

TREAT-NMD LGMD European Expert Masterclass

29th & 30th April 2021

Post Event Summary Report



TREAT-NMD would like to take this opportunity to thank our funding partners for supporting the development and delivery of this Masterclass. We are grateful to Sarepta Inc, Edgewise Therapeutics, ML Bio Solutions and the Jain Foundation. Without such generosity, this event would not have been possible.

Contents

Executive Summary.....	3
Objectives.....	4
Applicant and Attendee Analysis	5
Post Event Delegate Survey - Responses	8
Achievement of Masterclass Objectives.....	16
Masterclass Reflection and Lessons Learned - PMI	17
Appendix	19

N.B In some instances, % scores may not total 100% due to rounding of data sets.

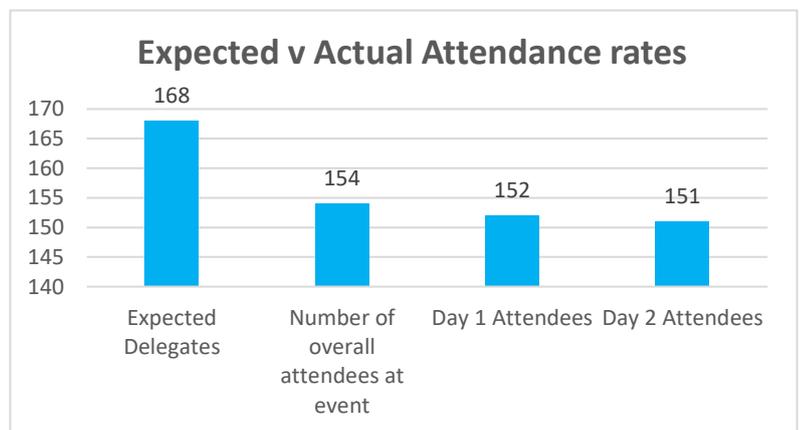
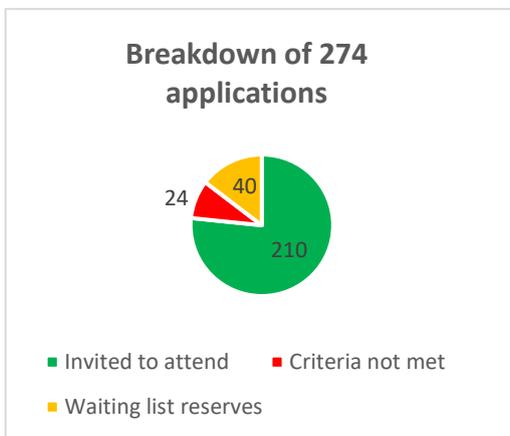
Executive Summary

The LGMD April Masterclass took place on the 29th & 30th April 2021. The event was originally scheduled to take place in Barcelona for a predominantly European audience, but due to Covid-19 was subsequently hosted as an online, virtual event. The event ran for a total of 9 hours and 5 minutes which was split over the 2 days. Content was presented in the English Language.

The event was planned and organised by the TREAT-NMD LGMD Education Working Group (EdWG). The Masterclass was awarded EACCME accreditation and eligible delegates could later claim up to 8 ECMEC accreditation points for attending the event.

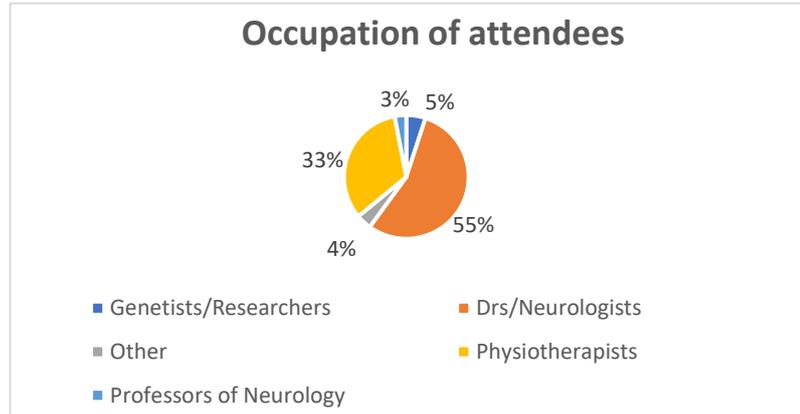
The co-chairs of the event were Professor Jordi Diaz-Manera and Dr Michela Guglieri from Newcastle University, UK. 20 guest speakers consisting of 18 expert clinicians/physiotherapists and 2 Patient Perspectives Speakers presented at the event. 5 sessions were pre-recorded by experts which produced an additional 3 hours and 15 minutes of educational content.

