## GENERAL CONGRESS INFORMATION

**04**

### WEDNESDAY JULY 6, 2016

#### PLENARY 1.0

**CHAIR:** James Dowling, CA

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>08:00 – 09:00</td>
<td>PL 1.1 GENOMIC APPROACHES TO DIAGNOSIS OF RARE MUSCLE DISEASE</td>
<td>Keynote Speaker: Daniel MacArthur, US</td>
</tr>
<tr>
<td>09:00 – 09:30</td>
<td>PL 1.2 GENE DISCOVERY IN CHARCOT-MARIE-TOOTH NEUROPATHIES</td>
<td>Stephan Züchner, US</td>
</tr>
<tr>
<td>09:30 – 10:00</td>
<td>PL 1.3 RNA SEQUENCE AND RNA ANALYSIS</td>
<td>James Dowling, CA</td>
</tr>
</tbody>
</table>

### PLENARY 2.0

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

<table>
<thead>
<tr>
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<tbody>
<tr>
<td>08:00 – 08:15</td>
<td>PL 2.1 STEM CELL THERAPY IN ALS</td>
<td>Eva Feldman, US</td>
</tr>
<tr>
<td>08:15 – 08:30</td>
<td>PL 2.2 RESULTS OF THE THYMECTOMY TRIAL IN MYASTHENIA GRAVIS</td>
<td>Gil Wolfe, US</td>
</tr>
<tr>
<td>08:30 – 08:45</td>
<td>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</td>
<td>James F. Howard, Jr., US</td>
</tr>
<tr>
<td>08:45 – 09:00</td>
<td>PL 2.4 APPROACH TO PATIENT-CENTERED OUTCOMES RESEARCH</td>
<td>Richard Barohn, US</td>
</tr>
<tr>
<td>09:00 – 09:15</td>
<td>PL 2.5 DO WE STILL NEED MUSCLE BIOPSY IN THE ERA OF ULTRASOUND?</td>
<td>Carsten Bonnemann, US</td>
</tr>
<tr>
<td>09:15 – 09:30</td>
<td>PL 2.6 THERAPEUTIC APPROACHES TO INCLUSION BODY MYOSITIS</td>
<td>Mazen Dimachkie, US</td>
</tr>
<tr>
<td>09:30 – 09:45</td>
<td>PL 2.7 TREATMENT OF AMYLOID NEUROPATHY</td>
<td>David Adams, France</td>
</tr>
<tr>
<td>09:45 – 10:00</td>
<td>PANEL DISCUSSION</td>
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</tbody>
</table>

### THURSDAY JULY 7, 2016

#### HOT TOPICS

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

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<tr>
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<tr>
<td>09:45 – 10:00</td>
<td>PANEL DISCUSSION</td>
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</tbody>
</table>

**LOCATION:** All Plenary Sessions will be held in the Plenary Hall, located in the Grand Ballroom Centre, Lower Concourse Level.
### FRIDAY JULY 8, 2016

#### PLENARY 3.0 MUSCULAR DYSTROPHY

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>08:00 – 08:30</td>
<td>PL 3.1 GENE THERAPY FOR MUSCULAR DYSTROPHY</td>
<td>Dongsheng Duan, US</td>
</tr>
<tr>
<td>08:30 – 09:00</td>
<td>PL 3.1 RNA THERAPEUTICS FOR DUCHENNE MUSCULAR DYSTROPHY</td>
<td>Dana Martin, US</td>
</tr>
<tr>
<td>09:00 – 09:30</td>
<td>PL 3.2 ANTISENSE THERAPY FOR MYOTONIC DYSTROPHY</td>
<td>Charles Thornton, US</td>
</tr>
<tr>
<td>09:30 – 10:00</td>
<td>PL 3.3 CRISPR BASED GENE EDITING FOR MUSCULAR DYSTROPHY</td>
<td>Ronald Cohn, CA</td>
</tr>
</tbody>
</table>

### SATURDAY JULY 9, 2016

#### PLENARY 4.0 MOTOR NEURON DISEASE

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
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</thead>
<tbody>
<tr>
<td>08:00 – 09:00</td>
<td>PL 4.1 ALS THERAPY DEVELOPMENT: CHALLENGES AND OPPORTUNITIES</td>
<td>Michael Benatar, US</td>
</tr>
<tr>
<td>09:00 – 09:30</td>
<td>PL 4.2 BIOLOGY OF C9ORF72 DISEASE</td>
<td>Leonard Petrucelli, US</td>
</tr>
<tr>
<td>09:30 – 10:00</td>
<td>PL 4.3 ANTISENSE THERAPY FOR SPINAL MUSCULAR ATROPHY</td>
<td>John Kissel, US</td>
</tr>
</tbody>
</table>
## PROGRAM AT A GLANCE

### REGISTRATION HOURS

<table>
<thead>
<tr>
<th>Day</th>
<th>Time</th>
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</thead>
<tbody>
<tr>
<td><strong>Tuesday, July 5</strong></td>
<td>07:00–20:00</td>
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<tr>
<td><strong>Wednesday, July 6</strong></td>
<td>07:00–17:00</td>
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<tr>
<td><strong>Thursday, July 7</strong></td>
<td>07:00–17:00</td>
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<tr>
<td><strong>Friday, July 8</strong></td>
<td>07:00–19:00</td>
</tr>
<tr>
<td><strong>Saturday, July 9</strong></td>
<td>07:00–17:00</td>
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### EXHIBIT HOURS

<table>
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<td><strong>Friday, July 8</strong></td>
<td>10:00–16:00</td>
</tr>
<tr>
<td><strong>Saturday, July 9</strong></td>
<td>10:00–15:30</td>
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**TUESDAY**

**JULY 5, 2016**

- **Teaching Courses (TC)** (08:00–09:50)
- **Networking Break** (09:50–10:10)
- **Teaching Courses (TC)** (10:10–12:00)
- **Networking Break** (12:00–13:00)
- **Teaching Courses (TC)** (13:00–14:50)
- **Networking Break** (14:50–15:10)
- **Teaching Courses (TC)** (15:10–17:00)

**Opening & Welcome Reception** (18:30–21:00)

**WEDNESDAY**

**JULY 6, 2016**

- **Plenary Session: Genetics** (08:00–10:00)
- **Networking Break** (10:00–10:30)
- **Poster Session 1** (10:30–12:00)
- **Industry-Supported Symposia** (12:00–13:30)
- **Workshop (WS) 1.0** (13:30–15:00)
- **Workshop (WS) 2.0** (15:30–17:00)
- **Industry-Supported Evening Seminars** (17:15–18:15)
Program listings are subject to change.

**THURSDAY**
**JULY 7, 2016**

- **Industry-Supported Seminars**
  - (07:00–08:00)
- **Plenary Session: Hot Topics**
  - (08:00–10:00)
- **Networking Break**
  - (10:00–10:30)
- **Poster Session 1**
  - (10:30–12:00)
- **Networking Break**
  - (11:00–11:30)
- **Free Lunch Time**
  - (12:00–13:30)
- **Workshop (WS) 3.0**
  - (13:30–15:00)
- **Networking Break**
  - (15:00–15:30)
- **Workshop (WS) 4.0**
  - (15:30–17:00)
- **Industry-Supported Evening Seminars**
  - (17:15–18:15)

**FRIDAY**
**JULY 8, 2016**

- **Plenary Session: Muscular Dystrophy**
  - (08:00–10:00)
- **Networking Break**
  - (10:00–10:30)
- **Poster Session 2**
  - (10:30–12:00)
- **Networking Break**
  - (11:00–11:30)
- **Industry-Supported Symposiums**
  - (12:00–13:30)
- **Networking Break**
  - (15:00–15:30)
- **Workshop (WS) 5.0**
  - (13:30–15:00)
- **Workshop (WS) 6.0**
  - (15:30–17:00)
- **Congress Dinner**
  - (18:30–22:00)

**SATURDAY**
**JULY 9, 2016**

- **Plenary Session: Motor Neuron Disease**
  - (08:00–10:00)
- **Networking Break**
  - (10:00–10:30)
- **Poster Session 2**
  - (10:30–12:00)
- **Networking Break**
  - (11:00–11:30)
- **Industry-Supported Symposia**
  - (12:00–13:30)
- **Networking Break**
  - (15:00–15:30)
- **Workshop (WS) 7.0**
  - (13:30–15:00)
- **Workshop (WS) 8.0**
  - (15:30–17:00)
- **Closing Ceremony**
  - (17:00–18:00)
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

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
### 5.5 Practical Demonstrations

<table>
<thead>
<tr>
<th>Time</th>
<th>Description</th>
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<tbody>
<tr>
<td>10:10–10:45</td>
<td><strong>Station 1</strong>&lt;br&gt;Median and ulnar nerves (upper limb)</td>
</tr>
<tr>
<td>10:45–11:20</td>
<td><strong>Station 2</strong>&lt;br&gt;Fibular and tibial nerves (lower limb)</td>
</tr>
<tr>
<td>11:20–11:55</td>
<td><strong>Station 3</strong>&lt;br&gt;Muscle +/- diaphragm</td>
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</tbody>
</table>

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

### Networking Break

<table>
<thead>
<tr>
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<tbody>
<tr>
<td>12:00–13:00</td>
<td><strong>Networking Break</strong></td>
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</table>

