July 5–9, 2016 Toronto, Canada

ICNMD 2016

14th International Congress on Neuromuscular Diseases

Onsite Program

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ICNMD2016.ORG

Ben | United Kingdom

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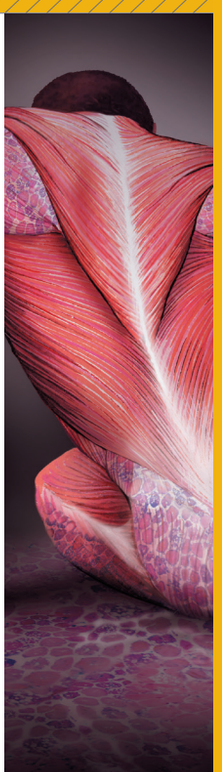
POMPE DISEASE

Sanofi Genzyme help's people with debilitating and complex conditions that are often difficult to diagnose and treat. Our approach is shaped by our experience developing highly specialized treatments and forging close relationships with physicians and patient communities.



POMPE
disease

To learn more, visit us at booth #111.

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CONTENTS

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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 of Medicine, US

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London Health Sciences Centre, CA

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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 Genge
Montreal 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 Russell
University of Maryland School of Medicine, US

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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

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LSUHSC School of Medicine, US

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Istituto Neurologico Carlo Besta, IT

Mary Reilly
University College London, UK

Gerard Said
Hôpital de la Salpétrière, 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, outlines, 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 Facioscapulohumeral 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

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

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**

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 SUB-JECTS 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

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

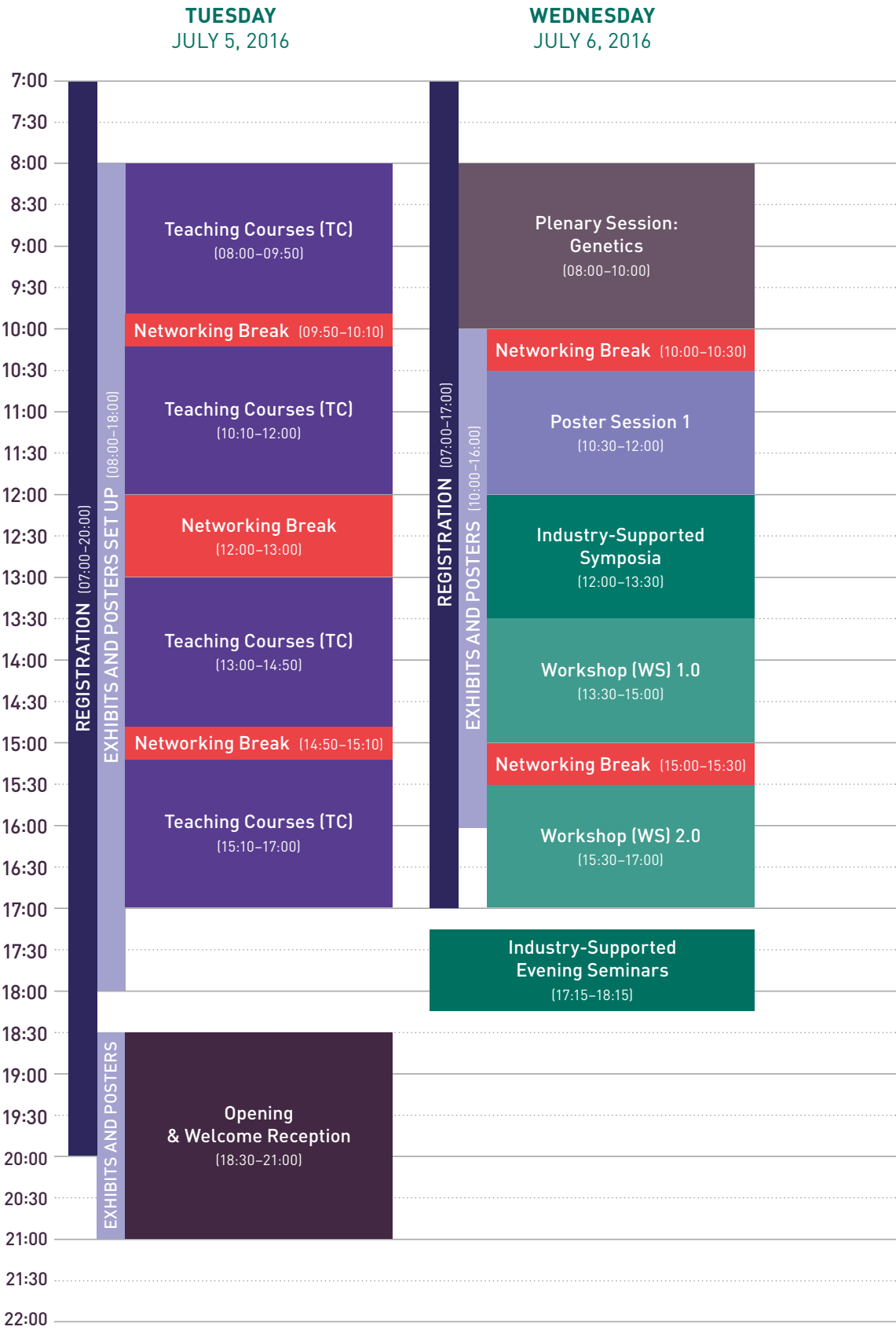
EXHIBIT HOURS

WEDNESDAY, JULY 6
- 10:00–16:00

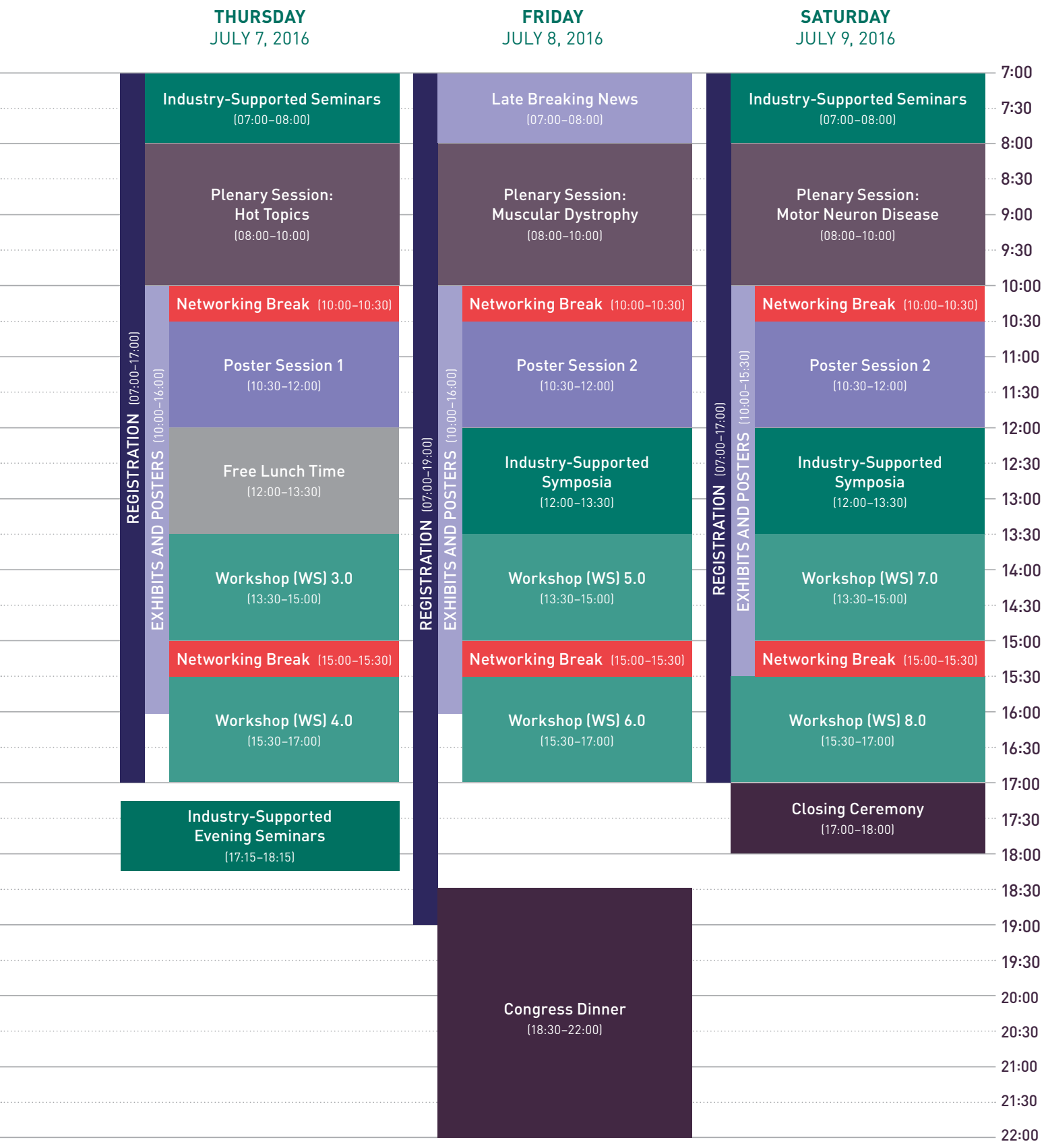
THURSDAY, JULY 7
- 10:00–16:00

FRIDAY, JULY 8
- 10:00–16:00

SATURDAY, JULY 9
- 10:00–15:30



Program listings are subject to change.



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 Thaisetthawatkul, 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 GLYCOGENOSES 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

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

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

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

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.

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

07:00–08:00 INDUSTRY-SUPPORTED SYMPOSIA
See page 33 for information

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

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

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.

12:00–13:30 INDUSTRY-SUPPORTED SYMPOSIUM
See page 33 for information

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 7.1.1–IGG4 AUTOANTIBODIES RELATED TO NEUROMUSCULAR DISEASES: THERAPEUTIC IMPLICATIONS**
Luis Querol, ES

14:15–15:00 **WS 7.1.2–IS IT ALL ABOUT THE ANTIBODIES**
Andrew Mammen, US

13:30–15:00 Workshop WS 7.2 Evaluation of Variants of Unknown Significance

ROOM› Osgoode Ballroom East

Chair: Mary Reilly, UK

13:30–14:15 **WS 7.2.1–NERVE**
Mary Reilly, UK

14:15–15:00 **WS 7.2.2–MUSCLE**
Raveen Basran, CA

13:30–15:00 Workshop WS 7.3 Inclusion Body Myopathy

ROOM› Grand Ballroom East

Chair: Mazen Dimachkie, US

13:30–14:15 **WS 7.3.1–GENERAL TREATMENT APPROACHES**
Mazen Dimachkie, US

14:15–15:00 **WS 7.3.2–ONGOING DEVELOPMENTS IN IBM**
Anthony A. Amato, US

13:30–15:00 Workshop WS 7.4 Modern Concepts in Spinal Muscular Atrophy

ROOM› Grand Ballroom West

Chair: Susan Iannaccone, US

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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AUDENTES

ON-SITE RESOURCES AND AMMENITIES

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 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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- Post Status Updates (2pts)
- Rate & Review Speakers, Exhibitors, & Sponsor (3pts)
- 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

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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.

TRAVEL AWARD

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

WIRELESS INTERNET

ICNMD is providing free Wi-Fi throughout the Sheraton Centre. To ensure a positive Wi-Fi experience for all users please do not use your own wireless Hotspot device. These additional Wi-Fi devices create significant RF interference which can affect all Wi-Fi networks. Please turn these devices off, connect to the Wi-Fi network ICNMD2016 and open your web browser to connect to the Internet. **Password: 2016**

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

akashi therapeutics
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.

