

Expert Masterclass on Limb Girdle Muscular Dystrophy

Wednesday 23rd - Thursday 24th September 2020, virtual online learning

Programme

Meeting Objectives

- Deliver high quality educational and interactive meeting for 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

Meeting Format

This is a wholly online masterclass arranged over two days. The format of the masterclass has been organised with online lectures and an interactive workshop.

Wednesday 23rd September

- Day one of the masterclass will include a series of online webinar lectures from a range of clinicians with expertise in LGMD. The masterclass also includes input from a small number of patients and parents who will discuss the impact of LGMD on their lives.

Thursday 24th September

- Day will begin with a further three online webinar lectures
- Delegates will then be organised into one of the two interactive online workshops running concurrently

Pre-recorded lectures

- A series of five pre-recorded webinars will be available to view in advance of the masterclass

Chairs

- **Professor Jordi Diaz Manera** Neurologist, John Walton Muscular Dystrophy Research Centre, UK
- **Dr Lindsay Alfano** - Physical Therapist, Nationwide Children's Hospital, Centre for Gene Therapy

Expert Masterclass on Limb Girdle Muscular Dystrophy

PROGRAMME CHECKLIST

PRE-RECORDED SESSIONS

N.B. Will be made available to delegates in advance of the live masterclass

PRE-RECORDED VEDEO - DURATRION	PROGRAMME SESSION	SPEAKER DETAILS
30 minutes	LGMD overview, definitions, and classification	Dr Matthew Wicklund , Neurologist, University of Colorado, USA
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 Tahseen Mozaffar , Neurologist, UC Irvine, USA
30 minutes	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, etc) 	Dr Jeff Statland , Neurologist, Kansas University, USA
60 minutes	Natural history of the most frequent LGMDs	Dr Lindsay Alfano , Physical Therapist, Nationwide Children’s Hospital – check if need slides? Dr Meredith James , Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK
20 minutes	Best practice for Care management <ul style="list-style-type: none"> Orthopaedics care 	Dr Linda Lowes , Physical Therapist, Nationwide Children’s Hospital

THE ONLINE MASTERCLASS (Day 1)

- Eastern Daylight Time (EDT) US – 11am – 3:15pm (4 hours 15 minutes)
- Pacific Daylight Time (PDT) US – 8am – 12:15pm (4 hours 15 minutes)
- British Summer Time (BST) – 4pm – 8:15pm (4 hours 15 minutes)

Day 1 - Wednesday 23rd September 2020

Duration	PDT	EDT	BST	Session Chair	PROGRAMME SESSION	SPEAKER
5 minutes	8.00am	11.00am	4.00pm	JDM/LA	Welcome, programme overview and introduction to TREAT-NMD	Professor Jordi Diaz Manera, Neurologist, John Walton Muscular Dystrophy Research Centre, UK Dr Lindsay Alfano, Physical Therapist, Nationwide Children's Hospital
8 minutes	8:05 – 8.13am	11:05 – 11.13am	4.05 – 4.13pm	LA	Patient perspective <ul style="list-style-type: none"> • Impact of LGMD on their lives and journey 	Dr Vovanti Jones
8 minutes	8.13 – 8.21am	11.13 – 11.21am	4.13 – 4.21pm	LA	Parent perspective <ul style="list-style-type: none"> • impact of LGMD on their lives and journey 	Scott and Char Frewing
8 minutes	8.21– 8.29am	11.21 – 11.29am	4.21 – 4.29pm	LA	Patient perspective impact of LGMD on their lives and journey.	Andrea Lane
30 minutes	8.30 – 9am	11.30 – 12.00pm	4.30 = 5pm	JDM	Most frequent recessive forms of LGMD R1 CAPN3, R2 DYSF, R12 ANO5 <ul style="list-style-type: none"> • Including an introduction to Pathophysiology 	Dr Nick Johnson, Neurologist, Virginia Commonwealth University, USA
30 minutes	9.00 – 9.30am	12:00pm – 12.30pm	5:00 – 5.30pm	LA	Most frequent recessive forms of LGMD R3- 6SGCA-G, and R9 FKR P and dystroglycanopathies <ul style="list-style-type: none"> • Including an introduction to Pathophysiology 	Dr Kathy Mathews, Neurologist University of Iowa, USA
30 minutes	9.30 = 10am	12:30 – 1pm	5.30 – 6pm	LA	Dominant LGMD <ul style="list-style-type: none"> • Including an introduction to Pathophysiology 	Dr C. Chris Wehl, Neurologist, University of Washington – St. Louis, USA
BREAK – (15 Minutes)						
30 minutes	10:15 – 10.45 am	1.15 – 1.45pm	6.15 – 6.45pm	JDM	Uses and applications of the muscle biopsy and other molecular methods for diagnosis in LGMD	Dr Jim Dowling, Neurologist, The Hospital for Sick Children, Canada
30 minutes	10.45 – 11.15 am	1.45 – 2.15pm	6.45 – 7.15pm	LA	Uses and applications of muscle MRI in LGMDs	Professor Jordi Diaz Manera, Neurologist, John Walton Muscular Dystrophy Research Centre, UK
30 minutes	11:15 – 11.45 am	2.15 – 2.45pm	7.15 – 7.45pm	JDM	Best practice on genetic diagnostic approaches When to use NGS, WES or WGS?	Dr Monkol Lek, Assistant Professor, Yale School of Medicine, USA

