

## TREAT-NMD Limb Girdle Muscular Dystrophy USA Expert Masterclass

3<sup>rd</sup> & 4<sup>th</sup> November 2022

North America, Online

### Programme

#### Masterclass Main Goals

- Deliver an interactive teaching course of excellence aimed at health personnel involved in the diagnosis, management, and treatment of patients with limb girdle muscular dystrophies (LGMD), by providing updated information on the diagnosis, natural history, and treatment of the disease.
- Provide a platform to promote the exchange of ideas, experiences, and common challenges in delivering the highest standard of care to patients with LGMD.
- Provide an updated overview on the development of different therapeutic trials as well as state of the art emerging therapies for LGMD, and the implications of its implementation.

#### Masterclass Format

This event will be delivered online in English to a North American based audience via the Whova event platform. There will be a series of lectures given by expert opinion leaders, two interactive workshops and group discussions between experts and participants.

#### Day 1

The first day of the course will begin with the testimony of patients and parents of patients affected by LGMD, who will discuss the impact of Limb Girdle muscular dystrophies on their lives. This will be followed by a series of lectures on the different aspects of LGMDs given by experts. Attendees will have the opportunity to ask questions and interact with patients and speakers.

#### Day 2

On the second day of the course there will be additional lectures on specific aspects of the management of LGMD and an update on state of the art of care practices. At the end of each lecture there will be time dedicated to questions from the audience. Next, the attendees will be separated into two groups to participate in one of the two interactive workshops that will take place simultaneously.

#### Chairs

- **Dr Linda P Lowes**, Nationwide Children's Hospital, USA
- **Dr Conrad Chris Wehl**, Washington University School of Medicine, USA

## Pre-recorded Sessions

These pre-recorded webinars will be made available to attending delegates in advance of the live masterclass.

Session Duration	Pre-Recorded Session	Speaker
30 min	<b>LGMD Overview, Definitions &amp; Classification.</b>	<b>J. Andoni Urtizberrea,</b> Maladies Orphelines Sans Frontières, France
30 min	<b>Frequency and distribution of LGMDs across the world.</b>	<b>Giacomo Comi,</b> University of Milan, Italy
30 min	<b>Standard of Care: The psychological approach to the LGMD patient</b>	<b>Marcela Gargiulo,</b> Hospital Pitié-Salpêtrière, France
25 min	<b>Registries. Why are they needed and how to help LGMD patients get into a registry?</b>	<b>Sarah Emmons,</b> Jain Foundation, USA
25 min	<b>Approach to patients with LGMD weakness</b>	<b>Namita Goyal,</b> University of California-Irvine, USA
25 min	<b>Approach to ordering genetic testing Panel based testing. Exome/Panel.</b>	<b>Chelsea Chambers,</b> Virginia Commonwealth University, USA
25 min	<b>Approach to Muscle Biopsy</b>	<b>Robert Bucelli,</b> Washington University School of Medicine, USA
25 min	<b>Physical Therapy for the non-physical therapist</b>	<b>Stephanie Poelker,</b> Washington University School of Medicine, USA
25 min	<b>Best practices for a multidisciplinary clinic</b>	<b>Anne Connolly,</b> Nationwide Children's Hospital, USA
30 min	<b>Best practice for care management: Orthopedic Care</b>	<b>Linda Lowes,</b> Nationwide Children's Hospital, USA <b>Meredith James,</b> Newcastle University, UK

Min	EDT Time zone, USA	DAY 1, THURSDAY 3 <sup>rd</sup> NOVEMBER 2022 Running Time: 5 hours & 15 mins  12:00 – 17:15pm EDT, USA	
5 min	12:00 – 12:05	<b>Welcome, programme overview and introduction to TREAT-NMD</b>	<b>Co-chairs</b>
20 min	12:05 – 12:25	<b>Patient perspectives</b> <ul style="list-style-type: none"> <li>Impact of LGMD on their lives and patient journey</li> </ul>	<b>Jessica Evans,</b> Speak Foundation, USA
5 min	12:25 – 12:30	<b>Q &amp; A</b>	
25 min	12:30 – 12:55	<b>Differential diagnosis of LGMD</b> <ul style="list-style-type: none"> <li>Differential diagnosis – other neuromuscular disorders with limb girdle patterns of weakness, (Pompe, adult-onset SMA, inflammatory myopathies etc.)</li> <li>Variant Resolution</li> </ul>	<b>Edmar Zanoteli,</b> University of São Paulo, Brazil
5 min	12:55 – 13:00	<b>Q&amp;A</b>	
25 min	13:00 – 13:25	<b>Getting ready for post marketing surveillance &amp; minimum clinical standards</b>	<b>Linda Lowes,</b> Nationwide Children’s Hospital, USA
5 min	13:25 – 13:30	<b>Q&amp;A</b>	
30 min	13:30 – 14:00	<b>Natural History of functional abilities</b> <ul style="list-style-type: none"> <li>Comparing Dysferlinopathy, Calpainopathy, FKRP and Sarcoglycanopathies &amp; different rates of decline.</li> </ul>	<b>Meredith James,</b> Newcastle University, UK
5 min	14:00 – 14:05	<b>Q&amp;A</b>	
25 min	14:05 – 14:30	<b>Genetic Testing in LGMD</b> <ul style="list-style-type: none"> <li>Limitations of genetic testing</li> <li>The importance of a genetic diagnosis and understanding genetic variants.</li> <li>When to revisit a genetic diagnosis</li> </ul>	<b>Peter Kang,</b> University of Minnesota, USA
5 min	14:30 – 14:35	<b>Q&amp;A</b>	
25 min	14:35 – 15:00	<b>BREAK</b>	

