

Expert Masterclass on Limb Girdle Muscular Dystrophy

29th and 30th April 2021, virtual online event

Masterclass Programme

Masterclass Objectives

- Deliver high quality educational and interactive event for health care providers diagnosing or managing the care of patients with the Limb Girdle Muscular Dystrophies (LGMD) by providing information on the latest in diagnosis, natural history, and care discussions.
- Provide a platform for health care providers to share ideas, challenges, and successes in delivering optimal care to people with LGMD.
- Provide an overview of the current clinical trial landscape and the emerging implications on care.

Masterclass Delivery Format

Due to the situation with COVID-19 a decision has now been made to deliver this expert Masterclass virtually, as a wholly online event using our interactive web-conferencing platform, Whova. The format will involve lectures from expert speakers, two interactive workshops and opportunities for Q&A throughout.

Thursday 29th April 2021

- Day one of the masterclass will include a series of lectures delivered by a range of clinicians with expertise in LGMD. The masterclass will also include input from patients with LGMD who will discuss the impact of LGMD on their lives. Delegates will be given opportunity for Q&A throughout.

Friday 30th April 2021

- The day will begin with a further six speakers delivering a diverse range of lectures with opportunity for Q&A.
- Delegates will then be organised into one of two interactive workshops; these workshops will be delivered concurrently.

Chairs

- **Professor Jordi Diaz Manera**, Neurologist, John Walton Muscular Dystrophy Research Centre, UK
- **Dr. Michela Guglieri**, Research Fellow/Honorary Consultant, John Walton Muscular Dystrophy Research Centre, UK

N.B. Please note this programme remains subject to change

PRE-RECORDED SESSIONS

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

PRE-RECORDED VIDEO - DURATION	PROGRAMME SESSION	SPEAKER DETAILS
30 minutes	LGMD Overview, Definitions & Classification	Dr. J. Andoni Urtizberea
30 minutes	Frequency and distribution of LGMDs across the world. <ul style="list-style-type: none"> Insights from the NGS screening focus in LATAM Recently published papers in USA 	Dr. Giacomo Comi
30 minutes	Clinical trials landscape & emerging therapies	Prof. Jordi Diaz Manera
60 minutes	Natural history of the most frequent LGMDs <ul style="list-style-type: none"> FKRP and CAPN3 Dysferlinopathies 	Dr. John Vissing Dr. Meredith James This pre-recorded webinar will be jointly delivered by both speakers
20 minutes	Best practice for care management 1. Orthopaedics Care	Dr. Linda Lowes

Virtual Masterclass (Day 1)

- British Summer Time (BST) – 09:00 – 13:15 (4 hours 15 minutes)
- Central European Time (CET) – 10:00 – 14:15 (4 hours 15 minutes)

DAY 1 THURSDAY 29 TH APRIL 2021				
DURATION	GMT	CET	PROGRAMME SESSION	SPEAKER DETAILS
5 minutes	09:00	10:00	Welcome, programme overview and introduction to TREAT-NMD	Professor Jordi Diaz Manera Dr. Michela Guglieri
30 minutes	09:06 – 09:14	10:06 – 10:14	Patient perspectives <ul style="list-style-type: none"> • Impact of LGMD on their lives and journey 	Lluís Garcia Pareras
	09:14 – 09:22	10:14 – 10:22	Patient perspectives <ul style="list-style-type: none"> • Impact of LGMD on their lives and journey 	TBA – In progress, final speaker names will be released once confirmed
	09:22 – 09:30	10:22 – 10:30	Patient / Parent perspectives <ul style="list-style-type: none"> • Impact of LGMD on their lives and journey 	TBA – In progress, final speaker names will be released once confirmed
30 minutes	09:30	10:30	Diagnostic journey of the LGMD patient. <ul style="list-style-type: none"> • Differential diagnosis – other neuromuscular disorders with limb girdle patterns of weakness (Pompe, adult-onset SMA) 	<ul style="list-style-type: none"> • Dr Carmen Paradas • Patient Speakers (TBA) This session will be jointly delivered by both speakers
	09:55	10:55	Questions and Answers	The speakers will answer a selection of moderated questions
30 minutes	10:00	11:00	Most frequent recessive forms: DYSF, ANO5 and SGCA <ul style="list-style-type: none"> • Including an introduction to Pathophysiology 	Dr. Elena Pegoraro
	10:25	11:25	Questions and Answers	The speaker will answer a selection of moderated questions
15	10:30 – 10:45	11:30 – 11:45	BREAK	
30 minutes	10:45	11:45	Most frequent recessive forms II CAPN3 and FKR1 and dystroglycanopathies <ul style="list-style-type: none"> • Including an introduction to Pathophysiology 	Dr. Adolfo Lopez de Munain
	11:10	12:10	Questions and Answers	The speaker will answer a selection of moderated questions