### TC 6.0 - Inflammatory Neuropathies

**Location**: City Hall  
**Co-sponsored with PNS**  
**Chairs**: Ingemar Merkies, NL & Ivo van Schaik, NL

<table>
<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>13:00–13:55</td>
<td><strong>6.1 Clinical Aspects of Immune-Mediated Neuropathies</strong>&lt;br&gt;Jean-Marc Léger, FR</td>
</tr>
<tr>
<td>13:55–14:50</td>
<td><strong>6.2 Clinical Metrics of Immune-Mediated Neuropathies</strong>&lt;br&gt;Ingemar Merkies, NL</td>
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<tbody>
<tr>
<td>14:50–15:10</td>
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### TC 7.0 - ALS

**Location**: Churchill  
**Chairs**: Angela Genge, CA & Michael Benatar, US

<table>
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<th>Time</th>
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<tbody>
<tr>
<td>13:00–13:55</td>
<td><strong>7.1 Evaluation of Patients with ALS</strong>&lt;br&gt;Angela Genge, CA</td>
</tr>
<tr>
<td>13:55–14:50</td>
<td><strong>7.2 The Role of Genetic Testing in Motor Neuron Disease</strong>&lt;br&gt;Matthew Harms, US</td>
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### Networking Break

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<tr>
<td>14:50–15:10</td>
<td><strong>Networking Break</strong></td>
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### TC 8.0 - Myopathy

**Location**: Dominion North

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<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>13:00–13:55</td>
<td><strong>8.1 Evaluation of Metabolic Myopathy</strong>&lt;br&gt;Mark Tarnopolsky, CA</td>
</tr>
<tr>
<td>13:55–14:50</td>
<td><strong>8.2 Exercise Therapy in Myopathy</strong>&lt;br&gt;Ronni Haller, US</td>
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### Networking Break

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<tr>
<td>14:50–15:10</td>
<td><strong>Networking Break</strong></td>
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### TC 9.0 - Autonomic Neuropathy

**Location**: Simcoe & Dufferin  
**Chairs**: Eva L. Feldman, US & Paola Sandroni, US

<table>
<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>13:00–13:55</td>
<td><strong>9.1 Introduction to Autonomic Neuropathy</strong>&lt;br&gt;Eva L. Feldman, US</td>
</tr>
<tr>
<td>13:55–14:50</td>
<td><strong>9.2 Assessment of Autonomic Neuropathy</strong>&lt;br&gt;Pariwat Thaisetthawatkul, US</td>
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### Networking Break

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<tr>
<td>14:50–15:10</td>
<td><strong>Networking Break</strong></td>
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</table>

### TC 10.0 - Adult Muscular Dystrophy and Myopathy

**Location**: Dominion South  
**Chairs**: Jiri Vajsar, CA & Mazen Dimachkie, US

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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<tbody>
<tr>
<td>13:00–13:55</td>
<td><strong>10.1 Update on Fascio-Scapulo-Humeral Dystrophy (FSHD)</strong>&lt;br&gt;Rabi Tawil, US</td>
</tr>
<tr>
<td>13:55–14:50</td>
<td><strong>10.2 The Need for Transition</strong>&lt;br&gt;Jiri Vajsar, CA</td>
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</table>

### Networking Break

<table>
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<tbody>
<tr>
<td>14:50–15:10</td>
<td><strong>Networking Break</strong></td>
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### Opening Ceremony & Welcome Reception

**Location**: Plenary Hall, Grand Ballroom Centre and Exhibit Hall, Lower Concourse

<table>
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<tbody>
<tr>
<td>18:30–21:00</td>
<td><strong>Opening Ceremony &amp; Welcome Reception</strong></td>
</tr>
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</table>
**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

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<td>PL 1.1–GENOMIC APPROACHES TO DIAGNOSIS OF RARE MUSCLE DISEASE</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Daniel MacArthur, US</td>
</tr>
<tr>
<td>09:00–09:30</td>
<td>PL 1.2–GENE DISCOVERY IN CHARCOT-MARIE-TOOTH NEUROPATHIES</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Stephan Züchner, US</td>
</tr>
<tr>
<td>09:30–10:00</td>
<td>PL 1.3–RNA SEQUENCE AND RNA ANALYSIS</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>James Dowling, CA</td>
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</table>

### 08:00–10:30  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 1.2

**LOCATION** Exhibit Hall, Lower Concourse

Chair: Hans Katzberg, CA

<table>
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<tbody>
<tr>
<td>13:30–14:15</td>
<td>WS 1.2.1–SYMPTOMATIC TREATMENT OF ALS</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Stacy Rudnick, US</td>
</tr>
<tr>
<td>14:15–15:00</td>
<td>WS 1.2.2–END OF LIFE ISSUES IN ALS</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Christen Shoesmith, US</td>
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</table>

### 13:30–15:00  Workshop WS 1.3

**LOCATION** Exhibit Hall, Lower Concourse

Chair: John Vissing, DE

<table>
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<tr>
<td>13:30–14:15</td>
<td>WS 1.3.1–EVALUATION AND TREATMENT OF POMPE DISEASE</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Mark Tarnopolsky, US</td>
</tr>
<tr>
<td>14:15–15:00</td>
<td>WS 1.3.2–DIETARY AND OTHER THERAPIES IN MUSCLE GLYCOGENOSIS AND DISORDERS OF MUSCLE LIPID OXIDATION</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>John Vissing, US</td>
</tr>
</tbody>
</table>

### 13:30–15:00  Workshop WS 1.4

**LOCATION** Exhibit Hall, Lower Concourse

Chair: Lawrence Korngut, CA

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<td>13:30–14:15</td>
<td>WS 1.4.1–NEUROMUSCULAR DATABASES</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Lawrence Korngut, US</td>
</tr>
<tr>
<td>14:15–15:00</td>
<td>WS 1.4.2–TREAT NMD</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Kevin Flanigan, US</td>
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### 13:30–15:00  Workshop WS 1.1

**LOCATION** Exhibit Hall, Lower Concourse

Chair: Ari Breiner, CA

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<td>13:30–14:15</td>
<td>WS 1.1.1–MUSCLE ULTRASOUND</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Carsten Bonnemann, US</td>
</tr>
<tr>
<td>14:15–15:00</td>
<td>WS 1.1.2–THE APPLICATION OF MRI IN MUSCLE DISEASE</td>
<td>Plenary Hall, Grand Ballroom Centre, Lower Concourse level</td>
<td>Volker Straub, UK</td>
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</tbody>
</table>
13:30–15:00  **Workshop WS 1.5**  
Treatement 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

13:30–17:00  **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

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.