30 mins	11.45 am – 12.15 pm	2.45 – 3.15 pm	7.45pm – 8.15pm	LA	Clinical trials landscape & emerging therapies	Dr Nick Johnson, Neurologist, Virginia Commonwealth University, USA
THE ONLINE MASTERCLASS (Day 2) <ul style="list-style-type: none"> Eastern Daylight Time (EST) US – 11am – 3.45 pm (4 hours, 45 minutes) Pacific Daylight Time (PDT) US – 8am – 12.45pm (4 hours, 45 minutes) British Summer Time (BST) – 4pm – 8.45pm (4 hours, 45 minutes) 						
Day 2 - Thursday 24th September 2020						
Duration	PDT	EDT	BST	Session chair	PROGRAMME SESSION	SPEAKER
30 mins	8:00am	11.00am	4.00pm	JDM	Best practice for Care management - Cardiological	Dr Linda Cripe, Cardiologist, Nationwide Children's Hospital, USA
30 mins	8.30am	11.30am	4.30pm	LA	Best practice for Care management - Respiratory	Dr Grace Paul, Paediatric Pulmonologist, Nationwide Children's Hospital
30 mins	9:00am	12:00	5.00pm	JDM	Registries <ul style="list-style-type: none"> Why are they needed Practicalities of setting up a registry 	Dr Victoria Hodgkinson, National Program Manager, TREAT-NMD SMA Registries, Canada
BREAK – (15 Minutes)						
15 mins	9.30 – 9.45	12.30 - 12.45	5.30 – 5.45		BREAK	

WE HAVE TWO WORKSHOPS RUNNING CONCURRENTLY FOR THE NEXT SESSION. WHERE POSSIBLE YOU HAVE BEEN ASSIGNED A WORKSHOP IN LINE WITH YOUR PREFERENCE. PLEASE SEE DETAILS OF EACH WORKSHOP BELOW, STARTING WITH WORKSHOP 1 AND FOLLOWED BY WORKSHOP 2.

Workshop 1

Physiotherapy and other clinical care considerations

Dr Lindsay Alfano, Physical Therapist, Nationwide Children's Hospital
 Dr Anna Mayhew, Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK
 Dr Linda Lowes, Physical Therapist, Nationwide Children's Hospital
 Dr Meredith James, Physiotherapist, John Walton Muscular Dystrophy Research Centre, UK

Duration	PDT	EDT	BST	Workshops session chair	Workshop session Title	Additional delegate Information
5	9.45 – 9.50am	12.45 – 12.50pm	5.45 – 5.50pm	Lindsay Alfano	Workshop Introduction	
30	9.50 – 10.20am	12.50 – 1.20pm	5.50 – 6.20pm	Meredith James	Multi-disciplinary care and principles of PT management of Limb Girdle Muscular Dystrophies	
20	10.20 – 10.40am	1.20 – 1.40pm	6.20 – 6.40pm	Anna Mayhew	Case study 1 Presentation of principles assessment and management	All delegates will be in one group for this case study