THURSDAY JULY 7

07:00-8:00

ROOM▸ Grand Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY

TERUMOBCT
Unlocking the Potential of Blood

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

CSL Behring
Biotherapies for Life™

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

idera

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

Alnylam PHARMACEUTICALS

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 wtih 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

PTC THERAPEUTICS
Translating Science • Transforming Lives

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

SATURDAY JULY 9

07:00-8:00

ROOM> Grand Ballroom West

BREAKFAST SYMPOSIUM SUPPORTED BY

ALEXION

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

GRIFOLS

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

santhera Pharmaceuticals

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 Marsollier[1], Lucasz Ciszewski[2], Virginie Mariot[1], Linda Popplewell[2], Thomas Voit[2], George Dickson[2], Julie Dumonceaux[1]; [1]Paris, FR, [2]London, 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 Mendell[1], Nathalie Goemans[2], Louise Rodino-Klapac[1], Z Sahenk[1], Linda Lowes[1], Lindsay Alfano[1], Katherine Berry[1], E Peterson[1], S Lewis[1], K Shontz[1], P Duda[3], C Donoghue[4], J Dworzak[3], B Wentworth[4], E Kaye[4], Eugenio Mercuri[5], DMD Italian Network[6]; [1]Columbus, OH, US, [2]Leuven, BE, [3]Cambridge, US, [4]Cambridge, MA, US, [5]Rome, IT, [6]Milano, 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 Madsen[1], Nicolai Preisler[1], Astrid Emilie Buch[1], Mads Stemmerik[1], Pascal Laforêt[2], John Vissing[1]; [1]Copenhagen, DK, [2]Paris, FR

PS2Group3–004 **SFEMG FINDINGS IN OCULAR COMPLICATIONS OF COSMETIC BOTOX INJECTIONS**
Daniela Navarrete[1], Carlos Navarrete[1], Raul Muñoz[1], Vera Bril[2], Mireya Balart[1]; [1]Santiago, CL, [2]Toronto, ON, CA

SCREEN 4

PS2Group1-068 **THE ANTI-CONVULSANTS LACOSAMIDE, LAMOTRIGINE AND RUFINAMIDE REDUCE MYOTONIA IN ISOLATED HUMAN AND RAT SKELETAL MUSCLE**
Thomas Pedersen[1], Martin Skov[2], Ole Nielsen[2]; [1]Aarhus C, DK, [2]Aarhus, DK

PS2Group1-064 **SUGAR INFUSION IMPROVES EXERCISE CAPACITY IN PATIENTS WITH GLYCOGENIN-1 DEFICIENCY**
Mads Stemmerik[1], Pascal Laforêt[2], Astrid Emilie Buch[1], Karen Madsen[1], John Vissing[1]; [1]Copenhagen, DK, [2]Paris, FR

SCREEN 5

PS2Group1-056 **TREATMENT RELATED EFFECTS OF ANTI-GAA ANTIBODIES IN LATE ONSET POMPE DISEASE**
Marie Wencel, 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

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PS1 Group 2

PS1Group2–001 **STATIN-INDUCED NECROTIZING AUTOIMMUNE MYOPATHY. RECURRENCE WITH FIBRATE USE**
Mario Fuentealba[1], Jorge Bevilacqua[2]; [1]Concepcion, CL, [2]Santiago, CL

PS1Group2–002 **STUDY OF HYALURONIDASE-FACILITATED SCIG IN CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP)**
Claudia Sommer[1], John England[2], Johannes Jakobsen[3], Russell Reeve[4], David Gelmont[5]; [1]Wurzburg, DE, [2]US, [3]Copenhagen, DK, [4]Durham, NC, US, [5]Westlake Village, CA, US

PS1Group2–003 **MECHANISM OF HYALURONIDASE-FACILITATED SCIG ALLOWING INVESTIGATIONS IN NEUROMUSCULAR DISEASE**
Christopher Rabbat[1], Tobin Chettiath[2], Martin Noel[3], Robert Peterman[4], Todd Berner[5]; [1]Kansas, AL, US, [2]Westlake Village, CA, US, [3]Mississauga,ON, CA, [4]Vienna, AT, [5]Bannockburn, US

PS1Group2–004 **RATIONALE FOR TOLL-LIKE RECEPTOR ANTAGONISM AS A POTENTIAL NOVEL THERAPEUTIC APPROACH FOR DERMATOMYOSITIS**
Kirsten Gruis[1], Kanneboyina Nagaraju[2], Tahseen Mozaffar[3], Anthony A. Amato[4], Julie Brevard[1], Lindsey Granlund[1], Joanna Horobin[1]; [1]Cambridge, MA, US, [2]Washington, DC, US, [3]Orange, CA, US, [4]Boston, US

PS1Group2–005 **INFLAMMATORY MYOPATHIES: NEEDLE ELECTROMYOGRAPHY CHARACTERISTCS 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 Thaisetthawatkul[1], Rodney McComb[2]; [1]Omaha, US, [2]Omaha,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 Mendell[1], Z Sahenk[1], Mark Hogan[1], Samiah Al-Zaidy[1], Kevin Flanigan[2], Louise Rodino-Klapac[1], Markus McColly[3], Kathleen Church[1], S Lewis[1], Linda Lowes[1], Lindsay Alfano[1], Katherine Berry[1], Natalie Miller[1], Igor Dvorchik[1], Melissa Moore-Clingenpeel[1], Brian Kaspar[1]; [1]Columbus, OH, US, [2]US, [3]Columbus, US

PS1Group2–010 **WHOLE-BODY MRI IN AMYOPLASIA CONGENITA**
Cam-Tu Emilie Nguyen[1], Sharan Goobie[1], Craig Campbell[2]; [1]London,ON, CA, [2]London, 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 Schaik[1], Vera Bril[2], Nan van Geloven[3], Hans-Peter Hartung[4], Richard Lewis[5], G Sobue[6], Billie Durn[7], John-Philip Lawo[4], Orell Mielke[7], David Cornblath[8], Ingemar Merkies[9], On on behalf of the PATH study group[1]; [1]Amsterdam, NL, [2]Toronto, ON, CA, [3]Leiden, NL, [4]Dusseldorf, DE, [5]Los Angeles, CA, US, [6]Nagoya, JP, [7]Marburg, DE, [8]Baltimore, MD, US, [9]Maastricht, NL

PS1Group4–002 **SWITCHING PATTERNS IN PATIENTS WITH ICD-9 DIAGNOSED CIDP INITIATING IVIG TREATMENT**
Jeffrey Guptil[1], Jeffrey Allen[2], Micheal Runken[3], Josh Noone[4], Emily Zacherle[5], Chris Blanchette[5]; [1]Durham, NC, US, [2]Minneapolis, MN, US, [3]Raleigh, NC, US, [4]Charlotte, NC, US, [5]Davidson, 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 Tirupathi[1], Matthew Sayers[2], John McConville[1], K Pang[1], Marie-Louise Kane[1]; [1]Btba, UK, [2]Bt126ba, UK

PS1Group4–006 **VASCULITIC NEUROPATHY COMPLICATED BY ANTERIOR SPINAL ARTERY SYNDROME**
Michael Ackerl[1], Wolfgang Grisold[2]; [1]Vienna, AT, [2]US

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 Nguyen[1], Craig Campbell[2], Hugh McMillan[3], Chantal Poulin[4], Michel Vanasse[4], Jiri Vajsar[5]; [1]London,ON, CA, [2]London, ON, CA, [3]Ottawa,ON, CA, [4]Montreal, QC, CA, [5]Toronto, 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 Desnuelle[1], Jean Pouget[2], Jean Christophe Antoine[3], Arnaud Lacour[4], Jerome De Seze[5], Christophe Vial[6], Anne-Laure Bedat Millet[7], Julien Cassereau[8], David Adams[9], Guilhem Sole[10], Yann Pereon[11], Philippe Corcia[12], Thibault Moreau[13], Steve Genestet[14], Rabye Ouaja[15], Anne Hufschmitt[15], Chrystelle Mercier[15]; [1]Nice, FR, [2]Marseille, FR, [3]St Etienne, FR, [4]Lille, FR, [5]Strasbourg, FR, [6]Lyon, FR, [7]Rouen, FR, [8]Angers, FR, [9]Le Kremlin Bicetre, FR, [10]Bordeaux, FR, [11]Nantes, FR, [12]Tours, FR, [13]Dijon, FR, [14]Brest, FR, [15]Les 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-Orazio[1], Richard Hughes[2], Isabel Illa[3], Jean Marc Leger[4], S J Ingemar Merkies[5], Luca Padua[6], Rabye Ouaja[7], Witold Malyszczak[7], Sophie Puget[8]; [1]Milan, IT, [2]London, UK, [3]Barcelona, ES, [4]Paris, FR, [5]Hoofddorg, NL, [6]Roma, IT, [7]Les Ulis, FR, [8]Les 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, Hélène 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 Leger[1], Richard Hugues[2], S J Ingemar Merkies[3], Eduardo Nobile-Orazio[4], Rabye Ouaja[5], Witold Malyszczak[5], Sophie Puget[6]; [1]Paris, FR, [2]London, UK, [3]Hoofddorg, NL, [4]Milan, IT, [5]Les Ulis, FR, [6]Les 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 Thaisetthawatkul[1], J Americo Fernandes, Jr[2], Ezequiel Piccione[2], Laetitia Truong[2], P James Dyck[3]; [1]Omaha, US, [2]Omaha,NE, US, [3]Rochester, MN, US

PS1Group4–017 **EGR2 MUTATION ENHANCE PHENOTYPE SPECTRUM OF DEJERINE-SOTTAS SYNDROME**
Elena Gargaun[1], Andreea Seferian[1], Ruxanda Cardas[1], Anne Gaelle Lemoing[1], Juliette Nectoux[1], Catherine Delanoe[1], Gisèle Bonne[1], Anne Boland[1], Jean-Francois Deleuze[2], Cécile Masson[1], Laurent Servais[1], Teresa Gidaro[1]; [1]Paris, FR, [2]Evry, 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 Valdrez, 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**
Pavel Seeman[1], Petra Lassuthova[2], Dana Šafka Brožková[1], Jana Neupauerová[2], Marcela Krůtová[2], Jana Šoukalová[3], Zdeněk Kalina[3], Dagmar Grečmalová[4], Jana Haberlová[1]; [1]Praha ,CZ, [2]Prague ,CZ, [3]Brno ,CZ, [4]Ostrava ,CZ

PS1Group4–021 **PREDICTION OF NERVE CONDUCTION STUDIES OUTCOMES IN PATIENTS WITH FAMILIAL AMYLOIDOTIC POLYNEUROPATHY RECEIVING TAFAMIDIS THERAPY**
Sandra Sousa[1], Katia Valdrez[2], Isabel Fonseca[2], Teresa Coelho[2]; [1]Cascais, PT, [2]Porto, PT

PS1Group4–022 **HEREDITARY MOTOR AND SENSORY NEUROPATHY WITH PYRAMIDAL SIGNS CAUSED BY NEFL GENE MUTATION**
Akihiro Hashiguchi[1], Akiko Yoshimura[1], Yujiro Higuchi[1], Tomonori Nakamura[1], Junhui Yuan[1], Eiji Matsuura[2], Hiroshi Takashima[2]; [1]Kagoshima, JP, [2]Kagoshima City, JP

PS1Group4–023 **ATYPICAL CIDP OR CMT IN THE ELDERLY? A CASE REPORT**
Min-Xia Wang[1], Penny Spring[2], John Pollard[3], Judy Spies[4]; [1]Camperdown,NSW, AU, [2]Concord,AU, [3]Sydney, ACT, AU, [4]Sydney, NSW, AU

PS1Group4–024 **ANTI-GRAVITY AEROBIC TRAINING IN PATIENTS WITH CHARCOT-MARIE-TOOTH DISEASE TYPES 1A AND 1X**
Kirsten Knak[1], Linda Andersen[1], John Vissing[2]; [1]Copenhagen, DK, [2]US

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**
Krish Chandrasekaran[1], Chen Chen[1], Avinash Sagi[2], James Russell[3]; [1]Baltimore, US, [2]Baltimore, MD, US, [3]US

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 BURSITIS**
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 Covaleski[1], Vanessa Mota[1], Otávio Lins[1], Wilson Marques[2]; [1]Recife, BR, [2]Ribeirão Preto, BR

PS1Group4–035 **QUALITY OF LIFE IN PATIENTS WITH DIABETIC PERIPHERAL NEUROPATHY: A LITERATURE REVIEW**
Semra Aciksoz, Ankara, TR

PS1Group4–036 **SEASONAL VARIATION OF BELL'S PALSY: A HOSPITAL BASED RETROSPECTIVE STUDY OVER 9 YEARS**
Byung-Nam Yoon[1], Jung-Joon Sung[2], Suk-Won Ahn[2], Ji-Eun Kim[2], Yoon-Ho Hong[2]; [1]Incheon, KR, [2]Seoul, KR