274 total applications were received. 210 applicants were identified as suitable candidates based on appropriate location, occupation, higher frequency of contact with LGMD and greater influence in the role of diagnosing and treating patients. 168 delegates confirmed their attendance of the event of which, 154 (not inclusive of the co-chairs and speakers) then attended over the two days.



Out of 107 delegates surveys:

100%	rated the organisation of the event as Excellent or Good.
98%	rated the Masterclass as Very Useful/Useful for their professional practice.
99%	rated the Masterclass event programme as being Excellent or Good.



Objectives

The main objectives of the April Masterclass were to:

- Deliver a 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.

The order of Day 1 included 2 patient perspective talks followed by several expert speaker webinars. Day 2 consisted of more expert speaker sessions followed by 2 delegate workshops. The 2 workshops covered the following specialisms: Physiotherapy and other clinical care considerations or Exemplary Clinical Cases. Each of these workshops was hosted by four expert speakers. This structure enabled delegates to be split into smaller Zoom break-out rooms, each led by one expert speaker. This enabled experts and delegates to discuss topics more freely, share best practice with the small groups and promote discussions and networking opportunities.

The online event was run via the Whova event platform. This platform was used as a tool to promote the event agenda, upload pre-recorded videos for delegates to watch prior to and after the event, upload workshop materials such as Workshop activity sheets as well as communicate and network with attendees. Delegates were encouraged to engage with Whova by using this platform to view speaker and delegate bios to network, post social events, and topics of discussion to each other, post questions to expert speakers during live sessions as well as take part in interactive polls.

The event programme in Appendix 1 provides details about the session themes and speaker involvement.

To ensure smooth delivery on the day of the event for both expert speakers and delegates, a series of online 'drop-ins' were arranged and hosted by TREAT-NMD staff in order to allow both parties the opportunity to test their system connections with Whova, ask any questions about Whova or the event programme and understand the system and technology requirements of the day. Alongside this, a TREAT-NMD webinar video was developed by staff for delegates to drive up the download and satisfaction rates of delegates using Whova. The video showed delegates how the platform worked, how to view the agenda and watch sessions as well as cover optimal system and technology requirements to access content and translation services on the day.

Applicant and Attendee Analysis

The initial target number of delegates to attend this event was 150. The TREAT-NMD Education Working Group (EdWG) later took the decision to increase the target attendee rate to 170 delegates and accept more applicants given the number of applications received, the flexibility of the Whova platform and EACCME regulations to accommodate a slight increase in numbers. Application forms were received over a 5-month period and a total of 274 applications from interested parties were received. To allow for delegate drop-out rates, 210 suitable delegates were contacted in order to try and reach the 170 delegate mark.

In order to qualify for selection, applicants were asked to complete the following selection criteria such as: occupation, affiliation, industry, and global region. Delegates were also required to answer how frequently they were in contact with people living with LGMD, how the masterclass would be of benefit to them, what they hoped to learn and how they would later cascade their learning to others. All applications were reviewed on a case-by-case basis and an initial cohort of suitable applications was sent to the Education Working Group for review and approval in order to quality assure the process of vetting and accepting suitable candidates.

Out of the 274 applicants, 210 were offered a place. 24 were identified as not applicable mostly due to location outside of Europe. In some instances, applicants were not of a medical background as applications were received from a Librarian, Teacher, Businessman, and a parent to a child with LGMD.

Priority places were given first to European applicants who originally applied to the USA LGMD Masterclass, but who were not accepted to that Masterclass because of their location outside of the USA. Physiotherapists, Neurologists, and then doctors/other medical job roles with frequent contact with LGMD patients were offered places during the next wave of allocations.