						The session will involve asking for delegate opinion using a system of polling
10	10.40 – 10.50am	1.40– 1.50pm	6.40 – 6.50pm	Linda Lowes	Case study 2 Introduction (one large group)	
15	10.50 – 11.05am	1.50 – 2.05pm	6.50 – 7.05pm	Break		
20	11.05 – 11.25am	2.05pm – 2.25pm	7.05 – 7.25pm	LA MJ LL AM	Case Care Consideration Discussion in Breakouts 4 smaller groups	Delegates will be separated into 4 zoom breakout rooms (4 chairs will lead the conversations in each room) One of the delegates will be nominated as spokesperson to feedback at the end
20	11.25 – 11.45am	2.25 – 2.45pm	7.25 – 7.45pm	LA	Case study 2 discussion (one large group)	4 nominated speakers from each sub-group to share group thoughts and observations to the wider group
15	11.45 – 12.00pm	2.45 – 3pm	7.45 – 8pm	Meredith James	Case study 3 (one large group) (case study to be provided to give to delegates in advance of workshop)	
15	12.00 – 12.15pm	3pm – 3.15pm	8pm – 8.15pm	LA MJ LL AM	Case Care Consideration Discussion in Breakouts 4 smaller groups	Delegates will be separated into 4 zoom breakout rooms (4 chairs will lead the conversations in each room) One of the delegates will be nominated as spokesperson to feedback at the end
15	12.15 – 12.30	3.15 – 3.30pm	8.15 – 8.30pm	AM	Case study 3 discussion (one large group)	4 nominated speakers from each sub-group to share group thoughts and observations to the wider group
15	12.30 – 12.45pm	3.30 – 3.45pm	8.30 – 8.45pm	Lindsay Alfano	Additional case considerations and workshop sum up	The workshop group will return as one group for the final presentation

Workshop 2

Exemplary clinical cases

Professor Jordi Diaz Manera, Neurologist, John Walton Muscular Dystrophy Research Centre, UK
Dr C. Chris Weihl, Neurologist, University of Washington – St. Louis, USA
Dr Nick Johnson, Neurologist, Virginia Commonwealth University, USA
Dr Jim Dowling, Neurologist, The Hospital for Sick Children, Canada

Duration	PDT	EDT	BST	Workshops session chair	Workshop session Title	Additional Information
5	9.45 – 9.50am	12:45 – 12.50pm	5.45 – 5.50pm	JDM	Introduction to the workshop session	

30	9.50 – 10.20am	12.50 – 1.20pm	5.50 – 6.20pm	JDM	Case Study 1 Professor Jordi Diaz Manera , Neurologist, John Walton Muscular Dystrophy Research Centre, UK	Prof. Diaz Manera will present a clinical case study to all workshop delegates as one large group. Throughout the workshop delegates will be asked to give their opinions in the form of a polling system that is built into the virtual conference app.
10	10.20 – 10.30am	1.20 – 1.30pm	6.20 – 6.30pm	JDM	Questions and Answers – following the case study one presentation	Delegates will be encouraged to ask questions using the group chat facility throughout the presentation. Appropriate questions will be moderated and answered where possible during this session.
30	10.30 – 11.00am	1.30 - 2pm	6.30- 7pm	CW	Case Study 2 Dr C. Chris Weihl , Neurologist, University of Washington – St. Louis, USA	Dr Weihl will present another clinical case study to all workshop delegates as one large group. Throughout the workshop delegates will be asked to give their opinions in the form of a polling system that is built into the virtual conference app.
10	11.00 – 11.10am	2:00 – 2.10pm	7.00 – 7.10pm	CW	Case Study 2 Q&A – following the case study one presentation	Delegates will be encouraged to ask questions using the group chat facility throughout the presentation. Appropriate questions will be moderated and answered where possible during this session.
10	11.10 - 11.20am	2.10 – 2.20pm	7.10 – 7.20pm		BREAK	
30	11.20 – 11.50am	2.20 – 2.50pm	7.20 – 7.50pm	NJ	Case Study 3 Dr Nick Johnson , Neurologist, Virginia Commonwealth University, USA	Dr Johnson will present a clinical case study to all workshop delegates as one large group. Throughout the workshop delegates will be asked to give their opinions in the form of a polling system that is built into the virtual conference app.
10	11.50 – 12.00 (Noon)	2.50 – 3.00pm	7.50 – 8.00pm	NJ	Case Study 3 Questions and Answers – following the case study one presentation	Delegates will be encouraged to ask questions using the group chat facility throughout the presentation. Appropriate questions will be moderated and answered where possible during this session.
30	12.00 – 12.30pm	3.00 – 3.30pm	8.00 – 8.30pm	JD	Case Study 4 Dr Jim Dowling , Neurologist, The Hospital for Sick Children, Canada	Dr Dowling will present a clinical case study to all workshop delegates as one large group. Throughout the workshop delegates will be asked to give

						their opinions in the form of a polling system that is built into the virtual conference app.
10	12.30 – 12.40pm	3.30 – 3.40pm	8.30 – 8.40pm	JD	Case Study 4 Questions and Answers – following the case study one presentation	Delegates will be encouraged to ask questions using the group chat facility throughout the presentation. Appropriate questions will be moderated and answered where possible during this session.
5	12.40 – 12.45pm	3.40 – 3.45pm	8.40 – 8.45pm	JDM	Close and sum up	JDM will sum up and close the session