNMD 2016.

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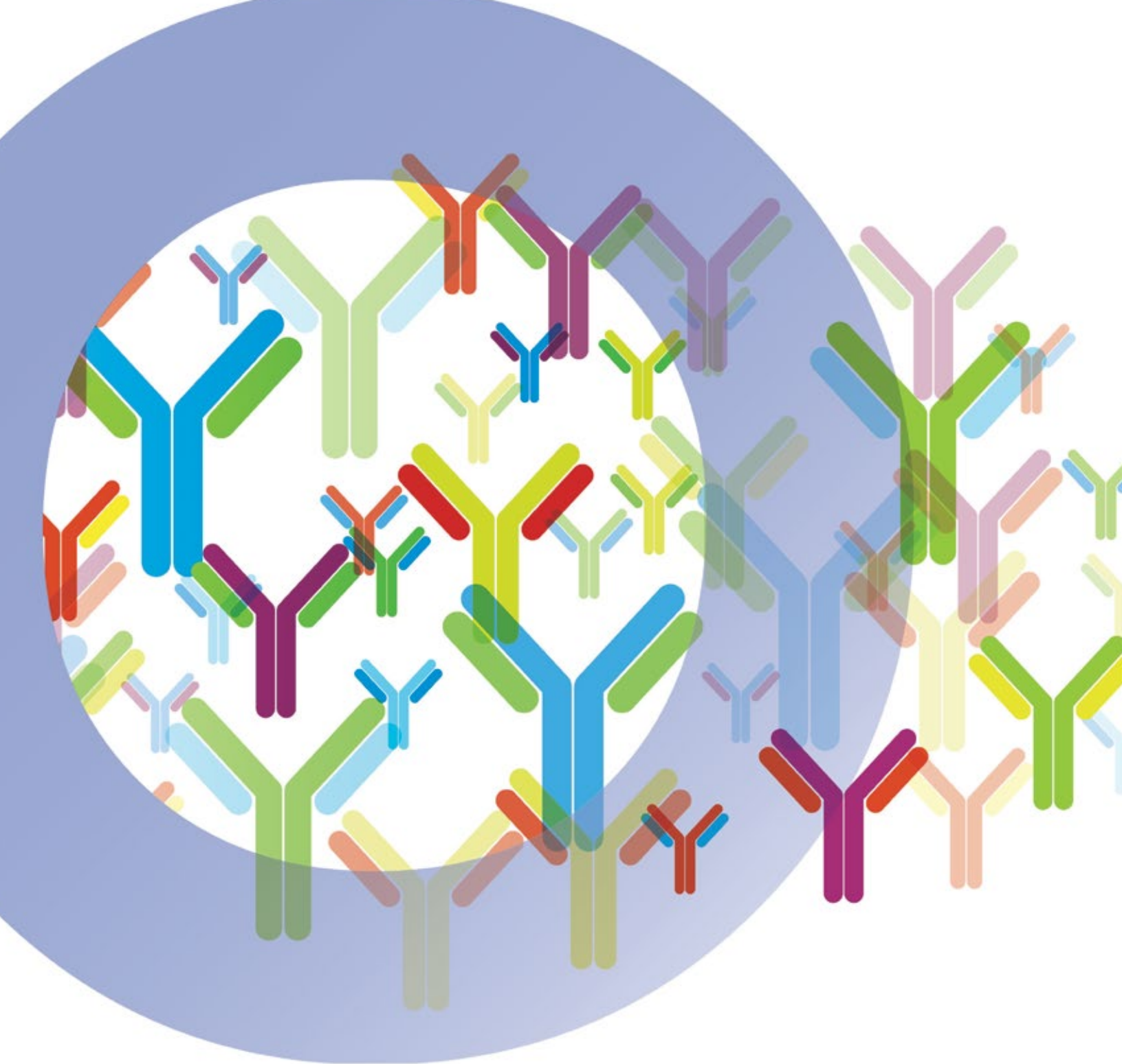
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ADVANCING MITOCHONDRIAL MEDICINE