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<td>07:00–17:00</td>
<td><strong>REGISTRATION OPEN</strong></td>
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<tr>
<td>07:00–08:00</td>
<td><strong>INDUSTRY-SUPPORTED SYMPOSIA</strong>  &lt;br&gt;See page 31 for information</td>
</tr>
<tr>
<td>10:00–16:00</td>
<td><strong>EXHIBITS AND POSTERS OPEN</strong>  &lt;br&gt;<strong>LOCATION</strong>  &gt;  Exhibit Hall, Lower Concourse</td>
</tr>
<tr>
<td>08:00–10:00</td>
<td><strong>Plenary Session PL 2.0</strong>  &lt;br&gt;<strong>Hot Topics</strong>  &lt;br&gt;<strong>ROOM</strong>  &gt;  Plenary Hall, Grand Ballroom Centre, Lower Concourse level  &lt;br&gt;Chairs: Vera Bril, CA &amp; Carlos Navarrete Maldonado, CL</td>
</tr>
<tr>
<td>08:00–08:15</td>
<td><strong>PL 2.1–STEM CELL THERAPY IN ALS</strong>  &lt;br&gt;Eva Feldman, US</td>
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<tr>
<td>08:15–08:30</td>
<td><strong>PL 2.2–RESULTS OF THE THYMECTOMY TRIAL IN MYASTHENIA GRAVIS</strong>  &lt;br&gt;Gil Wolfe, US</td>
</tr>
<tr>
<td>08:30–08:45</td>
<td><strong>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</strong>  &lt;br&gt;James Howard, Jr., US</td>
</tr>
<tr>
<td>08:45–09:00</td>
<td><strong>PL 2.4–APPROACH TO PATIENT-CENTERED OUTCOMES RESEARCH</strong>  &lt;br&gt;Richard Barohn, US</td>
</tr>
<tr>
<td>09:00–09:15</td>
<td><strong>PL 2.5–DO WE STILL NEED MUSCLE BIOPSY IN THE ERA OF ULTRASOUND?</strong>  &lt;br&gt;Carsten Bonnemann, US</td>
</tr>
<tr>
<td>09:15–09:30</td>
<td><strong>PL 2.6–THERAPEUTIC APPROACHES TO INCLUSION BODY MYOSITIS</strong>  &lt;br&gt;Mazen Dimachkie, US</td>
</tr>
<tr>
<td>09:30–09:45</td>
<td><strong>PL 2.7–TREATMENT OF AMYLOID NEUROPATHY</strong>  &lt;br&gt;David Adams, FR</td>
</tr>
<tr>
<td>09:45–10:00</td>
<td><strong>PANEL DISCUSSION</strong></td>
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<td>10:00–10:30</td>
<td><strong>NETWORKING BREAK</strong></td>
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<tr>
<td>10:30–12:00</td>
<td><strong>Poster Session 1</strong></td>
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<td><strong>ROOM</strong>  &gt;  Exhibit Hall</td>
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<td>&lt;br&gt;See page 34 for poster information.</td>
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<tr>
<td>12:00–13:30</td>
<td><strong>INDUSTRY-SUPPORTED SYMPOSIUM</strong>  &lt;br&gt;See page 31 for information</td>
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<tr>
<td>13:30–15:00</td>
<td><strong>Workshop WS 3.1</strong></td>
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<td><strong>CHALLENGES IN DESIGN OF INVESTIGATOR-INITIATED RESEARCH</strong>  &lt;br&gt;<strong>ROOM</strong>  &gt;  Osogoode Ballroom West  &lt;br&gt;Chair: Richard Barohn, US</td>
</tr>
<tr>
<td>13:30–14:15</td>
<td><strong>WS 3.1.1–CHALLENGES FOR INVESTIGATOR INITIATED TRIALS AND FOR CONDUCTING MULTICENTER TRIALS</strong>  &lt;br&gt;Richard Barohn, US</td>
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<tr>
<td>13:30–15:00</td>
<td><strong>Workshop WS 3.2</strong></td>
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<tr>
<td></td>
<td><strong>DIAGNOSIS AND TREATMENT OF MYOTONIC DYSTROPHY</strong></td>
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<td><strong>ROOM</strong>  &gt;  Grand Ballroom East</td>
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<td>&lt;br&gt;Chair: Charles Thornton, US</td>
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<tr>
<td>13:30–14:15</td>
<td><strong>WS 3.2.1–HOW TO TREAT MYOTONIC DYSTROPHY</strong>  &lt;br&gt;Charles Thornton, US</td>
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<tr>
<td>14:15–15:00</td>
<td><strong>WS 3.2.2–CURRENT KNOWLEDGE OF DISEASE PROGRESSION IN MYOTONIC DYSTROPHY</strong>  &lt;br&gt;Richard Moxley, III, US</td>
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<td>13:30–15:00</td>
<td><strong>Workshop WS 3.3</strong></td>
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<tr>
<td></td>
<td><strong>MODERN CONCEPTS IN GENETICS</strong></td>
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<td><strong>ROOM</strong>  &gt;  Grand Ballroom Centre</td>
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<tr>
<td></td>
<td>&lt;br&gt;Chair: Kevin Flanigan, US</td>
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<tr>
<td>13:30–14:15</td>
<td><strong>WS 3.3.1–MOLECULAR DIAGNOSTICS IN THE NEUROMUSCULAR CLINIC</strong>  &lt;br&gt;Grace Yoon, CA</td>
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<td>14:15–15:00</td>
<td><strong>WS 3.3.2</strong>  &lt;br&gt;Kevin Flanigan, US</td>
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<td>Time</td>
<td>Workshop</td>
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<tr>
<td>13:30–15:00</td>
<td>Workshop WS 3.4 – Small Fibre Neuropathy</td>
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<tr>
<td>13:30–14:15</td>
<td>WS 3.4.1–AMYLOID NEUROPATHY AS A MODEL OF SMALL FIBER NEUROPATHY</td>
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<tr>
<td>14:15–15:00</td>
<td>WS 3.4.2–DIAGNOSIS OF SMALL FIBRE NEUROPATHY</td>
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<tr>
<td>13:30–15:00</td>
<td>Workshop WS 3.5 – Ultrasound in Peripheral Nerve Disease, Lower Limb</td>
</tr>
<tr>
<td>13:30–15:00</td>
<td>WS 3.5.1–PRACTICAL DEMONSTRATION AND DISCUSSION</td>
</tr>
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<td>Equipment for the Practical Demonstration generously provided by GE Healthcare and Philips Healthcare</td>
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<tr>
<td>15:00–15:30</td>
<td>NETWORKING BREAK</td>
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<tr>
<td>15:30–17:00</td>
<td>Workshop WS 4.1 – Bioinformatics and Clinical Research</td>
</tr>
<tr>
<td>15:30–16:15</td>
<td>WS 4.1.1–TECHNOLOGY PLATFORMS FOR COLLABORATIONS IN CLINICAL RESEARCH</td>
</tr>
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<td>16:15–17:00</td>
<td>WS 4.1.2–USING THE ELECTRONIC MEDICAL RECORD FOR RESEARCH</td>
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<td>15:30–17:00</td>
<td>Workshop WS 4.2 – Controversies Over Large Nerve Biopsy</td>
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<tr>
<td>15:30–16:15</td>
<td>WS 4.2.1–NERVE BIOPSY ARE RARELY NEEDED</td>
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<td>16:15–17:00</td>
<td>WS 4.2.2–IT IS VALUABLE</td>
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<td>15:30–17:00</td>
<td>Workshop WS 4.3 – Genetics of Hereditary Polyneuropathy</td>
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<td>15:30–16:15</td>
<td>WS 4.3.1–OVERVIEW OF GENETICS OF HEREDITARY POLYNEUROPATHY</td>
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<td>16:15–17:00</td>
<td>WS 4.3.2–CELLULAR REPROGRAMMING AND INHERITED PERIPHERAL NEUROPATHIES: PERSPECTIVES AND CHALLENGES</td>
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<td>15:30–17:00</td>
<td>Workshop WS 4.4 – Outcome Measures in Inflammatory Neuropathy</td>
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<td>15:30–15:42</td>
<td>WS 4.4.1–WHAT THE PERINOMS STUDY TAUGHT US</td>
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<td>15:42–15:54</td>
<td>WS 4.4.2–HOW WE SHOULD ASSESS INFLAMMATORY NEUROPATHY</td>
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<td>15:30–17:00</td>
<td>Workshop WS 4.5 – Primer for Genetic Testing</td>
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<td>15:30–16:15</td>
<td>WS 4.5.1–EVALUATION OF VARIANTS OF UNKNOWN SIGNIFICANCE</td>
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<td>16:15–16:25</td>
<td>WS 4.5.2–CLINICAL WHOLE EXOME SEQUENCING</td>
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<td>16:25–16:45</td>
<td>WS 4.5.2–GENE PANELS</td>
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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 GIRDLLE 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
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<th>Details</th>
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| 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 5.6**  
Cramps in Neuromuscular Disease  
**Room**: Grand Ballroom Centre  
Chair: Nicholas Silvestri, US |  |  |
| 15:30–16:15 | **WS 5.6.1–TREATMENT OF MUSCLE CRAMPS**  
Hans Katzberg, CA |  |  |
| 16:15–17:00 | **WS 5.6.2–ASSESSMENT OF MUSCLE CRAMPS**  
Nicholas Silvestri, US |  |  |
| 15:30–17:00 | **Workshop WS 6.3**  
Diabetic Neuropathy  
**Room**: Osgoode Ballroom East  
Chair: James Russell, US |  |  |
| 15:30–16:15 | **WS 6.3.1–EPIDEMIOLOGY AND PATHOPHYSIOLOGY OF DIABETIC NEUROPATHY**  
James Russell, US |  |  |
| 16:15–17:00 | **WS 6.3.2–TREATMENT OF DIABETIC NEUROPATHY**  
Vera Bril, CA |  |  |
| 15:30–17:00 | **Workshop WS 6.4**  
Peripheral Nerve Tumors  
**Room**: Osgoode Ballroom West  
Chair: Wolfgang Grisold, AT |  |  |
| 15:30–16:15 | **WS 6.4.1–LYMPHOMA AND OTHER PERIPHERAL NERVE TUMORS**  
Wolfgang Grisold, AT |  |  |
| 16:15–17:00 | **WS 6.4.2–NEUROFIBROMATOSIS 1 AND MALIGNANT TRANSFORMATION OF PERIPHERAL NERVE SHEATH TUMORS**  
Gelareh Zadeh, US |  |  |
| 15:30–17:00 | **Workshop WS 6.5**  
Ultrasound of Muscle and Nerve  
**Room**: Grand Ballroom West  
Chair: Ari Breiner, CA |  |  |
| 15:30–16:15 | **WS 6.5.1–NEUROMUSCULAR PHYSICIANS SHOULD PERFORM NM ULTRASOUND**  
Steven Shook, US |  |  |
| 16:15–17:00 | **WS 6.5.2–RADIOLOGISTS SHOULD PERFORM NM ULTRASOUND**  
Linda Probyn, US |  |  |
| 17:00–22:00 | **CONGRESS DINNER**  
**Location**: Willow East & Centre located at the Sheraton on the Mezzanine level |  |  |
**CONGRESS PROGRAM ▶ SATURDAY JULY 9, 2016**

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

**07:00–19:00**  REGISTRATION OPEN

**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 SYMPOSUM  
See page 33 for information

**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**

**LOCATION** ▶ 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
### 14th International Congress on Neuromuscular Diseases

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#### SATURDAY | JULY 9, 2016