40 delegates amongst the applications were listed as suitable candidates but were classified as a 'reserve' invite due to the limiting of numbers, and in most instances, colleagues from the same institution with more frequent interaction with LGMD patients, had already been offered a place to attend first prior to the reserves.

The EdWG later agreed the decision to offer places to relevant applicants outside Europe to ensure more inclusivity to suitable delegates in underserved areas not regionally covered by current LGMD masterclasses. This translated to 9% of all attendees coming from outside of the European catchment area.

Out of the 210 applicants who were contacted by TREAT-NMD and offered a place, 31 delegates did not respond to confirm their place and 7 withdrew their application due to other emerging commitments. TREAT-NMD expected 168 delegates to attend prior to the event though 19 confirmed applicants did not attend. 5 extra delegates accepted their place on the day of the event bringing the total number of attending delegates to 154. The drop-

out rate between the expected vs attending delegates was 11%. Compared to other masterclass events, this dropout rate is lower than expected.

Location of applicants.

Applications were received across 47 countries. Attending delegates were geographically located across 38 countries.

The table below gives a breakdown of locations and number of applications and attendees from all applications.

Table: The Geographical location and attendance rates of delegates

Country	No. of applications	No. Invited to attend	No. of Attending delegates
Argentina	6	0	0
Australia	3	2	2
Belgium	7	7	7
Bulgaria	2	2	1
Canada	5	2	1
Croatia	3	2	1
Czech Republic	3	3	2
Denmark	3	3	3
Egypt	2	2	2
Finland	1	1	1
France	9	8	7
Germany	4	4	3
Greece	3	3	2
Haiti	1	0	0
Hungary	1	1	0
India	6	2	2
Indonesia	1	1	1
Ireland	2	2	2
Italy	15	12	10
Japan	1	1	1
Korea (Republic of)	1	0	0
Latvia	7	6	5
Lithuania	1	1	1
Mali	1	1	1
Moldova	1	1	1
Mozambique	1	1	0
Nepal	1	0	0
Netherlands	12	10	8

Norway	7	6	6
Oman	1	1	1
Philippines	1	1	1
Poland	10	9	7
Portugal	4	4	1
Romania	6	6	4
Russia	1	1	1
Saudi Arabia	2	1	1
Serbia	1	1	1
Slovenia	1	1	1
South Africa	1	1	0
Spain	30	28	12
Sudan	1	1	0
Sweden	5	3	2
Switzerland	1	1	1
Turkey	6	6	5
Ukraine	2	2	2
United Kingdom	88	57	44
USA	3	2	0

As the data in the table shows, the UK submitted the most applications, the proportion of which is 32% of total applications. This trend could be explained by the networking and promotions of the event being stronger amongst the UK market and could indicate that a wider network of promotional contacts is needed for future events. Spain submitted the second highest number of applicants (11% of total applications). 93% of these applicants were offered a place yet the actual attendance rate was unexpectedly low with only 43% attending. This is explained by 2 applicants withdrawing due to other commitments, 10 delegates not responding to communications to accept their place and 4 confirmed and expected delegates being no-shows at the event.

Delegate occupation:

Out of the 154 attending delegates, 63% of delegate's job titles were listed as being a Neurologist, Doctor, Clinical Geneticist, Clinical Researcher or Professor of Neurology. 33% of delegates were in the role of Physiotherapist. The 4% of 'other' occupations consisted of Muscular Dystrophy/Neuromuscular Care Advisors, Neuromuscular nurses and 1 Chair of an LGMD Patient Organisation.

Post Event Delegate Survey - Responses

The data and quotations in this section are accumulated from the 107 returned Delegate Surveys and via the Whova event platform. The details below provide an overview of satisfaction and engagement across all data sources. Appendix 2 provides a full copy of all returned Delegate Survey feedback.

Delegate perception regarding the usefulness of the Masterclass and the event programme.

98% of delegates found the event useful for their professional practice.