IN NEUROMUSCULAR DISEASES

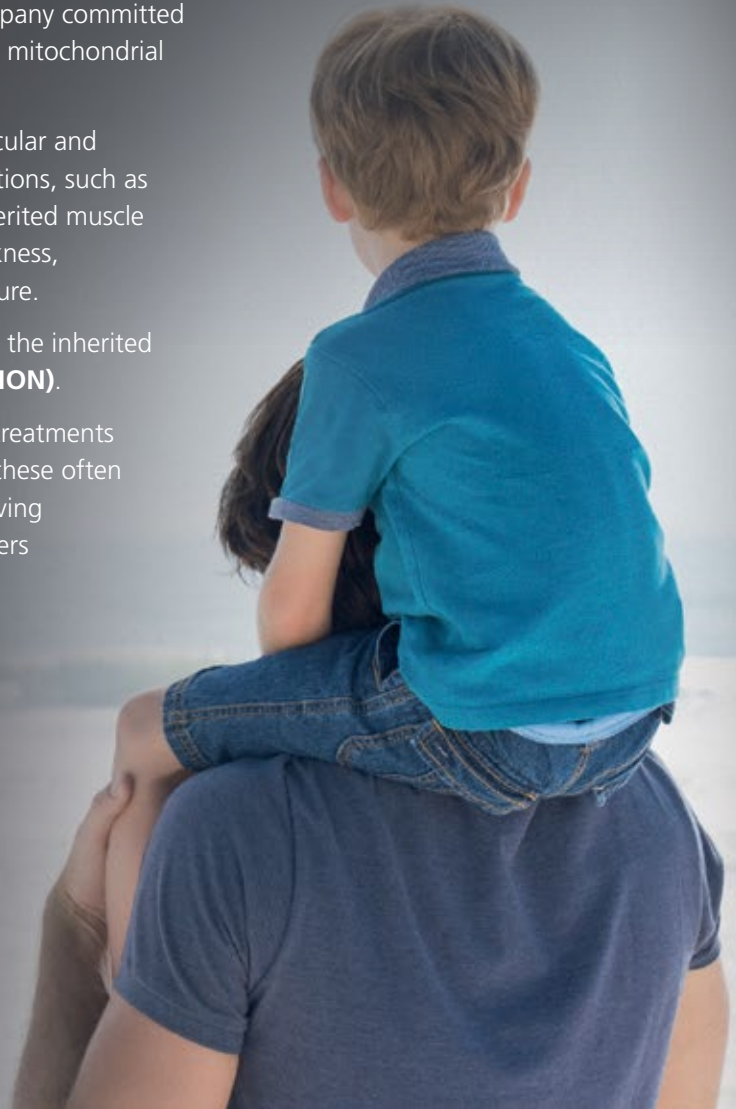
Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company committed to developing medicines to meet the needs of patients living with mitochondrial disorders and other rare diseases.

We are focused on the development of treatments for neuromuscular and neuro-ophthalmological diseases that currently lack treatment options, such as the devastating **Duchenne muscular dystrophy (DMD)**, an inherited muscle wasting disease that affects young boys causing progressive weakness, loss of mobility and ultimately death due to cardio-respiratory failure.

In the field of ophthalmology, we have developed a treatment for the inherited mitochondrial disease **Leber's hereditary optic neuropathy (LHON)**.

We have dedicated more than a decade to researching potential treatments in our ongoing mission to make available effective medicines for these often overlooked, life-altering diseases. We are passionate about improving patients' quality of life and are well on the way to becoming leaders in rare mitochondrial and neuromuscular disorders.

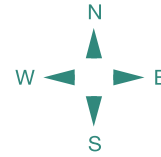
To find out more please visit our stand
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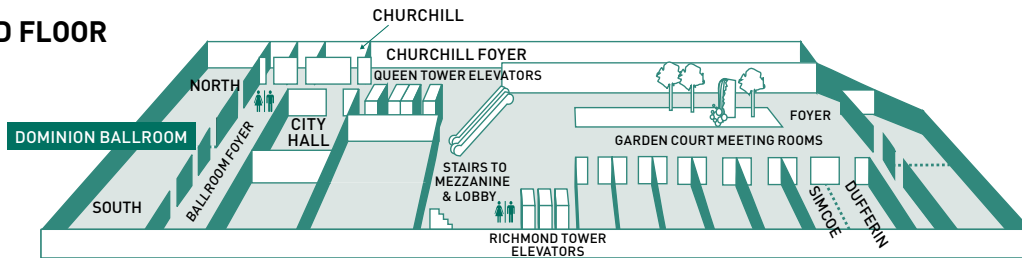
VENUE MAPS