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<td>13:30–15:00</td>
<td>Workshop WS 7.5 Update on FSHD</td>
<td>Osgoode Ballroom West</td>
<td>Rabi Tawil, US</td>
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<td>13:30–14:15</td>
<td><strong>WS 7.5.1—RECENT CONCEPTS IN FSHD</strong></td>
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<td>Jeffrey Statland, US</td>
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<td>15:00–15:30</td>
<td><strong>NETWORKING BREAK</strong></td>
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<td>15:30–17:00</td>
<td>Workshop WS 8.1 Exercise Therapy for Metabolic Myopathies</td>
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<td>15:30–16:15</td>
<td><strong>WS 8.1.1—EXERCISE THERAPY IN MITOCHONDRIAL DISORDERS</strong></td>
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<td>Ronni Haller, US</td>
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<td>16:15–17:00</td>
<td><strong>WS 8.1.2—EXERCISE TRAINING AND PATHOPHYSIOLOGY OF EXERCISE IN METABOLIC MYOPATHIES</strong></td>
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<td>John Vissing, DE</td>
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<td>15:30–17:00</td>
<td>Workshop WS 8.3 Metabolic Neuropathies</td>
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<td>A. Gordon Smith, US</td>
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<td>15:30–16:15</td>
<td><strong>WS 8.3.1—NEUROPATHY IN PRE-DIABETES &amp; THE METABOLIC SYNDROME</strong></td>
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<td>A. Gordon Smith, US</td>
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<td>16:15–17:00</td>
<td><strong>WS 8.3.2—NEUROPATHY DUE TO SYSTEMIC DISEASE</strong></td>
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<td>15:30–17:00</td>
<td>Workshop WS 8.4 Outcome Measures in Neuromuscular Disorders</td>
<td>Grand Ballroom Centre</td>
<td>Linda Lowes, US</td>
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<td>15:30–16:15</td>
<td><strong>WS 8.4.1—BEST OUTCOME MEASURES TO USE FOR NM PATIENTS</strong></td>
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<td>16:15–17:00</td>
<td><strong>WS 8.4.2—OUTCOME MEASURES IN MUSCULAR DYSTROPHY</strong></td>
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<td>Craig McDonald, US</td>
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<td>15:30–17:00</td>
<td>Workshop WS 8.5 Treatment of Muscular Dystrophy</td>
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<td>15:30–15:42</td>
<td><strong>WS 8.5.1—GENE-DIRECTED TREATMENT OF MUSCULAR DYSTROPHY</strong></td>
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<td>15:42–15:54</td>
<td><strong>WS 8.5.2—NON-GENE DIRECTED</strong></td>
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<td>17:00–19:00</td>
<td><strong>CLOSING CEREMONY</strong></td>
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## 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 Gammapathy/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
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, Lucasz Ciszewski, Virginie Mariot, Linda Popplewell, George Dickson, Julie Dumonceaux; Paris, FR, London, UK

SCREEN 2

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

SCREEN 3

PS2Group1-059  FAT OXIDATION IS LIMITED IN MADD DURING EXERCISE, BUT GLUCOSE INFUSION IMPROVES EXERCISE CAPACITY
Karen Madsen, Nicolai Preissler, Astrid Emilie Buch, Mads Stemmerik, Pascal Laforêt, John Vissing; Copenhagen, DK, Paris, FR

SCREEN 4

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

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 Vengatil, Veeramani Preethish-Kumar, Kiran Polavarapu, Atchayaram Nalin, 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

SANOFI GENZYME

PS1 Group 2

PS1Group2–001 STATIN-INDUCED NECROTIZING AUTOIMMUNE MYOPATHY. RECURRENCE WITH FIBRATE USE
Mario Fuentetblalb, Jorge Bevilacqua2; 1Concepcion, CL, 2Santiago, CL

PS1Group2–002 STUDY OF HYALURONIDASE-FACILITATED SCIG IN CHRONIC INFLAMMATORY DEMYELINATING POLYRADICULONEUROPATHY (CIDP)
Claudia Sommer1, John England2, Johannes Jakesben2, Russell Reeve1; 1Wurzburg, DE, 2US, 3Copenhagen, DK, 4Durham, NC, US, 5Westlake Village, CA, US

PS1Group2–003 MECHANISM OF HYALURONIDASE-FACILITATED SCIG ALLOWING INVESTIGATIONS IN NEUROMUSCULAR DISEASE
Christopher Rabbat1, Tobin Chettiath2, Martin Noel3, Robert Peterman4, Todd Berner5; 1Kansas, AL, US, 2Westlake Village, CA, US, 3Mississauga,ON, CA, 4Vienna, AT, 5Bannockburn, US

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

PS1Group2–005 INFLAMMATORY MYOPATHIES: NEEDLE ELECTROMYOGRAPHY CHARACTERISTICS IN A SERIES OF CASES
Rosana Scola, Paulo Lorenzoni, Claudia Kay, Renata Ducci, Paula Rodrigues, Lineu Wernack; 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 Wernack, Rosana Scola; Curitiba, BR

PS1Group2–007 SRP ANTIBODY ASSOCIATED NECROTIZING MYOPATHY MIMICKED LGMD: A CASE REPORT
Pariwat Thaisetthawatkul1, Rodney McComb2; 1Omaha, US, 2Omaha,NE, US

PS1Group2–008 MYASTHENIA GRAVIS AND POLYMYOSITIS PRESENTED SIMULTANEOUSLY
Florentina Berianu, 22, FL, US

PS1Group2–009 FOLLISTATIN GENE THERAPY IMPROVES SIX MINUTE WALK DISTANCE IN SPORADIC INCLUSION BODY MYOSITIS (SIBM)
Jerry Mendell1, Z Sahenk1, Mark Hogan1, Samiah Al-Zaidy1, Kevin Flanigan2, Louise Rodino-Klapac1, Markus McColly3, Kathleen Church1, S Lewis1, Linda Lowes1, Lindsay Alfano1, Katherine Berry1, Natalie Miller1, Igor Dvorochki1, Melissa Moore-Clingenee1, Brian Kaspar1; 1Columbus, OH, US, 2US, 3Columbus, US

PS1 Group 4

PS1Group4–001 RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY TO INVESTIGATE THE EFFICACY, SAFETY AND TOLERABILITY OF TWO DIFFERENT DOSES OF IGPRO20 (SUBCUTANEOUS IMMUNOGLOBULIN) FOR THE TREATMENT OF CIDP–IGG DEPENDENCY AND RESTABILIZATION PHASE
Ivo van Schaik1, Vera Brit2, Nan van Geloven3, Hans-Peter Hartung4, Richard Lewis5, G Söbbe6, Billie Durn7, John-Philip Lawo1, Orell Mielke7, David Cornblath4, Ingemar Merkies1, On on behalf of the PATH study group1; 1Amsterdam, NL, 2Toronto, ON, CA, 3Leiden, NL, 4Dusseldorf, DE, 5Los Angeles, CA, US, 6Nagoya, JP, 7Marburg, DE, 8Baltimore, MD, US, 9Maastricht, NL

PS1Group4–002 SWITCHING PATTERNS IN PATIENTS WITH ICD-9 DIAGNOSED CIDP INITIATING IVIG TREATMENT
Jeffrey Gupta1, Jeffrey Allen2, Micheal Runken3, Josh Noone4, Emily Zacherle4, Chris Blanchette5, 1Durham, NC, US, 2Minneapolis, MN, US, 3Raleigh, NC, US, 4Charlotte, NC, US, 5Davidson, NC, US
PS1Group4–003  SYSTEMIC LUPUS ERYTHEMATOSUS PRESENTING WITH AUTONOMIC AND SOMATIC SMALL FIBER NEUROPATHY
Oscar Trujillo, Juan Idiaquez, Ricardo Fadic; Santiago, CL

PS1Group4–004  INCIDENCE OF GUILLAIN-BARRE SYNDROME IN IRANIAN CHILDREN UNDER FIFTEEN YEARS OLD; NATIONAL AFP SURVEILLANCE REPORT (2008-2014)
Seyed Hassan Tonekaboni, Habibeh Nejad Biglari; Tehran, IR

PS1Group4–005  POLYNEUROPATHY IN THE LIMELIGHT: A CASE
Sandya Tirupathi, Matthew Sayers, John McConville, K Pang, Marie-Louise Kane; 3Btba, UK, 2B126ba, UK

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

PS1Group4–007  CLINICAL AND ELECTROPHYSIOLOGICAL CHARACTERISTICS OF CHRONIC INFLAMMATORY Demyelinating POLYNEUROPATHY IN KOREA
Seol-Hee Baek, Jun-Soon Kim, BongJe Kim, So Hyun Ahn, Kyomin Choi, Seek-Jin Choi, Jung-Joon Sung, Yoon-Ho Hong; Seoul, KR

PS1Group4–008  NON-TRAUMATIC PLEXOPATHIES AND RADICULOPATHIES IN CHILDREN
Cam-Tu Emilie Nguyen, Craig Campbell, Hugh McMillian, Chantal Poulin, Michel Vanasse, Jiri Vajsar; 1London, ON, CA, 2London, ON, CA, 3Ottawa, ON, CA, 4Montreal, QC, CA, 5Toronto, ON, CA

PS1Group4–009  PERIPHERAL T CELL LYMPHOMA PRESENTING AS MILLER FISHER’S SYNDROME
So Hyun Ahn, Seol-Hee Baek, Jun-Soon Kim, Kyomin Choi, Seek-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, Jean Pouget, Jean Christophe Antoine, Arnaud Lacour, Jerome De Seze, Christophe Vial, Anne-Laure Bedat Millet, Julien Cassereau, David Adams, Guilhem Sole, Yann Pereon, Philippe Corcia, Thibault Moreau, Steve Genestet, Rabye Ouaja, Anne Hufschmitt, Chrystelle Mercier; 1Nice, FR, 2Marseille, FR, 3St Etienne, FR, 4Lille, FR, 5Strasbourg, FR, 6‘Lyons, FR, 7Rouen, FR, 8Angers, FR, 9Le Cremlingo Bicetre, FR, 10Bordeaux, FR, 11Nantes, FR, 12Tours, FR, 13Dijon, FR, 14Brest, FR, 15Les Ulis, FR