Of the 2% who rated the masterclass as somewhat useful, the delegate surveys recorded the reasons of:

"{wishing} the subtypes of LGMD were taught a little slower to give {delegates} time to absorb the differences."

"For me as a resident doctor in medical genetics, {the masterclass} was somehow a bit too specific but I believe that for neurologists this was really great."

Delegate perception regarding the overall impression of the masterclass programme.

79% of delegates rated the Masterclass programme as 'Excellent', 21% as 'Good' and 1% as average.

When delegates were asked via the survey as to what other sessions they would have liked to have seen from the programme, 50 responses were recorded with varying results. No key items were repeatedly reported but some suggestions related to the theme of Physiotherapy in the sense that delegates would like to see more: sessions on orthotics, exercise specific lectures, more case studies similar to those presented in the physiotherapy workshop, detailed advice on exercise programmes for physical therapy, respiratory physiotherapy, specific outcome measures for physiotherapy and when/how to use them and more time for practical management and physiotherapy equipment.

Other comments included more interactive care presentations and clinical case scenarios and potentially more patient perspectives sessions.

Delegate perception of the event organisation.

100% of surveyed delegates indicated that the event was either ‘Very Well’ or ‘Well’ organised.

When asked how to improve organisation of future events, 55 responses were received but no repeating trends. Some feedback suggested that Whova was a little overwhelming to use at first however, 98% of delegates on Whova indicated they found Whova useful.

Some comments and points for consideration at future events suggested that it would be good to:

- have extended access to content online.*
- be able to view speaker recordings after the event.*
- take frequent breaks and perhaps more time for a lunch break.*
- be able to attend both workshops and not pick one of the parallel workshops.*
- include more multiple-choice question polls during sessions to keep the audience engaged.*
- avoid overlap of some content in sessions.*

Delegate perception on most useful/best element and least useful elements of the masterclass.

107 delegates answered what they found as the most useful element.

57 delegates answered what they found the least useful element of the masterclass.

Full answers can be found in Appendix 2.

Most Useful/best element

The key positive trends from the survey feedback relate to:

- Workshops and discussions of clinical cases
- Quality of expert speakers
- Sessions relating to MRI & Biopsy

“The clinical cases workshop was brilliant at getting you to think through differentials.”

“The workshop with clinical cases. Really liked the interaction and trying to apply what we have learned over these two days.”

“I really enjoyed the information about orthopaedics (Physio workshop) because it was new to me. I also consider it really smart that you provided those pre-recorded sessions so we can revise and prepare ourselves to enjoy the presentations more. Also, it was great when Dr Giorgio Tasca, as part of a workshop, enabled us to describe the MRI and biopsy findings in our own words, it is, in my opinion, the best way to learn.”

“Expert lecturers; up to date information.”

“Bringing in experts in the field. I felt also that there was a good interaction between speakers and audience.”

Least useful elements of the Masterclass

This feedback was very mixed, but some comments did highlight the following trends:

- some information or sessions were rushed
- content was not relevant or catered to all job roles
- registries workshop was least useful to some

Some survey comments included the following:

“On the first day, two speakers talked very rapidly making it difficult to follow their presentation.”

“As I work in paediatrics, some of the presentations on LGMD that present only in adults were difficult to keep up with but completely understandable with the target audience of the masterclass.”

“Heavy genetic based talks around the different subtypes were hard to follow. Appreciate as a physiotherapist they are less relevant for me.”

“The information about the registries, although it was important.”

Delegate perception on using Whova to promote networking and a virtual masterclass experience.

59 surveyed delegates provided their feedback on the platform used. To prepare delegates to use Whova, information about how to download and access the platform was published in the Delegate booklet alongside detail about how to access interpretation services. A 10-minute video webinar was later recorded and made available to delegates (specifically, those who had not downloaded the app in order to encourage download rates.) This webinar provided a visual overview of Whova features and functionality online and detail about accessing interpretation services.

To improve the user experience of the Whova app, TREAT-NMD also provided several ‘Delegate Online Drop-In’ sessions of which, members of the Education team were available to assist delegates with logging in and delegates had the opportunity to ask any questions about Whova and test the platform. The take up rate of this service was poorly received with only 3 delegates logging in to attend.