30 minutes	11:15	12:15	Dominant TTN and the dominant LGMD's • Including an introduction to Pathophysiology	Dr. Bjarne Udd
	11:40	12:40	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	11:45	12:45	Muscle biopsy as a tool to diagnose patients with LGMD	Dr. Montse Olive
	12:10	13:10	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	12:15	13:15	Uses and applications of muscle MRI in LGMD's	Dr. Giorgio Tasca
	12:40	13:40	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	12:45 – 13:10	13:45 – 14:10	Benefits and limitations of NGS in neuromuscular disease	Ana Topf
	13:10 – 13:15	14:10 – 14:15	Questions and Answers	The speaker will answer a selection of moderated questions

Virtual Masterclass (Day 2)

- British Summer Time (BST) – 09:00 – 13:50 (4 hours 50 minutes)
- Central European Time (CET) – 10:00 – 14:50 (4 hours 50 minutes)

DAY 2 FRIDAY 30TH APRIL 2021

DURATION	GMT	CET	PROGRAMME SESSION	SPEAKER
30 minutes	09:00	10:00	Best practice for care management - Cardiological	Dr. Karim Wahbi
	09:25	10:25	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	09:30	10:30	Best practice for care management - Respiratory	Dr. Stephan Wenninger
	09:55	10:55	Questions and Answers	The speaker will answer a selection of moderated questions

30 minutes	10:00	11:00	Registries <ul style="list-style-type: none"> • Why are they needed and the practicalities of setting up a registry 	Dr. Michela Guglieri
	10:25 – 10:30	11:25 – 11:30	Questions and Answers	The speaker will answer a selection of moderated questions
15 minutes	10:30 – 10:45	11:30 – 11:45	BREAK	
		<i>WE HAVE ARRANGED TWO INTERACTIVE WORKSHOPS; THESE WORKSHOPS WILL BE DELIVERED CONCURRENTLY. WHERE POSSIBLE DELEGATES WILL BE ASSIGNED ONTO A WORKSHOP OF THEIR CHOICE.</i>		
3 hours	10:45 – 13:45	11:45 – 14:45	<p style="text-align: center;"><u>Workshop 1</u></p> <p style="text-align: center;">Physiotherapy and other Clinical Care Considerations</p> <p>Workshops hosts:</p> <ul style="list-style-type: none"> • Dr Lindsay Alfano • Dr Anna • Dr Linda Lowes • Dr Meredith James <p>An interactive workshop which will break-out into small groups. Workshop discussions will include</p> <ul style="list-style-type: none"> • Physiotherapy clinical care considerations <ul style="list-style-type: none"> - Exercise, stretching, bracing and equipment • Outcome measures <ul style="list-style-type: none"> - Strength and functional testing - Patient reported measures • Occupational therapy • General nutrition <ul style="list-style-type: none"> - Examining the scientific evidence of some common diets and supplements 	<p style="text-align: center;"><u>Workshop 2</u></p> <p style="text-align: center;">Exemplary Clinical Cases</p> <p>Workshop hosts:</p> <ul style="list-style-type: none"> • Prof. Jordi Diaz Manera • Dr. Giorgio Tasca • Dr. Carmen Paradas • Dr. Elena Pegoraro <p>Demonstration and group discussions around 4 clinical cases Group interaction, feedback, Q&A</p>
5 Minutes	13:45 – 13:50	14:45 – 14:50	Masterclass close and sum up	Professor Jordi Diaz Manera Dr. Michela Guglieri