PS1Group4–037 **THE USE OF OMEGA-3 SUPPLEMENTATION FOR MANAGING DIABETIC NEUROPATHY: RESULTS FROM A CLINICAL PILOT TRIAL**
Evan Lewis[1], Bruce Perkins[2], Richard Bazinet[2], Thomas Wolever[2], Vera Bril[2]; [1]US, [2]Toronto, ON, CA

PS1Group4–038 **B12 DEFICIENCY IS A CAUSE OF REVERSIBLE AUTONOMIC FAILURE: A CASE REPORT**
Pariwat Thaisetthawatkul, Omaha, US

PS1 Group 6

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 Bishop[1], Rexford Newbould[1], Zhengning Lin[2], Robert Janiczek[3], Susanne Wang[2]; [1]London, ON, CA, US, [2]Novato, CA, US, [3]Middlesex, UK

PS1Group6–004 **USEFULNESS OF MRI IN CASES OF HYPERCKEMIA**
Pilar Marti[1], Nuria Muelas[1], Jordi Diaz-Manera[2], Juan J Vilchez[1]; [1]Valencia, ES, [2]Barcelona, ES

PS1Group6–005 **LOWER LIMB MUSCLE VOLUME TEST, RE-TEST VARIABILITY USING MRI**
Hui Jing Yu[1], Thomas Fuerst[2], Randall Stoltz[3], Juan Chavez[4]; [1]Princeton, NJ, US, [2]Newark, CA, US, [3]Indianapolis, IN, US, [4]Cambridge, MA, US

PS1Group6–006 **VITAMIN DEFICIENCIES IN PATIENTS WITH VARIOUS MYOPATHIES AND OTHER NEUROMUSCULAR CONDITIONS-PILOT STUDY**
Dubravka Dodig[1], Mark Tarnopolsky[2]; [1]Toronto, ON, CA, [2]Hamilton, ON, CA

PS1Group6–007 **POTENTIALLY CONFOUNDING VARIABLES OF GDF-15: NEW BIOMARKER OF MITOCHONDRIA DISEASES**
Akiko Ishii[1], Seitaro Nohara[1], Fumiko Yamamoto[1], Shuichi Yatsuga[2], Makoto Terada[1], Tetushi Aizawa[1], Tetsuto Yamaguchi[1], Kumi Yanagiha[1], Tetsuya Moriyama[1], Naoki Touzaka[1], Zenshi Miyake[1], Hiroshi Tsuji[1], Yasushi Tomidokoro[1], Kiyotaka Nakamagoe[1], Kazuhiro ISHII[1], Masahiko Watanabe[1], Yasutoshi Koga[2], Akira Tamaoka[1]; [1]Tsukuba, JP, [2]Kurume, JP

PS1Group6–008 **A NOVEL ASSESSMENT OF BAROREFLEX ACTIVITY BY PHOTOPLETHYSMOGRAPHY AND TERNARY ARITHMETIC CODING IN A RAT MODEL**
An-Bang Liu[1], Hsien-Tsai Wu[2], Chun-Keng Lin[2]; [1]Hualien,TW, [2]Shoufeng,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

PS1Group6–010 **USE OF CORNEAL NERVE FIBRE LENGTH (CNFL) FOR DIABETIC NEUROPATHY IDENTIFICATION IN OLDER PATIENTS WITH LONGSTANDING TYPE 1 DIABETES**
Mohammed Farooqi[1], Leif Lovblom[1], Daniel Scarr[1], Julie Lovshin[1], Yuliya Lytvyn[1], Genevieve Boulet[1], Alanna Weisman[1], Hillary Keenan[2], Michael Brent[1], Narinder Paul[1], Ilia Ostrovski[1], Vera Bril[1], David Cherney[1], Bruce Perkins[1]; [1]Toronto, ON, CA, [2]Boston, 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 Bradbury[1], Amanda Lindy[2], Amy Decker[2], Deborah Copenheaver[2], Sharon Suchy[2]; [1]Olney, MD, US, [2]Gaithersburg, 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 Briand[1], Christian Baudoin[2], Nadira Lagha[2], Pilvi Nigumann[2], Francoise Chapon[3], Tania Stojkovic[1], Christophe Vial[4], Francoise Bouhour[4], Elena Pegoraro[5], Philippe Petiot[4], Antony Behin[1], Bruno Eymard[1], Pascal Laforêt[1], Leonardo Salviati[5], Marc Jeanpierre[1], Michel Vidaud[1], Claude Desnuelle[2], Gael Cristofari[2], Sabrina Sacconi[2]; [1]Paris, FR, [2]Nice, FR, [3]Caen, FR, [4]Lyon, FR, [5]Padova, IT

PS1Group6–01 **ROBUST GENOTYPING IN THE DIAGNOSTICS OF LIMB GIRDLE MUSCULAR DYSTROPHIES**
Baiba Lace[1], Jurgis Strautmanis[2], Ieva Micule[2], Maruta Naudina[2], Loreta Cimbalistiene[3], Algirdas Utkus[3], Birute Burnyte[3], Janis Stavusis[2], Inna Inashkina[2]; [1]Quebec, QC, CA, [2]Riga, LV, [3]Vilnius, LT

PS1Group6–017 **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

PS1Group6–018 **GENETIC SEQUENCING OF PATIENTS WITH LIMB GIRDLE MUSCLE WEAKNESS USING AN NGS PANEL**
Elaine Lee[1], Madhuri Hegde[2], Hillarie Windish[1], Babi Nallamilli[2], Laura Rufibach[1]; [1]Seattle, WA, US, [2]Decatur, 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 Buyse[1], Oscar Mayer[2], R Donisa-Dreghici[3], F Couttet[4], Jodi Wolff[4], Nick Coppard[4]; [1]Leuven, BE, [2]Philadephia,PA, US, [3]Liestal, CH, [4]Liestal, 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 Alfano[1], Natalie Miller[1], Katherine Berry[1], Kevin Flanigan[2], Linda Cripe[1], Jerry Mendell[1], Linda Lowes[1]; [1]Columbus, OH, US, [2]US

PS1Group8–005 **INTRAVENOUS IMMUNOGLOBULIN "WEAR-OFF EFFECT" IN CIDP: STUDY DESIGN AND PROGRESS UPDATE**
Jeffrey Allen[1], Jeffrey Allen[2], Mamatha Pasnoor[3], Ted Burns[4], Senda Ajroud-Driss[2], John Ney[5], Albert Cook[6], Thomas Brannagan, III[7], John Kissel[8], Kenneth Gorson[9], Richard Lewis[10], Melvin Berger[11], Patty Riley[11], David Schaefer[12], Timothy Walton[12]; [1]Minneapolis, MN, US, [2]Chicago,IL, US, [3]Kansas City, KS, US, [4]US, [5]Seattle, WA, US, [6]Johns Creek,GA, US, [7]New York,NY, US, [8]Columbus, OH, US, [9]Boston, MA, US, [10]Los Angeles, CA, US, [11]King Of Prussia,PA, US, [12]Lenexa, KS, US

PS1Group8–006 **IDEBENONE REDUCES RESPIRATORY COMPLICATIONS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
Craig McDonald[1], Thomas Meier[2], Thomas Voit[3], Ulrike Schara[4], Chiara Straathof[5], Maria Grazia D'Angelo[6], Günther Bernert[7], Jean-Marie Cuisset[8], Richard Finkel[9], Nathalie Goemans[10], Christian Rummey[2], Mika Leinonen[2], Paolo Spagnolo[11], Gunnar Buyse[10]; [1]Sacramento, CA, US, [2]Liestal, CH, [3]London, UK, [4]Essen, DE, [5]Za Leiden, NL, [6]Bosiso Parini, IT, [7]Vienna, AT, [8]Lille, FR, [9]Orlando, FL, US, [10]Leuven, BE, [11]Padova, IT

PS1Group8–007 **TREATMENT EFFECT OF IDEBENONE ON INSPIRATORY FUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
Gunnar Buyse[1], Thomas Voit[2], Ulrike Schara[3], Chiara Straathof[4], MARIA GRAZIA D'ANGELO[5], Günther Bernert[6], Jean-Marie Cuisset[7], Richard Finkel[8], Nathalie Goemans[1], Christian Rummey[9], Mika Leinonen[10], Oscar Mayer[11], Paolo Spagnolo[12], Thomas Meier[9], Craig McDonald[13]; [1]Leuven, BE, [2]London, UK, [3]Essen, DE, [4]Za Leiden, NL, [5]Bosiso Parini, IT, [6]Vienna, AT, [7]Lille, FR, [8]Orlando, FL, US, [9]Liestal, CH, [10]Stockholm, SE, [11]Philadephia,PA, US, [12]Padova, IT, [13]Sacramento, CA, US

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 Rocha[1], Jetro Sguarezi[1], Sara Ferreira[1], Rodolfo Thomé[2], Liana Verinaud[2], Catarina Rapôso[2]; [1]Bragança Paulista, BR, [2]Campinas, 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**
Kristin Strandberg[1], Burcu Ayoglu[2], Mojgan Reza[3], Diana Johnsson[4], Joana Pisco Domingos[4], Alison Blain[3], Erik Niks[5], Mathias Uhlén[2], Annemieke Aartsma-Rus[5], Hanns Lochmuller[3], Francesco Muntoni[4], Peter Nilsson[2], Pietro Spitali[5], Cristina Al-Khalili Szigyarto[1]; [1]Stockholm, SE, [2]Solna, SE, [3]Newcastle Upon Tyne, UK, [4]London, UK, [5]Leiden, NL

PS1Group8–012 **SURVEY ON USAGE OF TELECOMMUNICATION TERMINALS IN JAPANESE PATIENTS WITH NEUROMUSCULAR DISEASES**
Katsuhisa Ogata[1], Mikiya Suzuki[1], Kana Yatabe[1], Kazunari Momma[1], Yuzo Tanaka[1], Ikuya Nonaka[1], Takuhisa Tamura[1], Mitsuru Kawai[1], Toshiaki Takahashi[2]; [1]Hasuda, Saitama, JP, [2]Sendai, JP

PS1Group8–013 **IS HOME TREATMENT IN AUTO-IMMUNE DISEASE PATIENTS TREATED BY IVIG SAFE?**
Guilhem Sole[1], Claude Desnuelle[2], Jean-Philippe Azulay[3], Gérard Besson[4], Jean Christophe Antoine[5], Francoise Buhour[6], Alain Creange[7], Gwendal Le Masson[1], Laurent Magy[8], Sebastien Marcel[9], Jean-Michel Paquet[10], Francois Rouhart[11], Rabye Ouaja[12], Marc Gauthier-Darnis[12], Sophie Puget[13]; [1]Bordeaux, FR, [2]Nice, FR, [3]Marseille, FR, [4]Grenoble, FR, [5]St Etienne, FR, [6]Lyon, FR, [7]Creteil, FR, [8]Limoges, FR, [9]Chambery, FR, [10]Laval, FR, [11]Brest, FR, [12]Les Ulis, FR, [13]Les Luis, FR

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**
Emilen Delmont[1], Claude Desnuelle[2], Guilhem Sole[3], Isabelle Durand-Zaleski[4], Marc Gauthier-Darnis[5], Rabye Ouaja[5], Sophie Puget[6]; [1]Marseille, FR, [2]Nice, FR, [3]Bordeaux, FR, [4]Creteil, FR, [5]Les Ulis, FR, [6]Les Luis, FR

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 Bai[1], Alanna Weisman[1], Mohammed Farooqi[1], Leif Lovblom[1], Elise Halpern[1], Genevieve Boulet[1], Devrim Eldelekli[1], Julie Lovshin[1], Yuliya Lytvyn[1], Hillary Keenan[2], Michael Brent[1], Narinder Paul[1], Vera Bril[1], David Cherney[1], Bruce Perkins[1]; [1]Toronto,ON, CA, [4]Boston, US

PS1Group8–016 **ORAL MOTOR COMMUNICATION INVENTORY FOR ALS: CONTENT VALIDATION**
Laura Ball[1], Gary Pattee[2], Jichuan Wang[1]; [1]Washington, DC, US, [2]Lincoln, US