Session Rooms

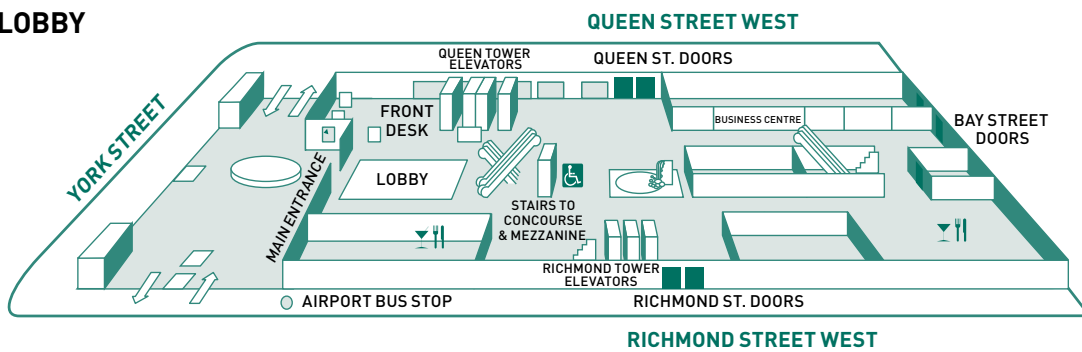
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 - 2 DOMINION BALLROOM
 - 2 DUFFERIN
 - LC GRAND BALLROOM
 - LC GRAND BALLROOM FOYER
 - LC OSGOODO BALLROOM
 - 2 SIMCOE
- LC EXHIBIT HALL
 - LC REGISTRATION



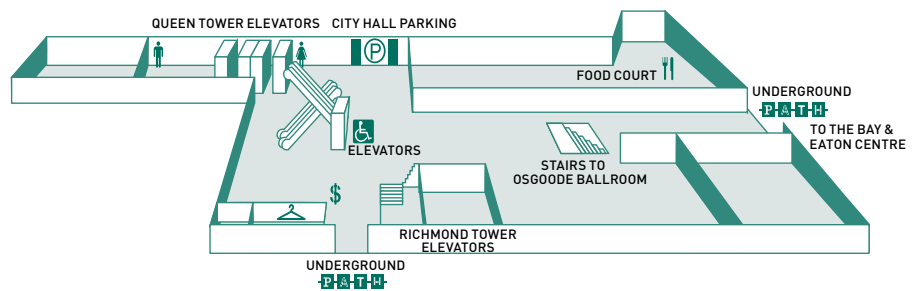
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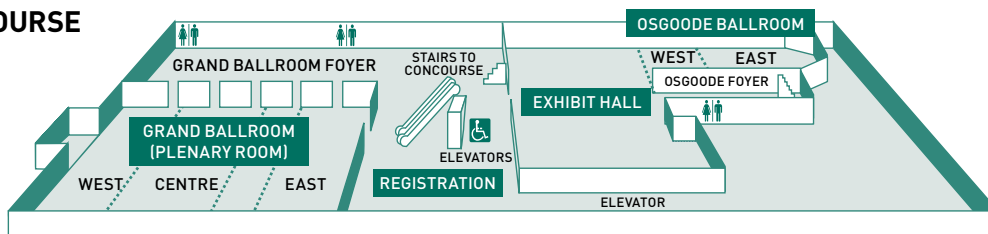
LOBBY