PS1Group4–011  AN INTERNATIONAL, MULTICENTRE, EFFICACY AND SAFETY STUDY OF 110E, IQYMUNE IN INITIAL AND MAINTENANCE TREATMENT OF PATIENTS WITH CHRONIC INFLAMMATORY Demyelinating POLYRADICULONEUROPATHY (CIDP)
Eduardo Nobile-Orazio, Richard Hughes, Isabel Illa, Jean Marc Leger, S J Ingemar Merkies, Luca Padua, Rabye Ouaja, Witold Malyszczak, Sophie Puget, Milan, IT, 1London, UK, 2Barcelona, ES, 3Paris, FR, 4Hoofddorg, NL, 5Roma, IT, 6Les Ulis, FR, 7Les 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, Richard Hughes, S J Ingemar Merkies, Eduardo Nobile-Orazio, Rabye Ouaja, Witold Malyszczak, Sophie Puget; 1Paris, FR, 2London, UK, 3Hoofddorg, NL, 4Milan, IT, 5Les Ulis, FR, 6Les Luis, FR

PS1Group4–015  CORRELATION BETWEEN IGM PARAPROTEINEMIA AND MORPHOMETRIC PARAMETERS OF SURAL NERVE IN ANTI-MAG, SGGL NEUROPATHY
Kon Ping Lin, Hua Chuan Chao, Cheng Ta Chou, Yi-Chung Lee; Taipei, TW

PS1Group4–016  INFLAMMATORY DIABETIC NEUROPATHY: HELPFUL DIAGNOSTIC PARAMETERS
PS1Group4–017 EGR2 MUTATION ENHANCE PHENOTYPE SPECTRUM OF DÉJERINE-SOTTAS SYNDROME
Elena Gargaun, Andreae Seferian, Ruxanda Cardas, Anne Gaelle Lemoing, Juliette Nectoux, Catherine Delanoe, Gisèle Bonne, Anne Boland, Jean-François Deleuze, Cécile Masson, Laurent Servais, Teresa Gidaro; ‘Paris, FR, ‘Evry, FR

PS1Group4–018 DEMELINATEING 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, Petra Lassuthova, Dana Šafka Brožková, Jana Neupauerová, Marcela Krůtová, Jana Šoukalová, Zdeněk Kalina, Dagmar Grečmalová, Jana Haberlová; ‘Prague, ‘Ostrava, ‘CZ

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

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

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

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

PS1Group4–030 FREQUENT LABORATORY TESTS ABNORMALITIES IN PERIPHERAL NEUROPATHY
Alon Abraham, Majed Majed Alabdali, Abdulla Alsulaiman, Hana Albulaieh, 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, Myungs-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, Vanessa Mota, Otávio Lins, Wilson Marques; ‘Recife, BR, ‘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 Yoon1, Jung-Joon Sung2, Suk-Won Ahn2, Ji-Eun Kim2, Yoon-Ho Hong2; 1Incheon, KR, 2Seoul, KR

PS1Group4–037 THE USE OF OMEGA-3 SUPPLEMENTATION FOR MANAGING DIABETIC NEUROPATHY: RESULTS FROM A CLINICAL PILOT TRIAL
Evan Lewis1, Bruce Perkins2, Richard Bazinet2, Thomas Wolever2, Vera Bril2; 1US, 2Toronto, ON, CA

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

PS1Group6–002 UNUSUAL CAUSE OF NOCTURNAL HAND PAIN–3 CASES DIAGNOSED BY POCUS AFTER NORMAL EMG
Abraham Chaiton, Toronto, ON, CA

PS1Group6–003 IMPACT OF DRISAPERSEN ON APPARENT FAT FRACTION IN DUCHENNE MUSCULAR DYSTROPHY
Courtney Bishop1, Rexford Newbould1, Zhengning Lin2, Robert Janiczek1, Susanne Wang1; 1London, ON, CA, US, 2Novato, CA, US, 3Middlesex, UK

PS1Group6–004 USEFULNESS OF MRI IN CASES OF HYPERCKEMIA
Pilar Marti1, Nuria Muelas1, Jordi Diaz-Manera2; 1Valencia, ES, 2Barcelona, ES

PS1Group6–005 LOWER LIMB MUSCLE VOLUME TEST, RETEST VARIABILITY USING MRI
Hui Jing Yu1, Thomas Fuerst2, Randall Stoltz1, Juan Chavez1; 1Newark, CA, US, 2Indianapolis, IN, US, 3Cambridge, MA, US

PS1Group6–006 VITAMIN DEFICIENCIES IN PATIENTS WITH VARIOUS MYOPATHIES AND OTHER NEUROMUSCULAR CONDITIONS—PILOT STUDY
Dubravka Dodig1, Mark Tarnopolsky2; 1Toronto, ON, CA, 2Hamilton, ON, CA

PS1Group6–007 POTENTIALLY CONFOUNDING VARIABLES OF GDF-15: NEW BIOMARKER OF MITOCHONDRIA DISEASES
Akiko Ishii1, Seitaro Nohara1, Fumiko Yamamoto1, Shuichi Yatsuga2, Makoto Terada1, Tetsushi Aizawa1, Tetsuto Yamaguchi1, Kumi Yanagihara1, Tetsuya Moriyama1, Naoki Youzaka1, Zenshi Miyake1, Hiroshi Tsuji1, Yasushi Tomidokoro1, Kiyotaka Nakamagoe1, Kazuhiro Ishii1, Masahiko Watanabe1, Yasutoshi Koga2, Akira Tamaoka1; 1Tsukuba, JP, 2Kurume, JP

PS1Group6–008 A NOVEL ASSESSMENT OF BAROREFLEX ACTIVITY BY PHOTOLETHYSMOGRAPHY AND TERNARY ARITHMETIC CODING IN A RAT MODEL
An-Bang Liu1, Hsien-Tsai Wu2, Chun-Keng Lin1; 1Hualien,TW, 2Shoufeng,TW

PS1Group6–009 AGREEMENT BETWEEN AUTOMATED AND MANUAL QUANTIFICATION OF CORNEAL NERVE FIBER LENGTH: IMPLICATIONS FOR DIABETIC NEUROPATHY RESEARCH
Daniel Scarr, Cesar Falappa, Ilia Ostrovski, Leif Lovblom, Mohammed Farooqi, Dylan Kelly, Tong Wu, Elise Halpern, Mylan Ngo, Eduardo Ng, Andrej Orszag, Vera Bril; 1Toronto, ON, CA

PS1Group6–010 USE OF CORNEAL NERVE FIBRE LENGTH (CNFL) FOR DIABETIC NEUROPATHY IDENTIFICATION IN OLDER PATIENTS WITH LONGSTANDING TYPE 1 DIABETES
Mohammed Farooqi1, Leif Lovblom1, Daniel Scarr1, Julie Lovshin1, Yuliya Lytvyn1, Genevieve Boulet1, Alanna Weisman1, Hillary Keenan2, Michael Brent1, Narinder Paul1, Ilia Ostrovski1, Vera Bril1, David Cherney1, Bruce Perkins1; 1Toronto, ON, CA, 2Boston, US

PS1Group6–011 VALIDITY OF AN AUTOMATED PROTOCOL OF IN VIVO CORNEAL CONFOCAL MICROSCOPY FOR DIABETIC SENSORMOTOR 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 SENSORMOTOR 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
PS1 Group 6–013 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

PS1 Group 6–014 HOW MULTI-GENE PANELS CAN CHANGE THE LANDSCAPE OF DIAGNOSING NEUROMUSCULAR DISORDERS
Margaret Bradbury1, Amanda Lindy2, Amy Decker2, Deborah Copenheaver2, Sharon Suchy2, Olney, MD, US, 2Gaithersburg, MD, US

PS1 Group 6–015 SENSITIVITY AND SPECIFICITY OF DR1 BISULFITE SEQUENCING IN DETECTING SMCHD1 MUTATION IN A COHORT OF FSHD1 AND FSHD-LIKE PATIENTS
Audrey Briand1, Christian Baudoin2, Nadira Lagha3, Pilvi Nigumann4, Francoise Chapon5, Tania Stojkovic1, Christophe Vial6, Francoise Bouhour7, Elena Pegraro8, Philippe Petiot9, Antony Behin1, Bruno Eymard1, Pascal Laforté1, Leonardo Salvati9, Marc Jeanpierre1, Michel Vidaud1, Claude Desnuelle9, Gael Cristofari2, Sabrina Sacconi9,1 Paris, FR, 2Nice, FR, 3Caen, FR, 4Lyon, FR, 5Paris, FR, 6Lyon, FR, 7Padova, IT

PS1 Group 6–016 ROBUST GENOTYPING IN THE DIAGNOSTICS OF LIMB GIRDLE MUSCULAR DYSTROPHIES
Baiba Lace1, Jurgis Strautmanis2, Ieva Micule3, Maruta Naudina2, Loreta Cimbalistiene3, Algirdas Uktus3, Birute Burnyte3, Janis Stavusis2, Inna Inashkina2; 1Riga, LV, 2Vilnius, LT

PS1 Group 6–017 PROFLECT: A USER-FRIENDLY TOOL TO DETECT COPY NUMBER VARIATION (CNV) AMONG AMPLICON SEQUENCING DATA
Paco Derouault, Claire-Cécile Barrot, Rémé Moulinas, Franck Sturtz, Stéphane Merillou, Anne-Sophie Lia; Limoges, FR