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 Sayın, 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 Roos[1], Sergi Beltran[2], Davide Piscia[2], Steven Laurie[2], Joan Protasio[2], Anastasios Papakonstantinou[2], Andrés Cañada[3], Jose Maria Fernández[3], Mark Thompson[4], Rajaram Kaliyaperumal[4], Séverine Lair[5], Pedro Sernadela[6], Marta Girdea[7], Michael Brudno[7], André Blavier[5], Rachel Thompson[1], Volker Straub[1], Matthew Bellgard[8], Justin Paschall[9], Marco Roos[4], Peter A C 't Hoen[4], Alfonso Valencia[3], David Salgado[10], Christophe Béroud[10], Ivo Glynne Gut[2], Hanns Lochmüller[1]; [1]Newcastle, UK, [2]Barcelona, ES, [3]Madrid, ES, [4]Leiden, NL, [5]Rouen, FR, [6]Aveiro, PT, [7]Toronto,ON, CA, [8]Perth,ACT, AU, [9]Cambridge, UK, [10]Marseille, 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 Kim[1], Dae-Seong Kim[2], Jin-Hong Shin[2], Jin-Sung Park[3], Sa-Yoon Kang[4], Ki-Jong Park[5], Tai-Seung Nam[6], So-Young Huh[7], Jong-kuk Kim[7]; [1]44033, KR, [2]–, KR, [3]Daegu, KR, [4]Jeju, KR, [5]Jinju, KR, [6]Gwangju, KR, [7]Pusan, 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 Tashiro[1], Eiji Matsuura[1], Satoshi Nozuma[1], Akihiro Hashiguchi[2], Osamu Watanabe[2], Hiroshi Takashima[1]; [1]Kagoshima City, JP, [2]Kagoshima, 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 Johnston[1], Christopher MacDonald[1], Jeff Dilworth[2], Hans Katzberg[3], Jean Mah[1], Lawrence Korngut[1]; [1]Calgary, AB, CA, [2]Ottawa, ON, CA, [3]Toronto, ON, CA

PS1Group8–030 **CANADIAN NEUROMUSCULAR DISEASES NETWORK (CAN-NMD) THE DEVELOPMENT & IMPLEMENTATION OF A WEB-BASED KNOWLEDGE SHARING AND EXCHANGE PLATFORM**
Gracia Mabaya[1], Craig Campbell[1], Cynthia Gagnon[2], Megan Johnston[3], Laura McAdam[4], Jeremy Dixon[3], Kelvin Jones[5], Charles Kassardjian[4], Aneal Khan[3], Jane Mitchell[4], Annie Plourde[2], Maryam Oskoui[6], Lawrence Korngut[3]; [1]London, ON, CA, [2]Jonquière, QC, CA, [3]Calgary, AB, CA, [4]Toronto, ON, CA, [5]Edmonton, AB, CA, [6]Montreal, QC, CA

PS1Group8–031 **UNDERSTANDING DECISION NEEDS FOR RESPIRATORY INTERVENTIONS IN PAEDIATRIC NEUROMUSCULAR DISORDERS FROM THE PERSPECTIVE OF HEALTHCARE PROVIDERS**
Gracia Mabaya[1], Sherri Katz[2], Margaret Lawson[2], April Price[1], Dhenuka Radhakrishnan[2], Jean Mah[3], Lawrence Korngut[3], Hugh McMillan[2], Cheryl Scholtes[1], Allyson Shephard[2], Melissa Heletea[2], Craig Campbell[1], Lynda Hoey[2]; [1]London,ON, CA, [2]Ottawa,ON, CA, [3]Calgary, AB, CA

PS1Group8–032 **A PROPOSAL: ISAACS SYNDROME (ACQUIRED NEUROMYOTONIA) DIAGNOSTIC CRITERIA**
Osamu Watanabe[1], Kimiyoshi Arimura[1], Hiroshi Takashima[2]; [1]Kagoshima, JP, [2]Kagoshima 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 Paolantonacci[1], Catherine Decoupade[1], Philippe Appourchaux[1], Catherine Michalski[1], Rabye Ouaja[1], Sophie Puget[2], Ludovic Burlot[1]; [1]Les Ulis, FR, [2]Les 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 DUCHNNE MUSCULAR DYSTROPHY TO DEVELOP A MEANINGFUL PATIENT REPORTED OUTCOME MEASURE**
Roni Propp[1], Sarah Buttle[2], Shannon Weir[1], Clarissa Encisa[1], Aileen Davis[1], Laura McAdam[1], Nancy Salbach[1], Unni Narayanan[1]; [1]Toronto, ON, CA, [2]Ottawa,ON, CA

POSTER SESSION 2 FRIDAY, JULY 8 & SATURDAY, JULY 9, 2016

10:30-12:00

ROOM▶ Exhibit Hall

FRIDAY SESSION SUPPORTED BY

Baxalta

SATURDAY SESSION SUPPORTED BY

Biogen

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 Kimura[1], Madoka Mori-Yoshimura[2], Satomi Mitsuhashi[2], Fumi Takeuchi[3], Harumasa Nakamura[3], Hirohumi Komaki[2], Ichizo Nishino[2], Mitsuru Kawai[4], Shin'ichi Takeda[2]; [1]Kodaira, JP, [2]Tokyo, JP, [3]Kodaira, Tokyo, JP, [4]Hasuda, 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-Yoshimura[1], Satomi Mitsuhashi[1], Hirohumi Komaki[1], Naohiro Yonemoto[2], Harumasa Nakamura[3], Fumi Takeuchi[3], Yukiko Hayashi[1], Miho Murata[1], Ichizo Nishino[1], Shin'ichi Takeda[1], En Kimura[2]; [1]Tokyo, JP, [2]Kodaira, JP, [3]Kodaira, Tokyo, JP

PS2Group1-005 **META-ANALYSES OF ATALUREN IN PATIENTS WITH NONSENSE MUTATION DUCHENNE MUSCULAR DYSTROPHY**
Craig Campbell[1], Francesco Muntoni[2], Eugenio Mercuri[3], Xiaohui Luo[4], Gary Elfring[4], Hans Kroger[5], Peter Riebling[5], Tuyen Ong[5], Robert Spiegel[5], Stuart W Peltz[5], Craig McDonald[6]; [1]London,ON, CA,

[2]London, UK, [3]Rome, IT, [4]South Plainfield, AL, US, [5]South Plainfield, NJ, US, [6]Sacramento, CA, US

PS2Group1-007 **LONGITUDINAL EFFECT OF DRISAPERSEN VERSUS HISTORICAL CONTROLS ON AMBULATION IN DUCHENNE MUSCULAR DYSTROPHY**
Nathalie Goemans[1], Már Tulinius[2], Anna-Karin Kroksmark[2], Marleen Van den Hauwe[1], Zhengning Lin[3], Susanne Wang[3], Giles Campion[4]; [1]Leuven, BE, [2]Gothenburg, SE, [3]Novato, CA, US, [4]Leiden, NL

PS2Group1-008 **IMPACT OF MUSCLE FUNCTION, NUTRITIONAL STATE AND SYSTEMIC INFLAMMATION, ON BONE MINERAL DENSITY IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY**
Oriana Cruz[1], Maricela Rodriguez-Cruz[2], Carlos Wong-Baeza[3], Salvador Atilano-Miguel[2], Tomas Almeida-Becerril[2]; [1]Mexico,MX, [2]D.f,MX, [3]Mexico, 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 Klingels[1], Anna Mayhew[2], Elena Mazzone[3], Michelle Eagle[2], Tina Duong[4], Valérie Decostre[5], Marion Main[6], Marleen Van den Hauwe[1], Ulla Werlauff[7], Imelda De Groot[8], Sonia Messina[9], Valeria Ricotti[6], Giles Campion[10], Laurent Servais[5], Elizabeth Vroom[11], Eugenio Mercuri[3], Nathalie Goemans[1]; [1]Leuven, BE, [2]Newcastle Upon Tyne, UK, [3]Rome, IT, [4]Stanford, US, [5]Paris, FR, [6]London, UK, [7]Aarhus, DK, [8]Nijmegen, NL, [9]Messina, IT, [10]Leiden, NL, [11]Veenendaal, 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 Vlodavets, 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 Vlodavets, 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 Goemans[1], James Signorovitch[2], Elyse Swallow[2], Jinlin Song[2], Susan Ward[3]; [1]Leuven, BE, [2]Boston, MA, US, [3]Cambridge, MA, US

PS2Group1-018 **DIAGNOSIS OF DUCHENNE MUSCULAR DYSTROPHY IN ITALY: CRITICAL ISSUES AND AREAS FOR IMPROVEMENTS**
Adele D'Amico[1], Michela Catteruccia[1], Marika Pane[1], Giovanni Baranello[2], Alessandra Govoni[2], Sonia Messina[3], Maria Grazia D'angelo[4], Luisa Politano[5], Ksenjia Gorni[2], Stefano Carlo Previtali[2], Antonella Pini[6], Roberta Battini[7], Angela Berardinelli[8], Federica Ricci[9], Elena Pegoraro[10], Claudio Bruno[11], Federica Trucco[11], Barbara Panasisi[2], Giuseppe Vita[3], Tiziana Mongini[9], Maurizio Moggio[2], Giacomo Pietro Comi[2], Eugenio Mercuri[1], Enrico Bertini[1]; [1]Rome, IT, [2]Milan, IT, [3]Messina, IT, [4]Bosiso Parini, IT, [5]Naples, IT, [6]Bologne, IT, [7]Pisa, IT, [8]Pavia, IT, [9]Turin, IT, [10]Padua, IT, [11]Genoa, 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 Grynpas, Jane Mitchell; Toronto, ON, CA

PS2Group1-020–TRPV2 INHIBITION THERAPY CAN BE EFFECTIVE FOR CARDIOMYOPATHY OF MUSCULAR DYSTROPHY
Tsuyoshi Matsumura[1], Misa Matsui[1], Yuko Iwata[2], Masanori Asakura[2], Toshio Saito[1], Harutoshi Fujimura[1], Saburo Sakoda[1]; [1]Toyonaka, JP, [2]Suita, JP

PS2Group1-021 **A MISSENSE MUTATION IN THE PUTATIVE SARCOPLASMIC RETICULUM TRANSMEMBRANE PROTEIN DCST2 CAUSES THE STRONGMAN SYNDROME**
Talita Conte[1], Martine Tétreault1[2], Marie-Josée Dicaire[3], Sylvie Provost[4], Najwa Al-Bustani[5], Marie-Pierre Dubé[5], Véronique Bolduc[5], Myriam Srour[6], Erin O'Ferrall[5], Jean-Pierre Bouchard[7], Gina Ravenscroft[8], Russell Hepple[5], Tanja Taivassalo[5], Nigel Laing[9], Phillipa Lamont[10], Jean Mathieu[11], Bernard Brais[5]; [1]2b4, QC, CA, [2]B, QC, CA, [3]B, CA, [4]C, QC, CA, [5]Montreal, QC, CA, [6]Montreal, QC, CA, [7]Quebec, QC, CA, [8]Nedlands,AU, [9]Nedlands, WA, AU, [10]Perth, WA, AU, [11]Jonquiere, QC, CA

PS2Group1-022 **HEREDITARY MYOPATHY WITH EARLY RESPIRATORY FAILURE**
Sandra Sousa[1], Jorge Oliveira[2], Emília Vieira[2], Teresa Coelho[2], Manuela Santos[2], Marcio Cardoso[2], Ricardo Taipa[2], Melo Pires[2], Rosário Santos[2]; [1]Cascais, PT, [2]Porto, PT

PS2Group1-023 **GNE MYOPATHY: MILESTONES AND DISEASE PROGRESSION BASED ON PATIENT SELF-REPORTED DATA COLLECTED THROUGH THE GLOBAL PATIENT REGISTRY**
Oksana Pogoryelova[1], Phillip Cammish[2], Supriya Rao[3], Ed Conner[3], Alison Skrinar[3], Hanns Lochmüller[4]; [1]Newcastle Upon Tyne, UK, [2]Newcastle Upon Tyne, UK, [3]Novato, US, [4]Newcastle, 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-Kumar[1], Oksana Pogoryelova[2], Kiran Polavarapu[1], Narayanappa Gayathri[1], Seena Vengalil[1], Judith Hudson[2], Chandrajit Prasad[1], Hanns Lochmüller[3], Atchayaram Nalini[1]; [1]Bangalore, IN, [2]Newcastle Upon Tyne, UK, [3]Newcastle, UK