CONCOURSE



LOWER CONCOURSE

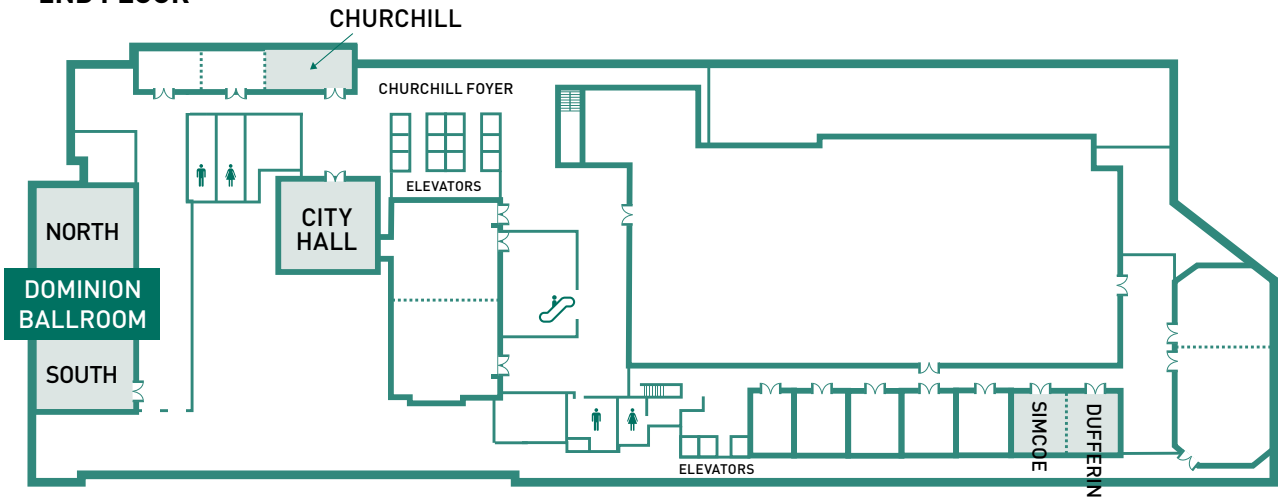


VENUE MAPS

PRE-CONGRESS TEACHING COURSES ▶ TUESDAY JULY 5, 2016

All Pre-Congress Teaching Course Sessions are located on the 2nd Floor

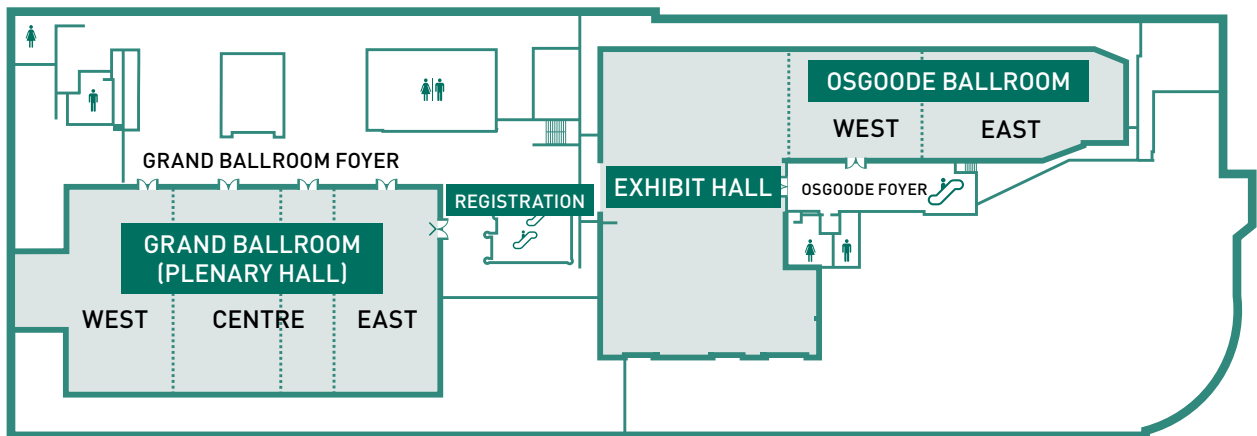
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CONGRESS PROGRAM ▶ WEDNESDAY JULY 6, 2016
 ▶ THURSDAY JULY 7, 2016
 ▶ FRIDAY JULY 8, 2016
 ▶ SATURDAY JULY 9, 2016

All Plenary and Workshop Sessions are located on the Lower Concourse

LOWER CONCOURSE





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Recognizing the Patient with Refractory Myasthenia Gravis: An Interactive Workshop

Sheraton Centre Toronto Hotel | Grand Ballroom West
Saturday, 9 July | 7:00 – 8:00 AM

A light breakfast will be served from 6:45 – 7:00 AM

OVERVIEW

Join us for an interactive workshop with our expert faculty panel to discuss the patient with refractory MG. This discussion will provide an unique forum to engage with faculty on refractory case profiles and the use of assessment tools in the clinic, such as the MG-ADL scale. Our faculty panel will directly engage you and all members of the audience through an ongoing question-and-answer session.

No CME/CNE credits are associated with this program.

FACULTY



Srikanth Muppidi, MD
Stanford University
School of Medicine



Richard J. Nowak, MD, MS
Yale School of Medicine



Nicholas J. Silvestri, MD
University at Buffalo
School of Medicine and
Biomedical Sciences



Gil I. Wolfe, MD, FAAN
University at Buffalo
School of Medicine and
Biomedical Sciences

AGENDA

Welcome and
Introductions

7:00 – 7:10 AM

Concluding
Remarks

7:50 – 8:00 AM

7:10 – 7:50 AM

Interactive
Workshop

8:00 AM

Meeting
Adjournment

This presentation was approved by the Program Committee as an independent activity held in conjunction with the 14th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2016.