PS1 Group 6–018 GENETIC SEQUENCING OF PATIENTS WITH LIMB GIRDLE MUSCLE WEAKNESS USING AN NGS PANEL
Elaine Lee1, Madhuri Hegde2, Hillarie Windish1, Babi Nallamilli2, Laura Rufibach1; 1Seattle, WA, US, 2Decatur, US

PS1 Group 8

PS1 Group 8–001 A PHASE III DOUBLE-BLIND, RANDOMIZED, PLACEBO-CONTROLLED STUDY (SIDEROS) ASSESSING THE EFFICACY OF IDEBENONE IN SLOWING THE RATE OF RESPIRATORY FUNCTION LOSS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY RECEIVING GLUCOCORTICOID STEROIDS
Gunnar Buyse1, Oscar Mayer2, R Donisa-Dreghici3, F Couttet4, Jodi Wolff1, Nick Copppard4, Leuven, BE, 2Philadelphia, PA, US, 3Liestal, CH, 4Liestal, SE

PS1 Group 8–003 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

PS1 Group 8–004 RELIABILITY AND VALIDITY OF THE 100 METER TIMED TEST AS AN OUTCOME MEASURE IN DUCHENNE MUSCULAR DYSTROPHY
Lindsay Alfano1, Natalie Miller1, Katherine Berry1, Kevin Flanigan2, Linda Cripe1, Jerry Mendell1, Linda Lowes1; 1Columbus, OH, US, 2US

PS1 Group 8–005 INTRAVENOUS IMMUNOGLOBULIN “WEAR-OFF EFFECT” IN CIDP: STUDY DESIGN AND PROGRESS UPDATE

PS1 Group 8–006 IDEBENONE REDUCES RESPIRATORY COMPLICATIONS IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
Craig McDonald1, Thomas Meier2, Thomas Voit2, Ulrike Schara3, Chiara Straathof9, Maria Grazia D’Angelo6, Günter Bernert2, Jean-Marie Cuisset8, Richard Finkel7, Nathalie Goemans10, Christian Rummey7, Mika Leinonen2, Paolo Spagnolo3, Gunnar Buyse6, 1Sacramento, CA, US, 2Liestal, CH, 3London, UK, 4Essen, DE, 5Za Leiden, NL, 6Bosiso Parini, IT, 7Vienna, AT, 8Lille, FR, 9Orlando, FL, US, 10Leuven, BE, 11Padova, IT
PS1Group8–007 TREATMENT EFFECT OF IDEBENONE ON INSPIRATORY FUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
Gunnar Buyse1, Thomas Voit2, Ulrike Schara3, Chiara Straathof4, MARIA GRAZIA D’ANGELO5, Günther Bernert1, Jean-Marie Cuisset1, Richard Finkel6, Nathalie Goemans5, Christian Rummey3, Mika Leinonen7, Oscar Mayer2, Paolo Spagnolo1, Thomas Meier4, Craig McDonald5, 1Leuven, BE, 2London, UK, 3Essen, DE, 4Za Leiden, NL, 5Bosiso Parini, IT, 6Vienna, AT, 7Lille, FR, 8Orlando, FL, US, 9Liestal, CH, 10Stockholm, SE, 11Philadelphia, PA, US, 12Padova, IT, 13Sacramento, 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 Rocha1, Jetro Sguarezi1, Sara Ferreira1, Rodolfo Thomé2, Liana Verinaud2, Catarina Rapôso2; 1Bragança Paulista, BR, 2Campinas, BR

PS1Group8–010 WHEN SHOULD WE TREAT HYPERCKEMIA?
Astrid Emilie Buch, Karen Pedersen, Sofie Ostergaard, Jesper Thomassen, Ruth Frikke-Schmidt, Nanna Witting, John Vissing; Copenhagen E, DK

PS1Group8–011 VALIDATION OF PROTEIN BIOMARKERS FOR DUCHENNE MUSCULAR DYSTROPHY
Kristin Strandberg1, Burcu Ayoglu2, Mojgan Reza3, Diana Johnsson4, Joana Pisco Domingos4, Alison Blain5, Erik Niks6, Mathias Uhlén2, Annetteke Aartsma-Rus5, Hans Lochmuller1, Francesco Muntoni7, Peter Nilsson1, Pietro Spitali2, Cristina Al-Khalili Szigarto2, 1Stockholm, SE, 2Solna, SE, 3Newcastle Upon Tyne, UK, 4London, UK, 5Leiden, NL

PS1Group8–012 SURVEY ON USAGE OF TELECOMMUNICATION TERMINALS IN JAPANESE PATIENTS WITH NEUROMUSCULAR DISEASES
Katsuhisa Ogata1, Mikiya Suzuki2, Kana Yatabe3, Kazunari Momma4, Yuzo Tanaka4, Ikuya Nonaka5, Takahisa Tamura1, Mitsuhiro Kawai1, Toshiaki Takahashi2, 1Hasuda, Saitama, JP, 2Sendai, JP

PS1Group8–013 IS HOME TREATMENT IN AUTO-IMMUNE DISEASE PATIENTS TREATED BY IVIG SAFE?
Guilhem Sole1, Claude Desnuelle2, Jean-Philippe Azulay3, Gérard Besson1, Jean Christophe Antoine1, Françoise Buhour4, Alain Creange5, Gwendal Le Masson5, Laurent Magy6, Sébastien Marcel7, Jean-Michel Paquet8, François Rouhart1, Rabye Ouaja9, Marc Gauthier-Darnis10, Sophie Puget11, 1Bordeaux, FR, 2Nice, FR, 3Marseille, FR, 4Grenoble, FR, 5St Etienne, FR, 6Lyon, FR, 7Creteil, FR, 8Limoges, FR, 9Chambery, FR, 10Laval, FR, 11Brest, FR, 12Les Ulis, FR, 13Les Louis, 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 Delmont1, Claude Desnuelle2, Guilhem Sole3, Isabelle Durand-Zaleski4, Marc Gauthier-Darnis5, Rabye Ouaja5, Sophie Puget1, 1Marseille, FR, 2Nice, FR, 3Bordeaux, FR, 4Creteil, FR, 5Les Ulis, FR, 6Les Louis, 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 Bai1, Alanna Weisman1, Mohammed Farooqi1, Leif Lovblom1, Elise Halpern1, Genevieve Boulet1, Devrim Eldelekli2, Julie Lovshin1, Yuliya Lytvyn1, Hillary Keenan2, Michael Brent1, Narinder Paul1, Vera Bril1, David Cherney1, Bruce Perkins1; 1Toronto, ON, CA, 2Boston, US

PS1Group8–016 ORAL MOTOR COMMUNICATION INVENTORY FOR ALS: CONTENT VALIDATION
Laura Ball1, Gary Pattee2, Jichuan Wang1; 1Washington, DC, US, 2Lincoln, 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 Sayin1, Coskun Ozdemir1; Istanbul, 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  REFRATORY POSTURAL ORTHOSTATIC TACHYCARDIA SYNDROME: EFFICACY AND SAFETY OF WEEKLY ALBUMIN INFUSIONS
Zaeem Siddiqi, Aimee Soloway, Derrick Blackmore; Edmonton, AB, CA

PS1Group8–021  RD-CONNECT: DATA SHARING AND ANALYSIS FOR RARE DISEASE RESEARCH WITHIN THE INTEGRATED PLATFORM AND THROUGH GA4GH BEACON AND MATCHMAKER EXCHANGE
Andreas Roos1, Sergio Beltran2, Davide Piscia2, Steven Laurie2, Joan Protasio2, Anastasios Papakonstantinou2, Andrés Cañada2, Jose Maria Fernández2, Mark Thompson1, Rajaram Kaliyaperumal2, Séverine Lairy2, Pedro Sernadela4, Marta Girdea2, Michael Brudno2, André Blavier3, Rachel Thompson2, Volker Straub2, Matthew Bellgard3, Justin Paschall3, Marco Roos2, Peter A C’t Hoent3, Alfonso Valencia1, David Salgado26, Christophe Béroud26, Ivo Glynné Gut26, Hanns Lochmüller26; ’Newcastle, UK, 2Barcelona, ES, 3Madrid, ES, 4Leiden, NL, 5Rouen, FR, 6Aveiro, PT, 7Toronto,ON, CA, 8Perth,ACT, AU, 9Cambridge, UK, 10Marseille, FR

PS1Group8–022  CASE REPORT OF RECURRENT MENINGITIS SECONDARY TO CSF RHINORRHEA
Aleena Soomro, Karachi, PK

PS1Group8–023  CLINICAL PRESENTATION OF ANTI-NMDA ENCEPHALITIS
Rabail Karim, Bashir Soomro; Karachi, PK

PS1Group8–024  NUDT15 VARIANT IS THE MOST COMMON VARIANT ASSOCIATED WITH THIOPURINE-INDUCED EARLY LEUKOPENIA AND ALOPECIA IN KOREAN PATIENTS WITH VARIOUS NEUROLOGICAL DISEASES

PS1Group8–025  DATABASE OF NEUROMUSCULAR DISEASES IN REGION OF CONCEPCION IN CHILE
Mario Fuentenalba, Concepcion, CL

PS1Group8–026  SEROPREVALENCE OF HUMAN T-LYMPHOTROPIC VIRUS TYPE 1 IN PATIENTS WITH SURGICAL HISTORY IN KAGOSHIMA, SOUTHERN JAPAN
Yuichi Tashiro1, Eiji Matsuura1, Satoshi Nozuma1, Akihiro Hashiguchi1, Osamu Watanabe2, Hiroshi Takashima1; ’Kagoshima City, JP, 2Kagoshima, JP