PS2Group1-026 **CALPAINOPATHIES IN CHILE**
Jorge Bevilacqua[1], Yves Mathieu[2], Martin Krahn[2], Marc Bartoli[2], Claudia Castiglioni[1], Karin Kleinsteuber[1], Jorge Díaz[1], Francesca Puppo[2], Mathieu Cerino[2], Sebastien Courrier[2], Svetlana Gorokhova[2], Alejandra Trangulao[1], Natalia Miranda[1], Patricio Gonzalez-Hormazabal[1], María De Los Ángeles Avaria[1], J Urtizberea[3], Pablo Caviedes[1], Lilian Jara[1], Nicolas Levy[2]; [1]Santiago, CL, [2]Marseille, FR, [3]Hendaye, 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 Michaud[1], Mathieu Fernandes[1], Eve Duchemin-Pelletier[1], Pauline Poydenot[1], Pauline Menager[2]; [1]Grenoble, FR, [2]Bethesda, 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 Fecchio[1], Marcello Carotti[1], Elisa Bianchini[1], Romeo Betto[1], Roberta Sacchetto[2], Dorianna Sandona[1]; [1]Padova, IT, [2]Legnaro (pd), IT

PS2Group1-030 **CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: ONE-YEAR FOLLOW-UP**
Meredith James[1], Ursula Moore[1], Anna Mayhew[1], Michelle Eagle[1], Karen Bettinson[1], Elena Pegoraro[2], Kate Bushby[1]; [1]Newcastle Upon Tyne, UK, [2]Padova, IT

PS2Group1-031 **HISTOPATHOLOGICAL AND CLINICAL CHARACTERIZATION OF A SPORADIC TNPO3–MUTATED PATIENT**
Alessandra Ruggieri[1], Sara Gibertini[1], Barbara Pasanisi[1], Vincenzo Nigro[2], Marco Savarese[2], Maurizio Moggio[1], Corrado Angelini[3], Renato Mantegazza[1], Lorenzo Maggi[4], Lucia Morandi[1], Marina Mora[1]; [1]Milan, IT, [2]Naples, IT, [3]Venezia Lido, IT, [4]Milano, IT

PS2Group1-032 **ACE-083, A LOCALLY-ACTING MUSCLE AGENT, INCREASES MUSCLE VOLUME IN HEALTHY VOLUNTEERS**
Kenneth Attie[1], Chad Glasser[1], Michael Gartner[2], Brian Boes[2], R Pearsall[1], Xiaosha Zhang[1], Jade Sun[1], Brian Vidal[1], Ashley Bellevue[1], Monty Hankin[1], Matthew Sherman[1]; [1]Cambridge, MA, US, [2]Lincoln,NE, US

PS2Group1-033 **CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: CLINICAL DATA FROM BASELINE ASSESSMENTS**
Elizabeth Harris[1], Ursula Moore[1], Catherine Bladen[1], Anna Mayhew[1], Meredith James[1], Karen Bettinson[1], Heather Hilsden[1], Hillarie Windish[2], The Jain Foundation COS Consortium[1], Kate Bushby[1]; [1]Newcastle Upon Tyne, UK, [2]Seattle, WA, US

PS2Group1-034 **MOLECULAR PATHOGENESIS OF CAVEOLIN-3–RELATED LIMB-GIRDLE-MUSCULAR-DYSTROPHY**
José Andrés González Coraspe[1], Denisa Hathazi[2], Hanns Lochmüller[3], René Zahedi[2], Joachim Weis[1], Andreas Roos[3]; [1]Aachen, DE, [2]Dortmund, DE, [3]Newcastle, UK

PS2Group1-035 **TRIM32 GENE MUTATIONS DETECTED BY NEXT GENERATION SEQUENCING**
Judith Hudson[1], Eileen Graham[1], Chiara Marini Bettolo[1], Teresinha Evangelista[1], Volker Straub[1], Fiona Norwood[2], Kate Bushby[1], Rita Barresi[1]; [1]Newcastle Upon Tyne, UK, [2]London, 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 Farrugia[1], Cheryl Longman[1], William Stewart[1], Volker Straub[2], Richard Petty[1]; [1]Glasgow, UK, [2]Newcastle Upon Tyne, UK

PS2Group1-038 **MOLECULAR DIAGNOSIS OF BETA-SARCOGLYCANOPATHY: REPORTING TWO NOVEL MUTATIONS IN IRAN**
Marzieh Mojbafan[1], Sirous Zeinali[1], Seyed Hasan Tonekaboni[1], Abdolazim Nejati Zadeh[2], Yalda Nili Pour[1]; [1]Tehran, IR, [2]Bandar 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 Cocanougher[1], Pomi Yun[2], Lauren Flynn[3], Mina Jain[2], Melissa Waite[2], Ruhi Vasavada[2], Jason Wittenbach[4], Sabine de Chastonay[5], Sandra Donkervoort[6], A Foley[2], Carsten Bonnemann[2]; [1]Ashburn, US, [2]Bethesda, US, [3]Bethesda, MD, US, [4]Ashburn, VA, US, [5]Torrance, CA, US, [6]1477, MD, US

PS2Group1-042 **P4HA1 MUTATIONS CAUSE A UNIQUE CONGENITAL DISORDER OF CONNECTIVE TISSUE INVOLVING TENDON, BONE, MUSCLE AND THE EYE**
Sandra Donkervoort[1], Yaqun Zou[2], Antti Salo[3], Aileen Barnes[4], Ying Hu[2], A Foley[2], Elena Makareeva[2], Meganne Leach[2], Wendy DiNonno[4], Jahannaz Dastgir[2], Ronald Cohn[5], Sergey Leikin[2], Joan Marini[2], Johanna Myllyharju[3], Carsten Bonnemann[2]; [1]1477, MD, US, [2]Bethesda, MD, US, [3]Oulu, FI, [4]Newport News, VA, US, [5]Toronto, 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'Amico[1], Fabiana Fattori[1], Francesca Gualandi[2], Stefania Petrini[1], Valentina Doria[1], Giorgio Tasca[1], Michela Catteruccia[1], Marcello Niceta[1], Marco Tartaglia[1], Alessandra Ferlini[2], Enrico Bertini[1]; [1]Rome, IT, [2]Ferrara, IT

PS2Group1-044 **A PROSPECTIVE STUDY OF HISTOLOGY, PHENOTYPES AND GENETICS IN CONGENITAL MYOPATHY PATIENTS ABOVE 5 YEARS OF AGE IN DENMARK**
Nanna Witting[1], Ulla Werlauff[2], Morten Duno[1], John Vissing[1]; [1]Copenhagen, DK, [2]Aarhus, DK

PS2Group1-045 **IDENTYFYING 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 Bevilacqua[1], Lidia González-Quereda[2], Ivonne Zamorano[3], Claudia Castiglioni[1], Lorena Acevedo[1], Jorge Díaz[1], María José Rodríguez[2], Alejandra Trangulao[1], Mario Rivera[1], Pia Gallano[2]; [1]Santiago, CL, [2]Barcelona, ES, [3]Puerto 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 Nikolenko[1], Libby Wood[2], Chris Turner[3], David Hilton-Jones[4], Antonio Atalaia[1], Chiara Marini-Bettolo[1], Paul Maddison[5], Margaret Philips[6], Mark Roberts[7], Mark Rogers[8], Volker Straub[1], Simon Hammans[9], Hanns Lochmuller[1]; [1]Newcastle Upon Tyne, UK, [2]Bz, UK, [3]London, UK, [4]Oxford, UK, [5]Nottingham, UK, [6]Derby, UK, [7]Salford, UK, [8]Cardiff, UK, [9]Southampton, 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, Leposava 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 Hamilton[1], Yvonne Robb[2], Helen Gregory[3], Sarah Cumming[1], Berit Adam[1], Josephine McGhie[1], Anneli Cooper[1], Jillian Couto[1], Alexis Duncan[1], Monika Rahman[1], Anne McKeown[1], Alison Wilcox[1], Catherine McWilliam[4], Maria Elena Farrugia[1], Richard Petty[1], Cheryl Longman[1], Iain Findlay[1], Alan Japp[2], Darren Monckton[1], Martin Denvir[2]; [1]Glasgow, UK, [2]Edinburgh, UK, [3]Aberdeen, UK, [4]Dundee, UK

PS2Group1-051 **ONE-YEAR MRI-FOLLOW-UP IN 45 PATIENTS WITH FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY**
Grete Andersen[1], Julia Dahlqvist[1], Christoffer Vissing[1], Karen Pedersen[2], Carsten Thomsen[1], John Vissing[1]; [1]Copenhagen, DK, [2]Copenhagen 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 Hodge[1], Jia Feng[2], Jean Mah[3], Cooperative International Neuromuscular Research Group Investigators[2]; [1]Edmonton, AB, CA, [2]Washington, DC, US, [3]Calgary, AB, CA

PS2Group1-057 **POMPE DISEASE IN AUSTRIA**
Wolfgang Löscher[1], Thomas Stulnig[2], Phillip Simschitz[3], Michaela Brunner-Krainz[4], Martina Huemer[5], Stephan Iglseder[6], Florian Lagler[7], Herman Moser[8], Stefan Quasthoff[4], Julia Wanschitz[1], Wolfgang Grisold[9]; [1]Innsbruck, AT, [2]Vienna, AT, [3]Klagenfurt, AT, [4]Graz, AT, [5]Bregenz, AT, [6]Linz, AT, [7]Salzburg, AT, [8]Altmünster Am Traunsee, AT, [9]US

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 Pena[1], Richard Barohn[2], Barry Byrne[3], Claude Desnuelle[4], Ozlem Goker-Alpan[5], Shafeeq Ladha[6], Pascal Laforêt[7], Eugen Mengel[8], Alan Pestronk[9], Benedikt Schoser[10], Volker Straub[11], Jaya Trivedi[12], Philip Van Damme[13], John Vissing[14], Peter Young[15], Beth Thurberg[16], Raheel Shafi[16], Kerry Culm-Merdek[16], Gerard Short[16], Ans van der Ploeg; for the NE01 Investigator Group[17]; [1]Durham, NC, US, [2]Kansas City, KS, US, [3]Jacksonville, FL, US, [4]Nice, FR, [5]Fairfax, VA, US, [6]Phoenix, AZ, US, [7]Paris, FR, [8]Mainz, DE, [9]St Louis,MO, US, [10]München, DE, [11]Newcastle Upon Tyne, UK, [12]Dallas, TX, US, [13]Leuven, BE, [14]Copenhagen, DK, [15]Münster, DE, [16]Cambridge, MA, US, [17]Rotterdam, NL

PS2Group1-060 **REGULATION OF LIPOPHAGY AND LIPOLYSIS IN LIPID STORAGE MYOPATHIES AND CPT DEFICIENCY**
Corrado Angelini[1], Elisabetta Tasca[2], Anna Nascimbeni[3]; [1]Venezia Lido, IT, [2]Venice, IT, [3]Paris, FR

PS2Group1-062 **ENERGY DEFICIT IN THE MCARDLE MOUSE MODEL AFFECTS CALCIUM HOMEOSTASIS AND FORCE GENERATION**
Thomas Krag[1], Tomas Pinos[2], John Vissing[1]; [1]Copenhagen, DK, [2]Barcelona, ES

PS2Group1-065 **EFFECT OF SENSORY INTEGRATION THERAPY ON FINE MOTOR FUNCTION IN MITOCHONDRIAL MYOPATHY**
Berkan Torpil, Semin Akel, Sedef Şahin; Ankara, TR