15 min	15:00 – 15:15	<b>Autosomal Dominant LGMD</b>	<b>Andrew Findlay,</b> Washington University, USA
5 min	15:15 – 15:20	<b>Q&amp;A</b>	
25 min	15:20 – 15:45	<b>Recessive LGMDs: DYSF, CAPN3, Sarcoglycanopathies, FKRP, dystroglycanopathies &amp; ANO5</b>	<b>Katherine Mathews,</b> University of Iowa, USA
5 min	15:45 – 15:50	<b>Q &amp; A</b>	
15 min	15:50 – 16:05	<b>Rare and overlapping LGMD subtypes</b> <ul style="list-style-type: none"> <li>• Titinopathy, LGMD-myasthenia, new genes.</li> </ul>	<b>Bjarne Udd,</b> University of Tampere, Finland
5 min	16:05 – 16:10	<b>Q &amp; A</b>	
25 min	16:10 – 16:35	<b>Pathophysiology</b>	<b>Jorge Bevilacqua,</b> University of Chile, Chile
5 min	16:35 – 16:40	<b>Q &amp; A</b>	
25 min	16:40 – 17:05	<b>The additional contribution of muscle biopsy &amp; MRI in the diagnosis of LGMD</b>	<b>Jordi Diaz-Manera,</b> Newcastle University, UK
5 min	17:05 – 17:10	<b>Q &amp; A</b>	
5 min	17:10 - 17:15	<b>Summary</b>	<b>Co-chairs</b>
<b>Min</b>	<b>EDT Time Zone, USA</b>	<b>DAY 2, FRIDAY 4<sup>th</sup> NOVEMBER 2022</b> <b>Running time: 5 hours 30 mins</b>  <b>12:00- 17:15 pm EDT, USA</b>	
5 min	12:00 – 12:05	<b>Welcome</b>	
25 min	12:05 – 12:30	<b>Clinical trials landscape &amp; emerging therapies.</b>	<b>Nick Johnson,</b> Children’s Hospital of Richmond at VCU, USA
5 min	12:30 – 12:35	<b>Q &amp; A</b>	

25 min	12:35 – 13:00	<b>Understanding Variants of Unknown Significance in LGMD genetic testing.</b>	<b>Chris Weihl,</b> Washington University, USA
5 min	13:00 – 13:05	<b>Q&amp;A</b>	
15 min	13:05 – 13:20	<b>Best practice for care management: Cardiac</b>	<b>Nathan Stitzel,</b> Washington University, USA
5 min	13:20 – 13:25	<b>Q &amp; A</b>	
15 min	13:25 – 13:40	<b>Best practice for care management: Respiratory</b>	<b>Grace Paul,</b> Nationwide Children’s Hospital, USA
5 min	13:40 – 13:45	<b>Q &amp; A</b>	
30 min	13:45- 14:15	<b>BREAK</b>	
3 hours	14:15 – 17:15	<b>Workshops</b> <i>(Includes a short break)</i>	

**WE HAVE TWO WORKSHOPS RUNNING CONCURRENTLY.**

## **Workshop 1: Physiotherapy and other clinical care considerations**

<b>Min</b>	<b>EDT Time</b>	<b>Physiotherapy and other clinical care considerations</b>
<b>3 hours</b> <i>(includes a short break)</i>	<b>14:15 – 17:15</b>	<ul style="list-style-type: none"> <li>-Host 1 Lindsay Alfano, Nationwide Childrens Hospital, USA</li> <li>-Host 2 Katie Laubscher, University of Iowa, USA</li> <li>-Host 3 Megan Iammorino, Nationwide Children’s Hospital, USA</li> </ul> <p>This interactive workshop will provide an overview and discussion on the multidisciplinary care team and principles of management of the limb girdle muscular dystrophies. Through case presentations and discussion attendees will be guided through the diagnostic process, clinical evaluation, and considerations for management in the home, school, work, and other community environments.</p>

## Workshop 2: Exemplary Clinical Cases

Min	EDT Time	Exemplary Clinical Cases
<b>3 hours</b>  <i>(includes a short break)</i>	<b>14:15 – 17:15</b>	<ul style="list-style-type: none"> <li>-Host 1 Edmar Zanoteli, University of São Paulo, Brazil</li> <li>-Host 2 Jorge Bevilacqua, University of Chile</li> <li>-Host 3 Chris Wehl, Washington University School of Medicine, USA</li> </ul> <p>Through presentations and group discussion, attendees will be guided through the diagnostic process for some exemplary clinical cases of LGMD.</p>