PS1Group8–027  HIGH RISK BREAST CANCER SCREENING IN WOMEN WITH NEUROFIBROMATOSIS TYPE 1
Jeanna McCuaig, Shelley Westergard, Catherine Maurice, Paul Kongkham, Galareh Zadeh, Carolina Barnett-Tapia, Vera Britl, Raymond Kim; Toronto, ON, CA

PS1Group8–029  UNDERSTANDING THE CANADIAN NEUROMUSCULAR DISEASE RESEARCH LANDSCAPE
Megan Johnston1, Christopher MacDonald1, Jeff Dilworth1, Hans Katzberg1, Jean Mah1, Lawrence Kornngut1; ’Calgary, AB, CA, 2Ottawa, ON, CA, 3Toronto, ON, CA

PS1Group8–030  CANADIAN NEUROMUSCULAR DISEASES NETWORK (CAN-NMD) THE DEVELOPMENT & IMPLEMENTATION OF A WEB-BASED KNOWLEDGE SHARING AND EXCHANGE PLATFORM
Gracia Mabay1, Craig Campbell1, Cynthia Gagnon1, Megan Johnston2, Laura McAdam3, Jeremy Dixon4, Kelvin Jones3, Charles Kassardjian5, Aneal Khan1, Jane Mitchell1, Annie Plourde1, Maryam Oscou1, Lawrence Kornngut1; ’London, ON, CA, 2Jonquière, QC, CA, 3Calgary, AB, CA, 4Toronto, ON, CA, 5Edmonton, AB, CA, 6Montreal, QC, CA

PS1Group8–031  UNDERSTANDING DECISION NEEDS FOR RESPIRATORY INTERVENTIONS IN PAEDIATRIC NEUROMUSCULAR DISORDERS FROM THE PERSPECTIVE OF HEALTHCARE PROVIDERS
Gracia Mbay1, Sherri Katz2, Margaret Lawson3, April Price4, Dhenuka Radhakrishnan5, Jean Mah1, Lawrence Kornngut1, Hugh McMillan2, Cheryl Schoites1, Allyson Shephard2, Melissa Heleeta1, Craig Campbell1, Lynda Hoey3; ’London,ON, CA, ’Ottawa,ON, CA, ’Calgary, AB, CA

PS1Group8–032  A PROPOSAL: ISAACS SYNDROME (ACQUIRED NEUROMYOTONIA) DIAGNOSTIC CRITERIA
Osamu Watanabe1, Kimiyoshi Arimura1, Hiroshi Takashima2; ’Kagoshima, JP, 2Kagoshima City, JP

PS1Group8–033  UNDERSTANDING THE PERSPECTIVES OF YOUNG ADULTS WITH DUCHENNE MUSCULAR DYSTROPHY AS THEY TRANSITION TO ADULTHOOD AND ADULT HEALTH CARE
Sally Lindsay, Laura McAdam, Tania Mahenderin; Toronto, ON, CA
PS1Group8–034  CHARACTERISTICS DEVELOPMENT OF A NEW IVIG [I10] THE QUALITY BY DESIGN APPROACH (QBD)
Philippe Paolantonacci, Catherine Decoupade, Philippe Appourchaux, Catherine Michalski, Rabye Ouaja, Ousmane Alfa Cisse, Ludovic Burlot; Les Ulis, FR

PS1Group8–035  PURIFICATION OF IVIG INTRAVENOUS IMMUNOGLOBULIN (IQYMUNE® 100 MG ML, SOLUTION FOR INFUSION)
Philippe Paolantonacci1, Catherine Decoupade1, Philippe Appourchaux1, Catherine Michalski1, Rabye Ouaja1, Sophie Puget1, Ludovic Burlot1; 1Les Ulis, FR, 2Les Luis, FR

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

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

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

PS2 Group 1

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

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

PS2Group1-003  CURRENT STATUS OF DYSTROPHINOPATHY NATIONAL REGISTRY IN JAPAN
En Kimura1, Madoka Mori-Yoshimura2, Satomi Mitsushashi3, Fumi Takeuchi3, Harumasa Nakamura3, Hirohumi Komaki2, Ichizo Nishino2, Mitsuhiro Kawai2, Shin’ichi Takeda3; 1Kodaira, JP, 2Tokyo, JP, 3Kodaira, Tokyo, JP, 4Hasuda, Saitama, JP

PS2Group1-004  CHARACTERISTICS OF JAPANESE PATIENTS WITH BECKER MUSCULAR DYSTROPHY IN A JAPANESE NATIONAL REGISTRY OF MUSCULAR DYSTROPHY (REMUDY): HETEROGENEITY AND CLINICAL VARIATION
Madoka Mori-Yoshimura1, Satomi Mitsushashi1, Hirohumi Komaki2, Naohiro Yonemoto2, Harumasa Nakamura3, Fumi Takeuchi3, Yukiko Hayashi3, Miho Murata2, Ichizo Nishino2, Shin’ichi Takeda2, En Kimura3; 1Tokyo, JP, 2Kodaira, JP, 3Kodaira, Tokyo, JP

PS2Group1-005  META-ANALYSES OF ATALUREN IN PATIENTS WITH NONSENSE MUTATION DUCHENNE MUSCULAR DYSTROPHY
Craig Campbell1, Francesco Muntoni2, Eugenio Mercuri2, Xiaohui Luo4, Gary Elfing1, Hans Kroger1, Peter Riebling2, Tuyen Ong2, Robert Spiegel2, Stuart W Peltz2, Craig McDonald6; 1London,ON, CA,
PS2Group1-007  LONGITUDINAL EFFECT OF DRISAPERSEN VERSUS HISTORICAL
 CONTROLS ON AMBULATION IN DUCHENNE MUSCULAR DYSTROPHY
 Nathalie Goemans1, Már Tulinius2, Anna-Karin Kroksmark2, Marleen Van den Hauwe1, Zhengning Lin3, Susanne Wang3, Giles Campion4; 1Leuven, BE, 2Gothenburg, SE, 3Novato, CA, US, 4Leiden, NL

PS2Group1-008  IMPACT OF MUSCLE FUNCTION, NUTRITIONAL STATE AND SYSTEMIC INFLAMMATION, ON BONE MINERAL DENSITY IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
 Oriana Cruz1, Maricela Rodriguez-Cruz2, Carlos Wong-Baeza3, Salvador Atliano-Miguel2, Tomas Almeida-Becerril2; 1Mexico,MX, 2D.f,MX, 3Mexico, D.f,MX

PS2Group1-009  MIBG THERAPY FOR AN INOPERABLE PARAGANGLIOMA IN DUCHENNE MUSCULAR DYSTROPHY
 Denis Duboc, Marine Paul, Laurie Fanon, Karim Wahbi, Marco Alifano, Florence Tenenbaum, Laurence Guignat; Paris, FR

PS2Group1-010  EFFECT OF METFORMIN ON IN VIVO AND EX VIVO PATHOLOGY SIGNS IN EXERCISED DYSTROPHIC MDX MICE
 Roberta Capogrosso, Anna Cozzoli, Arcangela Giustino, Paola Mantuano, Francesca Sanarica, Michela De Bellis, Annamaria De Luca; Bari, IT

PS2Group1-011  DEVELOPMENT OF A PATIENT-REPORTED OUTCOME MEASURE FOR UPPER LIMB FUNCTION IN DUCHENNE MUSCULAR DYSTROPHY (DMD-UPPER LIMB PROM)
 Katrin Klingels1, Anna Mayhew2, Elena Mazzone2, Michelle Eagle1, Tina Duong1, Valérie Decostre1, Marion Main1, Marleen Van den Hauwe1, Ulla Werlauff1, Imelda De Groot6, Sonia Messina1, Valeria Ricotti1, Giles Campion1, Laurent Servais1, Elizabeth Vroom1, Eugenio Mercuri1, Nathalie Goemans1; 1Leuven, BE, 2Newcastle Upon Tyne, UK, 3Rome, IT, 4Stanford, US, 5Paris, FR, 6London, UK, 7Aarhus, DK, 8Nijmegen, NL, 9Messina, IT, 10Leiden, NL, 11Veenendaal, NL

PS2Group1-012  GENOTYPE PHENOTYPE ANALYSIS OF MULTIPLEX LIGATION DEPENDENT PROBE AMPLIFICATION (MLPA) POSITIVE DUCHENNE, BECKER MUSCULAR DYSTROPHY (DMD, BMD) PATIENTS
 Seena Vengalil, Kiran Polavarapu, Veeramani Preethish-Kumar, Atchayaram Nalini, Meera Purushottam, Deepha Sekar; Bangalore, IN

PS2Group1-013  CLINICAL AND MUSCLE BIOPSY CHARACTERISTICS OF A COHORT OF CHILDREN UNDER TWO YEARS OF AGE WITH DUCHENNE MUSCULAR DYSTROPHY
 Ana Sousa, Elisa Costa, Ricardo Taipa, Melo Pires, Manuela Santoso; Porto, PT

PS2Group1-014  NOVEL MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY WITH DELETION OF EXONS 834
 Tatiana Dimitrieva, Alexey Deikin, Denis Reshetov, Dmitry Vlodavets, Eugenia Zотовa; 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 Goemans1, James Signorovitch2, Elyse Swallow2, Jinlin Song2, Susan Ward3; 1Leuven, BE, 2Boston, MA, US, 3Cambridge, MA, US