PS2Group1-066 **PREVALENCE STUDY OF MUSCLE CHANNELOPATHIES IN ITALY**
Lorenzo Maggi[1], Mauro Lo Monaco[2], Simona Portaro[3], Giovanni Meola[1], Jean Francois Desaphy[4], Sabrina Lucchiari[1], Serena Pagliarani[1], Raffaele Dubbioso[5], Pia Bernasconi[1], Raffaella Brugnoni[1], Paola Imbrici[1], Giacomo Pietro Comi[6], Renato Mantegazza[6], Maria Trojano[4], Adele D'amico[7], Lucio Santoro[5], Elena Pegoraro[8], Luisa Politano[9], Tiziana Mongini[10], Liliana Vercelli[11], Gabriele Siciliano[12], Giulia Ricci[12], Diana Conte-Camerino[4], Antonio Toscano[3], Valeria Sansone[1]; [1]Milano, IT, [2]Roma, IT, [3]Messina, IT, [4]Bari, IT, [5]Napoli, IT, [6]Milan, IT, [7]Rome, IT, [8]Padua, IT, [9]Naples, IT, [10]Turin, IT, [11]Torino, IT, [12]Pisa, 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 Kim[1], Joong-Yang Cho[1], Kyung Seok Park[2]; [1]Goyangsi, KR, [2]Seongnamsi, 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 Kassardjian[1], Cullen O'Gorman[2], Eric Sorenson[3]; [1]Toronto, ON, CA, [2]Brisbane,QLD, AU, [3]Rochester, MN, US

PS2 Group 3

PS2Group3–001 **CLINICAL AND EPIDEMIOLOGICAL FEATURE OF MYASTHENIA GRAVIS IN CHILEAN POPULATION**
Gabriel Cea, David Martinez, Radrigo Salinas, Andres Stuardo; Santiago, CL

PS2Group3–002 **EXOME SEQUENCING IDENTIFIES TARGETS IN OPHTHALMOPLEGIC MYASTHENIA GRAVIS**
Melissa Nel, Mahjoubeh Jalali Sefid Dashti, Junaid Gamieldien, Jeannine Heckmann; Cape Town, ZA

PS2Group3–005 **A PHASE II TRIAL TO ASSESS THE EFFICACY, SAFETY AND FEASIBILITY OF 20% SUBCUTANEOUS IMMUNOGLOBULIN IN PATIENTS WITH MYASTHENIA GRAVIS EXACERBATION–SAFETY AND FEASIBILITY**
Zaeem Siddiqi, Derrick Blackmore, Ashley Mallon; Edmonton, AB, CA

PS2Group3–006 **A PHASE II TRIAL TO ASSESS THE EFFICACY, SAFETY AND FEASIBILITY OF 20% SUBCUTANEOUS IMMUNOGLOBULIN IN PATIENTS WITH MYASTHENIA GRAVIS EXACERBATION–INTERIM ANALYSIS**
Zaeem Siddiqi, Derrick Blackmore, Ashley Mallon, Cecile Phan; Edmonton, AB, CA

PS2Group3–007 **SUBCUTANEOUS IMMUNOGLOBULIN IN MYASTHENIA GRAVIS: TRIAL DESIGN AND PROGRESS UPDATE**
Zaeem Siddiqi, Derrick Blackmore, Ashley Mallon; Edmonton, AB, CA

PS2Group3–008 **DISTAL MYASTHENIA GRAVIS: AN UNUSUAL PRESENTATION WITH SLOWLY PROGRESSIVE DISTAL WEAKNESS AND ATROPHY**
Renata Andrade, Alexandre Januario, João Magalhaes; Recife, BR

PS2Group3–009 **LATE ONSET MYASTHENIA GRAVIS (LOMG): WHEN DOES IT START?**
Nestor Genco, Mariela Bettini, Maria Ines Araoz, Felipe Peralta Calderon, Edgardo Cristiano, Hernan Gonorazky, Marcelo Rugiero; Buenos Aires, AR

PS2Group3–010 **ROLE OF THYMECTOMY IN PREVENTING MYASTHENIC CRISIS IN GENERALIZED MYASTHENIA GRAVIS**
Gillella Pavan, A Meena, R Kumar, S Jabeen, K Rukmini, Rupam Borgohain; Hyderabad, IN

PS2Group3–011 **OCULAR VERSUS GENERALIZED MYASTHENIA GRAVIS: 17 YEARS' EXPERIENCE OF A TURKISH NEUROMUSCULAR CLINIC**
Belgin Mutluay, Aysun Soysal, Fikret Aysal, Ayhan Koksal, Mesude Ozerden, Ayten Dirican, Dİlek Atakli, Sevim Baybas; Istanbul, TR

PS2Group3–012 **MYCOPHENOLATE MOTETILN MYASTHENIA. EXPERIENCES AN ACADEMIC NEUROMUSCULAR CLINIC**
Ratna Bhavaraju-Sanka[1], Mithila Fadia[2], Alejandro Tobon[2], Carlayne Jackson[2]; [1]San Antonio, TX, US, [2]San Antonio, US

PS2Group3–013 **BULBAR MYASTHENIA PRESENTING AS UNILATERAL PALATAL PALSY**
Veena Vasi, Andrew Thompson, Keith Trimble, Sandya Tirupathi; Belfast, UK

PS2Group3–014 **PREPURTAL MYASTHENIA - IS THERE A POSTINFECTIOUS ENTITY?**
Sandya Tirupathi, Clare Loughran; Belfast, UK

PS2Group3–015 **ANTIGEN-SPECIFIC DEPLETION OF PLASMA CELLS IN MYASTHENIA GRAVIS**
Andreas Pelz[1], Qingyu Cheng[1], Adriano Taddeo[2], Andreas Radbruch[1], Andreas Meisel[1], Falk Hiepe[1], Siegfried Kohler[1]; [1]Berlin, DE, [2]Bern, CH

PS2Group3–016 **CLINICAL FOLLOW-UP OF THE PREGNANCY IN MYASTHENIA GRAVIS PATIENTS**
Renata Ducci, Paulo Lorenzoni, Claudia Kay, Lineu Werneck, Rosana Scola; Curitiba, BR

PS2Group3–017 **CHOLINERGIC TRANSMISSION OF OUTER HAIR CELL IMPAIRED IN MYASTHENIA GRAVIS**
Nam-Hee Kim[1], Joong-Yang Cho[1], Kyung Seok Park[2]; [1]Goyangsi, KR, [2]Seongnamsi, KR

PS2Group3–018 **RITUXIMAB FOR THE TREATMENT OF PEDIATRIC AUTOIMMUNE NEUROMUSCULAR DISORDERS**
Cam-Tu Emilie Nguyen[1], Elie Haddad[2], Guy D'Anjou[2], Jean Mathieu[3], Michel Vanasse[2]; [1]London,ON, CA, [2]Montreal, QC, CA, [3]Jonquiere, QC, CA

PS2Group3–019 **EPIDEMIOLOGICAL AND CLINICAL CHARACTERISTICS OF ELDERLY MG**
Jiwon Yang[1], Byung-Nam Yoon[1], Seol-Hee Baek[2], Jung-Joon Sung[2]; [1]Incheon, KR, [2]Seoul, KR

PS2Group3–020 **IMPACT OF REFRACTORY MYASTHENIA GRAVIS ON EMPLOYMENT**
Audra Boscoe[1], Gary Cutter[2], Haichang Xin[2]; [1]Lexington, MA, US, [2]Birmingham, AL, US

PS2Group3–021 **IMPACT OF REFRACTORY MYASTHENIA GRAVIS ON QUALITY OF LIFE**
Audra Boscoe[1], Gary Cutter[2], Haichang Xin[2]; [1]Lexington, MA, US, [2]Birmingham, AL, US

PS2Group3–022 **CLINICAL AND ECONOMIC BURDEN OF REFRACTORY GENERALIZED MYASTHENIA GRAVIS IN THE UNITED STATES**
Nicole Engel-Nitz[1], Audra Boscoe[2], Ryan Wolbeck[1], Jonathan Johnson[1], Nicholas Silvestri[3]; [1]Eden Prairie, MN, US, [2]Lexington, MA, US, [3]US

PS2Group3–023 **MYASTHENIA GRAVIS INDUCED BY A MEK INHIBITOR: FIRST CASE REPORTED**
Majed Majed Alabdali[1], Vera Bril[2], Catherine Maurice[2]; [1]Toronto, ON, CA, [2]Toronto, ON, CA

PS2Group3–024 **INCIDENCE AND PREVALENCE OF MYASTHENIA GRAVIS IN KOREA: A POPULATION-BASED STUDY USING THE NATIONAL HEALTH INSURANCE CLAIMS DATABASE**
Su-yeon Park[1], Jee-Eun Kim[1], Jin Yong Lee[1], Nam Gu Lim[2], Yoon-Ho Hong[1]; [1]Seoul, KR, [2]Daejeon, KR

PS2Group3–025 **CLINICAL CHARACTERISTICS OF MUSK-MG IN KOREA: COMPARISON WITH DOUBLE SERONEGATIVE MG**
Kee Hong Park[1], Jung-Joon Sung[2], Suk-Won Ahn[2], Byung-Nam Yoon[3], Ji-Eun Kim[2], Seol-Hee Baek[2], So Hyun Ahn[2], Yoon-Ho Hong[2]; [1]Jinju, KR, [2]Seoul, KR, [3]Incheon, KR

PS2Group3–026 **LATE-ONSET MYASTHENIA GRAVIS: COMPARISON WITH EARLY-ONSET AND VERY LATE-ONSET MYASTHENIA GRAVIS**
Eun Bin Cho[1], Ju-Hong Min[2], Sujin Lee[3], Cindy W Yoon[3], Hye-Jin Cho[2], Jin Myoung Seok[2], Hye Lim Lee[2], Byoung Joon Kim[2]; [1]Changwon, KR, [2]Seoul, KR, [3]Incheon, KR

PS2Group3–027 **DISEASES OF NEUROMUSCULAR JUNCTION, CLINICAL FEATURES, PATHOPHYSIOLOGY, THERAPY**
Varleen kaur, Patiala, IN

PS2Group3–028 **FETAL AND ADULT ACETYLCHOLINE RECEPTOR FUNCTION IN MYASTHENIC SYNDROMES**
Hakan Cetin, David Beeson, Richard Webster, Angela Vincent; Oxford, UK

PS2Group3–029 **MILD LIMB GIRDLE CONGENITAL MYASTHENIC SYNDROME CAUSED BY HOMOZYGOUS C.686–2A>G MUTATION IN GFPT-1**
Hacer Durmus[1], Serdar Ceylaner[2], Yesim Parman[1], Feza Deymeer[1], Piraye Oflazer-Serdaroglu[1]; [1]Istanbul, TR, [2]Ankara, TR

PS2Group3–030 **CONGENITAL MYASTHENIC SYNDROMES: REPORT ON 8 CASES FROM INDIA**
Atchayaram Nalini[1], Veeramani Preethish-Kumar[1], Kiran Polavarapu[1], Seena Vengalil[1], Xin-Ming Shen[2], Andrew Engel[2]; [1]Bangalore, IN, [2]Minnesota, US

PS2Group3–031 **PERSISTENT DIFFUSE DEEP T WAVE INVERSION: AN ECG MANIFESTATION OF MYASTHENIA GRAVIS IN CRISIS**
Jose Eduardo Duya, Michael Joseph Agbayani; Manila, PH

PS2 Group 5

PS2Group5–001 **PRESERVATION OF MOTOR NEURON EXCITABILITY DURING THE CUTANEOUS SILENT PERIOD IN AMYOTROPHIC LATERAL SCLEROSIS**
Wookyung Kim, Kee Duk Park; Seoul, KR

PS2Group5–002 **PARKINSON DISEASE IS NOT ASSOCIATED WITH C9ORF72 REPEAT EXPANSIONS AND C19ORF12 MUTATIONS IN IRANIAN PATIENTS**
Afagh Alavi, Elahe Elahi, Maryam Malakouti Nejad, Gholamali Shahidi; Tehran, IR

PS2Group5–003 **MULTI-MODALITY, CERVICAL SPINAL CORD MRI IN ALS: A VALIDATION STUDY**
Barry Bedell[1], Thanh Nguyen[1], Vincent Auclair[1], Julien Cohen-Adad[1], Maxime Descoteaux[2], Felix Morency[2], Angela Genge[1], Jim Paskavitz[3], Guillaume Gilbert[1], Donald McLaren[1], Alex Zijdenbos[1]; [1]Montreal, QC, CA, [2]Sherbrooke, QC, CA, [3]Cambridge, MA, US