PS2Group1-018  DIAGNOSIS OF DUCHENNE MUSCULAR DYSTROPHY IN ITALY: CRITICAL ISSUES AND AREAS FOR IMPROVEMENTS
 Adele D’Amico, Michela Catteruccia, Marika Pane, Giovanni Baranello, Alessandra Govoni, Sonia Messina, Maria Grazia D’angelo, Luisa Politano, Ksenija Gorni, Stefano Carlo Prevaliti, Antonella Pini, Roberta Battini, Angela Berardinelli, Federica Ricci, Elena Pegoraro, Claudio Bruno, Federica Trucco, Barbara Panasissi, Giuseppe Vita, Tiziana Mongini, Maurizio Moggi, Giacomo Pietro Comi; 1Rome, IT, 2Milan, IT, 3Messina, IT, 4Bosiso Parini, IT, 5Naples, IT, 6Bologne, IT, 7Pisa, IT, 8Pavia, IT, 9Turin, IT, 10Padua, IT, 11Genoa, IT

PS2Group1-019  THE PROPHYLACTIC USE OF PAMIDRONATE ON GLUCOCORTICOID-INDUCED BONE LOSS IN THE MDX MOUSE MODEL OF DUCHENNE MUSCULAR DYSTROPHY
 Sung-Hee Yoon, Jinhwan Chen, Marc Grynpas, Jane Mitchell; Toronto, ON, CA

PS2Group1-020  TRPV2 INHIBITION THERAPY CAN BE EFFECTIVE FOR CARDIOMYOPATHY OF MUSCULAR DYSTROPHY
 Tsuyoshi Matsumura, Misa Matsui, Yuko Iwata, Masanori Asakura, Toshio Saito, Harutoshi Fujimura, Saburo Sakoda; 1Toyonaka, JP, 2Suita, JP
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**PS2Group1-021** A MISSENSE MUTATION IN THE PUTATIVE SARCOPLASMIC RETICULUM TRANSMEMBRANE PROTEIN DCST2 CAUSES THE STRONGMAN SYNDROME

Talita Conte1, Martine Têtreault1, Marie-Josée Dicaire2, Sylvie Provost1, Najwa Al-Bustani3, Marie-Pierre Dubé4, Véronique Bolduc4, Myriam Sour4, Erin O’Ferrall1, Jean-Pierre Bouchard5, Gina Ravenscroft6, Russell Hepple6, Tanja Taivassalo6, Nigel Laing1, Phillipa Lamont1, Jean Mathieu1, Bernard Brais2, 3, 4, QC, CA, 9, QC, CA, 6, Montreal, QC, CA, 10, Nedlands, WA, AU, 11, Novato, CA, 11, Jonquiere, QC, CA

**PS2Group1-022** HEREDITARY MYOPATHY WITH EARLY RESPIRATORY FAILURE

Sandra Sousa1, Jorge Oliveira2, Emília Vieira3, Teresa Coelho2, Manuela Santos2, Marcio Cardoso2, Ricardo Taipa2, Melo Pires2, Rosário Santos2, Cascais, PT, 1, Porto, PT

**PS2Group1-023** GNE MYOPATHY: MILESTONES AND DISEASE PROGRESSION BASED ON PATIENT SELF-REPORTED DATA COLLECTED THROUGH THE GLOBAL PATIENT REGISTRY

Oksana Pogoryelova1, Phillip Cammish2, Supriya Rao3, Alison Skrinar3, Hanns Lochmüller4, Newcastle Upon Tyne, UK, 1, Newcastle Upon Tyne, UK, 2, Cascais, PT, 1, Porto, PT

**PS2Group1-024** MULTISYSTEM PROTEINOPATHY WITH MOTOR NERVE CONDUCTION BLOCKS

Oscar Trujillo, Juan Casar, Roger Gejman, Ricardo Fadic, Santiago, CL

**PS2Group1-025** PHENOTYPIC CHARACTERIZATION AND PATTERN OF MUSCLE INVOLVEMENT IN GNE MYOPATHY

Veeramani Preethish-Kumar1, Oksana Pogoryelova1, Kiran Polavarapu1, Narayanappa Gayathri1, Seena Vengalii1, Judith Hudson2, Chandrajit Prasad1, Hanns Lochmüller1, Newcastle Upon Tyne, UK, 1, Newcastle Upon Tyne, UK, 2, Newcastle Upon Tyne, UK

**PS2Group1-026** CALPAINOPATHIES IN CHILE

Jorge Bevilacqua1, Yves Mathieu2, Martin Krahn3, Marc Bartoli2, Claudia Castiglion3, Karin Kleinsteuber1, Jorge Díaz4, Francesca Puppo5, Mathieu Cerino6, Sebastien Courrier7, Svetlana Gorokhova8, Alejandra Trangulo9, Natalia Miranda9, Patricia Gonzalez-Hormazabal9, María De Los Ángeles Avaria1, J Urtizberea1, Pablo Caviedes1, Lilian Jara1, Nicolas Levy1, Santiago, CL, 1, Marseille, FR, 2, 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 Michaud1, Mathieu Fernandes1, Eve Duchemin-Pelletier1, Pauline Poydenot1, Pauline Menager1, Grenoble, FR, 1, 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-SARCOCYLAN MUTANTS BY MEANS OF PROTEIN FOLDING CORRECTORS

Chiara Fecchio1, Marcello Carotti1, Elisa Bianchini1, Romeo Betto1, Roberta Sacchetto1, Dorianna Sandona1, Padova, IT, 1, Legnano (pd), IT

**PS2Group1-030** CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: ONE-YEAR FOLLOW-UP

Meredith James1, Ursula Moore1, Anna Mayhew1, Michelle Eagle1, Karen Bettinson1, Elena Pegoraro2, Kate Bushby1, Newcastle Upon Tyne, UK, 1, Padova, IT

**PS2Group1-031** HISTOPATHOLOGICAL AND CLINICAL CHARACTERIZATION OF A SPORADIC TNPO3–MUTATED PATIENT

Alessandra Ruggieri1, Sara Gibertini1, Barbara Pasanisi1, Vincenzo Nigro2, Marco Savarese1, Maurizio Moggio3, Corrado Angelini1, Renato Mantegazza2, Lorenzo Maggi2, Lucia Morandi3, Marina Mora4, Milan, IT, 1, Naples, IT, 2, Venezia Lido, IT, 3, Milano, IT

**PS2Group1-032** ACE-083, A LOCALLY-ACTING MUSCLE AGENT, INCREASES MUSCLE VOLUME IN HEALTHY VOLUNTEERS

Kenneth Attie1, Chad Glasser1, Michael Gartner2, Brian Boes3, R Pearsall1, Xiaoza Zhang1, Jade Sun1, Brian Vidal1, Ashley Bellevue1, Monty Hankin1, Matthew Sherman1, Cambridge, MA, US, 1, Lincoln, NE, US

**PS2Group1-033** CLINICAL OUTCOME STUDY FOR DYSFERLINOPATHY: CLINICAL DATA FROM BASELINE ASSESSMENTS

Elizabeth Harris1, Ursula Moore2, Catherine Bladen1, Anna Mayhew1, Meredith James1, Karen Bettinson1, Heather Hillsden1, Hillarie Windish1, The Jain Foundation COS Consortium1, Kate Bushby1, Newcastle Upon Tyne, UK, 1, Seattle, WA, US
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José Andrés González Coraspe1, Denisa Hathazi2, Hanns Lochmüller2, René Zahedi2, Joachim Weis1, Andreas Roos1; 1Aachen, DE, 2Dortmund, DE, 3Newcastle, UK

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Judith Hudson1, Eileen Graham1, Chiara Marini Bettolo1, Teresinha Evangelista1, Volker Straub1, Fiona Norwood2, Kate Bushby1; 1Glasgow, UK, 2Newcastle Upon Tyne, UK

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Sofie Østergaard, Copenhagen, DK

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Maria Elena Farrugia1, Cheryl Longman1, William Stewart1, Volker Straub2, Richard Petty1; 1Glasgow, UK, 2Newcastle Upon Tyne, UK

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Sandra Donkervoort1, Yaqun Zou1, Antti Salo1, Aileen Barnes1, Ying Hu1, A Foley1, Elena Makareeva1, Meganne Leach1, Wendy DiNonno1, Taharey Dastgir1, Ronald Cohn1, Sergey Leikin1, Joan Marini1, Johanna Mylyharju1, Carsten Bonnemann1; 11477, MD, US, 2Bethesda, MD, US, 3Oulu, FI, 4Newport News, VA, US, 5Torrance, CA, US

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Megan Hodge¹, Jia Feng², Jean Mah³, Cooperative International Neuromuscular Research Group Investigators²; ¹Edmonton, AB, CA, ²Washington, DC, US, ³Calgary, AB, CA

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Wolfgang Löscher¹, Thomas Stulnig², Philip Simschitz¹, Michaela Brunner-Krainz³, Martina Huemer³, Stephan Iglseder³, Florian Lagler¹, Herman Moser³, Stefan Quasthoff¹, Julia Wanschitz¹, Wolfgang Grisold³; ¹Innsbruck, AT, ²Vienna, AT, ³Klagenfurt, AT, ⁴Graz, AT, ⁵Bregenz, AT, ⁶Linz, AT, ⁷Salzburg, AT, ⁸Altmünster Am Traunsee, AT, ⁹US

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