PS2Group5–004 **IMMUNOHISTOCHEMICAL STUDIES OF VALOCIN-CONTAINING PROTEIN IN THE SKIN OF PATIENTS WITH AMYOTROPHIC LATERAL SCLEROSIS**
Seiitsu Ono, Ichihara, JP

PS2Group5–005 **LOWER MOTOR NEURONE DISEASE WITH BICLONAL IGM-PARAPROTEINAEMIA**
Jochen Schaefer, Heike Kostka, Lisa Klingelhoefer, Karsten Conrad; Dresden, DE

PS2Group5–007 **FUNCTIONAL OUTCOMES IN HEREDITARY SPASTIC PARAPLEGIA: A PROSPECTIVE COHORT STUDY**
Nicolas Chrestian[1], Nicolas Dupré[2], Ziv Gan-or[3], Jean Denis Brisson[2], Anna Szuto[4], Shiyi Chen[4], Kym Boycott[5], Jodi Warman-Chardon[6], Sohnee ahmed[4], Peter ray[4], Oksana Suchowersky[7], Guy Rouleau[3], Grace Yoon[8]; [1]4g2, QC, CA, [2]Z, QC, CA, [3]B, QC, CA, [4]X,ON, CA, [5]L, AB, CA, [6]L,ON, CA, [7]Edmonton, AB, CA, [8]Toronto, ON, CA

PS2Group5–008 **GENE THERAPY FOR SPINAL MUSCULAR ATROPHY TYPE 1 SHOWS POTENTIAL TO IMPROVE SURVIVAL AND MOTOR FUNCTIONAL OUTCOMES**
Samiah Al-Zaidy[1], Richard Shell[1], Doug Sproule[2], William Arnold[1], Louise Rodino-Klapac[1], John Kissel[1], Thomas Prior[1], Carlos Miranda[1], Linda Lowes[1], Lindsay Alfano[1], Katherine Berry[1], Christopher Petek[1], Kathleen Church[1], Lyndsey Braun[1], Jessica Cardenas[3], Sarah Corcoran[1], Kathrin Meyer[1], Shibi Likhite[1], Arthur Burghes[1], Kevin Foust[1], Brian Kaspar[1], Jerry Mendell[1]; [1]Columbus, OH, US, [2]Chicago, US, [3]Dallas, TX, US

PS2 Group 7

PS2Group7–001 **BIOENERGETIC IMPAIRMENT IN CONGENITAL MUSCULAR DYSTROPHY TYPE 1A AND LEIGH SYNDROME**
Cibely Fontes-Oliveira[1], Maarten Steinz[1], Peter Schneiderat[2], Madeleine Durbeej[1]; [1]Lund, SE, [2]Munich, DE

PS2Group7–002 **TRANSFORMING GROWTH FACTOR-Î²1 INHIBITS ADIPOGENESIS IN REGENERATING GLYCEROL-INJURED MUSCLE**
Mohamed Mahdy, Katsuhiko Warita, Yoshinao Hosaka; Tottori, JP

PS2Group7–003 **ROLE OF INTERLEUKIN-6 ON UPREGULATION OF MYOSIN HEAVY CHAIN TYPE IIB MESSENGER RNA IN MOUSE MYOCYTES**
Junko Yamaji[1], Yoshiaki Mori[1], Reiko Hiroshima[1], Ayako Miyazaki[2]; [1]Kashiwara, JP, [2]Nishinomiya, JP

PS2Group7–004 **UPREGULATION OF MYOSIN HEAVY CHAIN TYPE I AND INTERLEUKIN-6 MESSENGER RNA LEVELS BY EXOGENOUS APPLICATION OF CALCINEURIN ACTIVATORS IN C2C12 SKELETAL MYOCYTES**
Yoshiaki Mori[1], Junko Yamaji[1], Reiko Hiroshima[1], Ayako Miyazaki[2]; [1]Kashiwara, JP, [2]Nishinomiya, JP

PS2Group7–005 **CYTOPLASMIC NOTCH AND MEMBRANAL BETA-CATENIN LINK CELL FATE CHOICE TO EMT DURING MYOGENESIS**
Christophe Marcelle, Lyon, FR

PS2Group7–006 **FUNCTIONS OF THE SIL1-BIP CHAPERONE SYSTEM IN MAINTAINING MUSCLE FIBER INTEGRITY**
Andreas Roos[1], Laxmikanth Kollipara[2], Stephan Buchkremer[3], José Andrés González Coraspe[3], Joachim Weis[3], René Zahedi[2]; [1]Newcastle, UK, [2]Dortmund, DE, [3]Aachen, DE

PS2Group7–007 **ANTI-INFLAMMATORY EFFECT OF ORAL-FORMULATED TACROLIMUS IN EXPERIMENTAL AUTOIMMUNE ENCEPHALOMYELITIS (EAE) MICE**
Suk-Won Ahn, Myung-Jin Kim, Dae-Woong Kang, Chang-Seop Kim, Jung-Joon Sung, Yoon-Ho Hong; Seoul, KR

PS2Group7–008 **MUSCLE ADAPTATIONS AND MOTOR DYSFUNCTION IN PERIPHERAL NEURODEGENERATIVE DISEASES MODELS**
Ole Nielsen, Jeppe Bayley, Martin Broch-Lips, Anders Riisager, Thomas Pedersen; Aarhus, DK

PS2Group7–009 **NORMAL MITOCHONDRIAL TRANSPLANTATION MAY BE USEFUL FOR THE TREATMENT OF MITOCHONDRIA-ASSOCIATED NEUROMUSCULAR DISEASES: EVIDENCES IN VITRO**
Xianpeng Jiang, Robert Elliott, Jonathan Head; Baton Rouge, LA, US

DIRECTORY OF EXHIBITORS

EXHIBITORS › **ALPHABETICALLY**

EXHIBITOR	EXHIBIT #
Alexion	110
Baxalta	115-116
Cadwell	108
CSL Behring Canada	113
GBS-CIDP Canadian and International Foundations	TT5
GeneDx	105
Grifols	107
IOS Press	TT4
LFB Biomedicaments	102
LifeLabs	106
Muscular Dystrophy Canada	TT2
Myasthenia Gravis Society of Canada	TT3
Myositis/Myosite Canada	TT3B
PTC Therapeutics	112
RYR-1 Foundation	TT1
Sanofi Genzyme	111
Santhera Pharmaceuticals	117
Terumo BCT	109
TREAT-NMD	TT6
Ultragenyx Pharmaceutical	103

EXHIBITORS › **BY EXHIBIT NUMBER**

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102	LFB Biomedicaments
103	Ultragenyx Pharmaceutical
105	GeneDx
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107	Grifols
108	Cadwell
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111	Sanofi Genzyme
112	PTC Therapeutics
113	CSL Behring Canada
115-116	Baxalta
117	Santhera Pharmaceuticals
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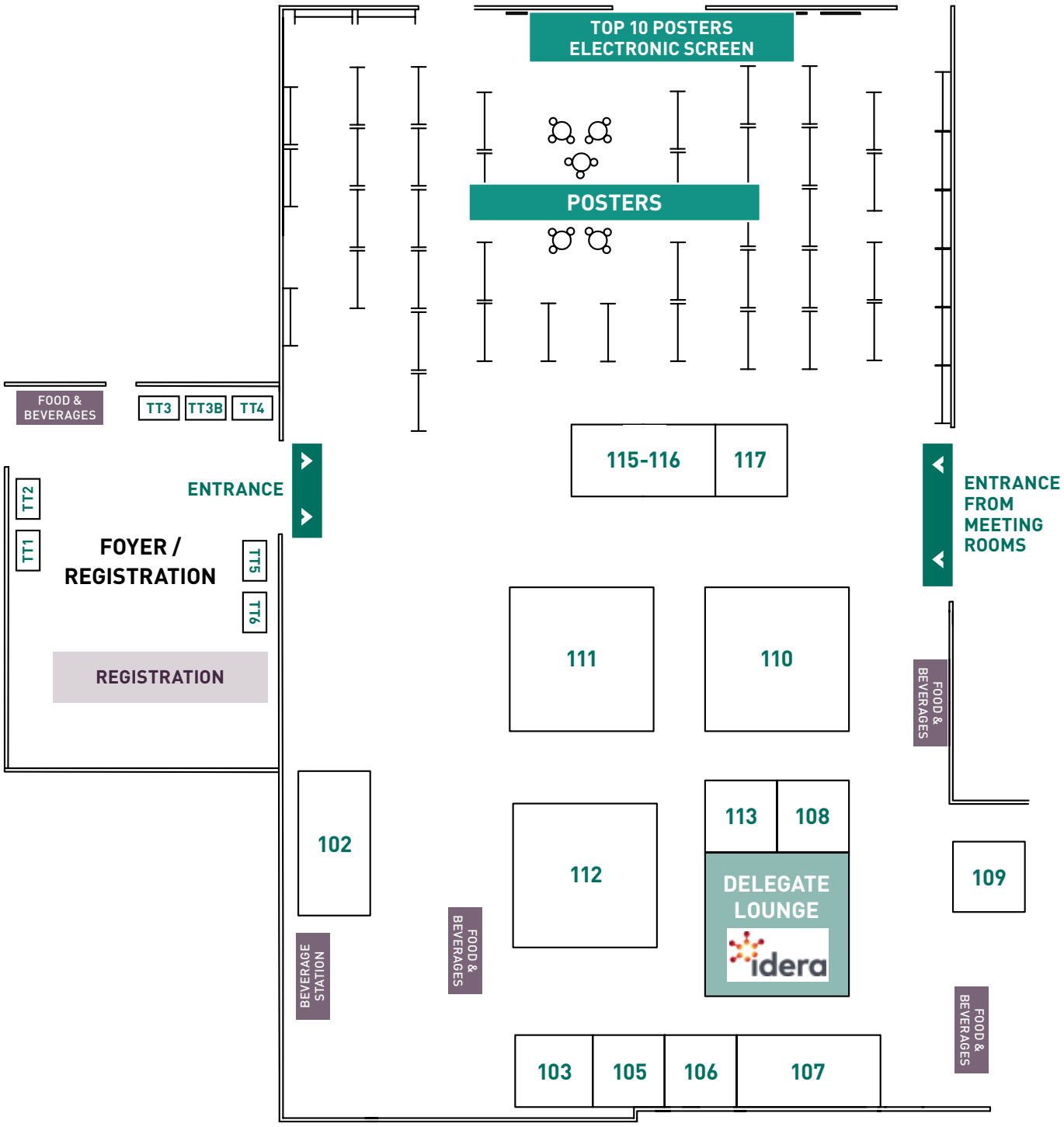
TT2	Muscular Dystrophy Canada
TT3	Myasthenia Gravis Society of Canada
TT3B	Myositis/Myosite Canada
TT4	IOS Press
TT5	GBS-CIDP Canadian and International Foundations
TT6	TREAT-NMD

EXHIBITOR MAP



EXHIBITOR BIOGRAPHIES

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: BXLT) 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 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 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 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)

idera

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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.

SPEAKER AND POSTER PRESENTER INDEX

A

B

C

D

E

F

G

H

I

J

K

L

M

N

O

P

Q

R

S

T

U

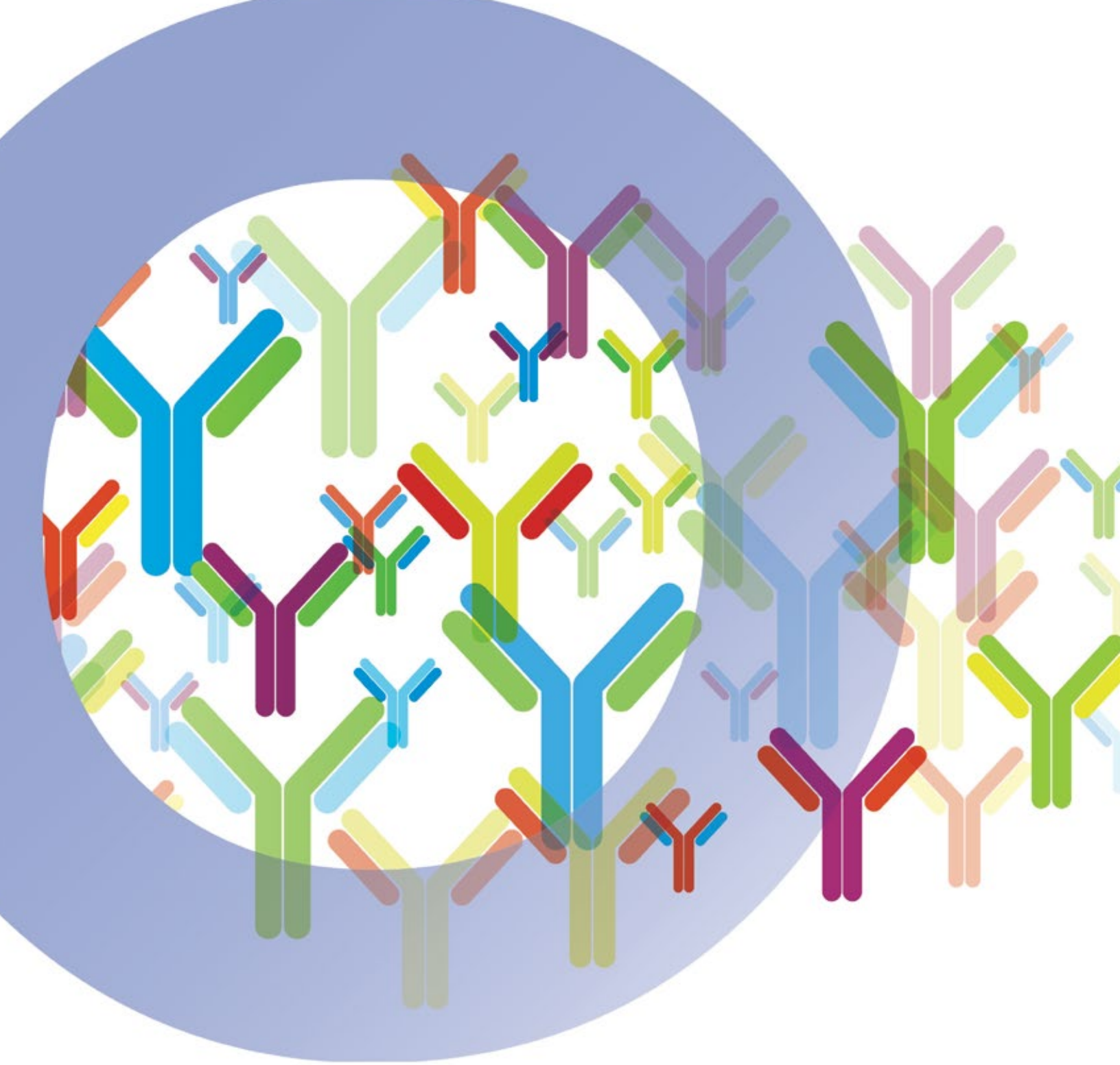
V

W

X

Y

Z



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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 at **ICNMD 2016** and our website: **www.santhera.com**

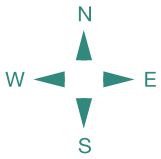
0-D-0021-0416-V1-1 Date of preparation: April 2016.

santhera Pharmaceuticals

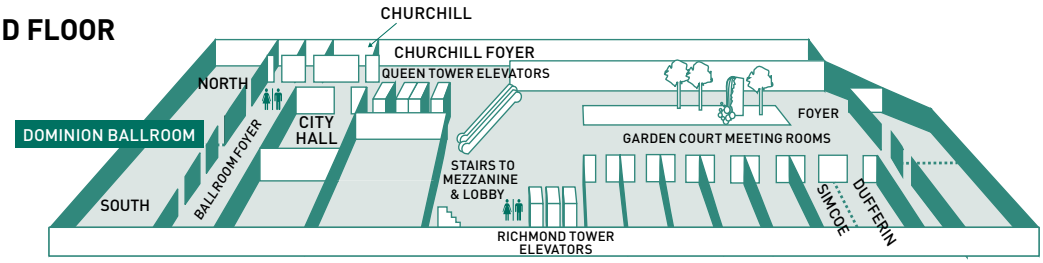
VENUE MAPS

Session Rooms

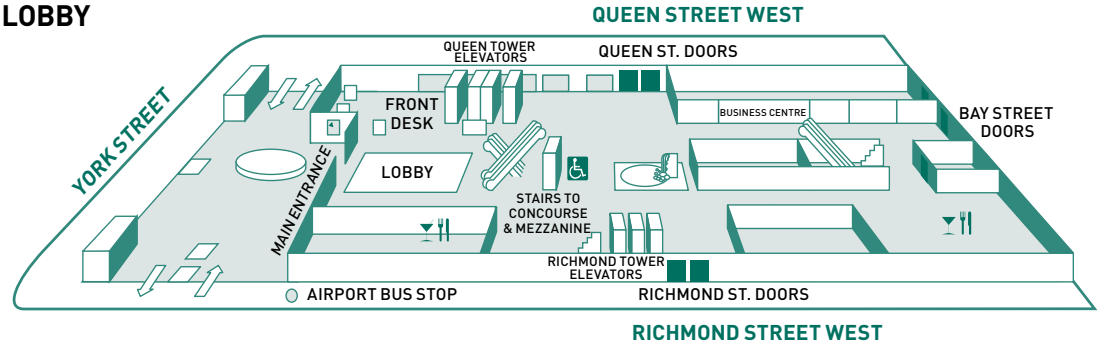
2	CHURCHILL
2	CITY HALL
2	DOMINION BALLROOM
2	DUFFERIN
LC	GRAND BALLROOM
LC	GRAND BALLROOM FOYER
LC	OSGOODE BALLROOM
2	SIMCOE
LC	EXHIBIT HALL
LC	REGISTRATION



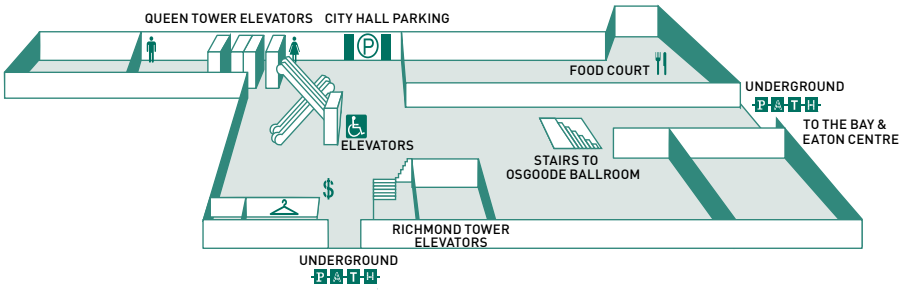
2ND FLOOR



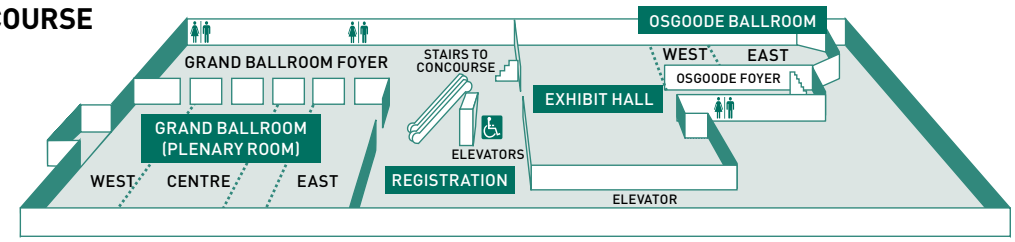
LOBBY



CONCOURSE



LOWER CONCOURSE

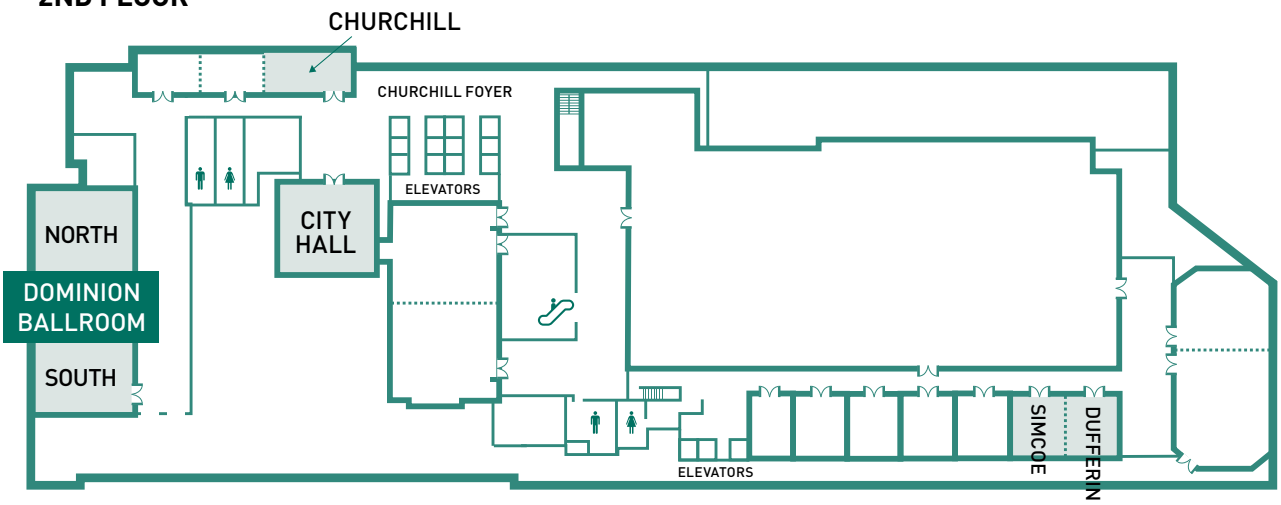


VENUE MAPS

PRE-CONGRESS TEACHING COURSES › TUESDAY JULY 5, 2016

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

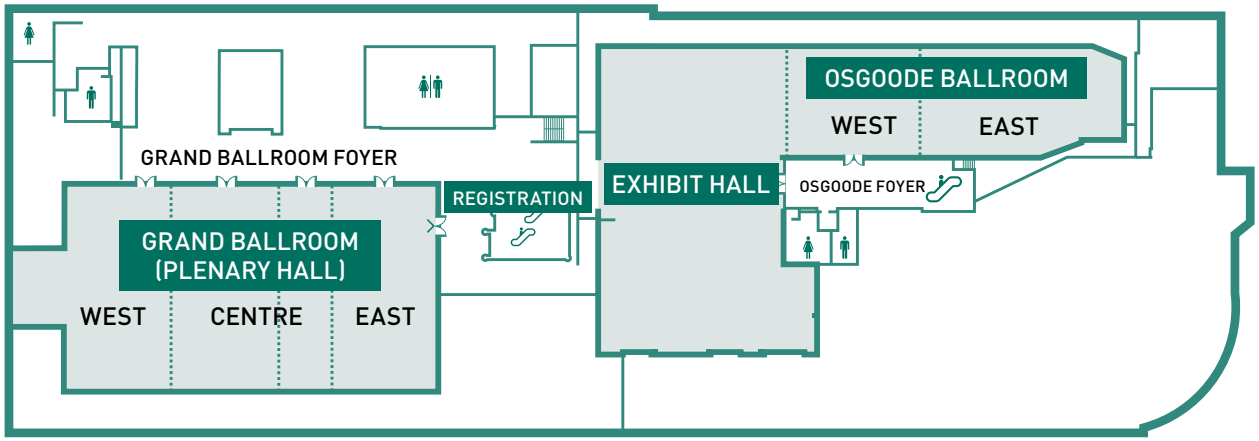
2ND FLOOR



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



SAREPTA
THERAPEUTICS

TRANSFORMATION,
WITHIN REACH™

Sarepta Therapeutics is a biopharmaceutical company focused on developing innovative RNA-targeted therapeutics. Our goal is to harness the power of cutting-edge technology to improve the lives of people affected by serious and life-threatening diseases. Our rapidly advancing clinical programs, including our exon-skipping platform for Duchenne muscular dystrophy, have positioned us on the threshold of realizing the potential of our proprietary RNA-based technology.

DISCOVER SAREPTA.COM

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

7:10 – 7:50 AM
Interactive Workshop

Concluding Remarks
7:50 – 8:00 AM

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.

ALEXION