The table below provides some comments about delegates experiences across the board.

What went well with Whova	What did not go well with Whova
<p><i>“Whova was a very good platform. It was actually the best I’ve seen for virtual meetings.”</i></p> <p><i>“It worked very well and interactive sessions were impressive. Well-co-ordinated and ambitious!”</i></p> <p><i>“Networking has been successfully promoted.”</i></p> <p><i>“I really liked the virtual experience.”</i></p>	<p>“Less emails maybe (maybe I could have switched that off?)”</p> <p>“The presentations didn’t work correctly on my laptop, so I needed to watch on my phone – this was difficult over a 2-day period when also trying to make notes. Ensuring presentations worked correctly on the webpage on a laptop would be really useful.”</p> <p>“The slides froze at times, as did the videos in real-time. On-site masterclasses are the best!”</p> <p>“The final link didn’t work and I’m not sure if that was my error. It was also not possible to use the translation function on the desktop and I could only use this on the app.”</p> <p>“Potentially use break out rooms as a dedicated session in the agenda so people have a greater chance to network with others.”</p>

Overall delegate perception of the masterclass.

82% rated the masterclass as ‘Excellent’,

107 delegates recorded an answer to this survey question. Delegates were able to rate the event as either Excellent, Good, Fair or Poor. 17% rated the masterclass as ‘Good’ and 1% as ‘fair’.

Overall Rating of the Speakers and Education Materials

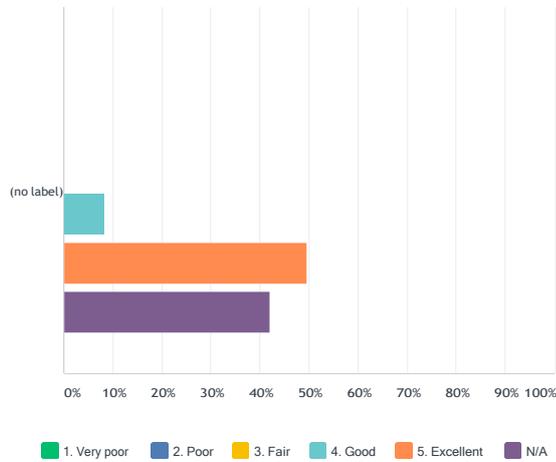
The next section provides detail from Whova and Delegate survey feedback relating to the quality of Masterclass Speaker sessions, workshops, and video watch rates.

Pre-recorded Video Views in Ranking order of most viewed on Whova:	
LGMD Overview, Definitions & Classifications	135
Natural History of most frequent LGMDs	96
Clinical Trials & Emerging Therapies	93
Frequency & Distribution of LGMDs across the world	93
Best practice for care management: Orthopaedics	70

Ranking order of delegate survey feedback regarding the quality of the Live Expert Speaker Sessions.		
Theme	Speaker	Weighted average score (out of 5 points)
Best practice - Respiratory	Stephen Wenninger	4.69
Uses and applications of muscle MRI in LGMDs	Giorgio Tasca	4.64
Diagnostic journey of the LGMD patient	Carmen Paradas	4.63
LGMD Phenotypes caused by Titinopathies and the dominant LGMDs	Bjarne Udd	4.63
Best practice - cardiological	Karim Wahbi	4.56
Muscle Biopsy as a tool to diagnose patients with LGMD	Montse Olive	4.50
Most frequent recessive forms: DYSF, ANO5, SGC	Elena Pegoraro	4.48
Benefits and limitations of NGS in NMD	Ana Topf	4.47
Registries	Michela Guglieri	4.41
Frequent recessive forms IICAPN3 and FKRP and dystroglycanopathies	Adolfo Lopez De Munain	4.30

Ranking order by delegate feedback of workshop quality.

Exemplary Clinical Cases. Score of 4.85 (weighted average out of 5)



	1. VERY POOR	2. POOR	3. FAIR	4. GOOD	5. EXCELLENT	N/A	TOTAL	WEIGHTED AVERAGE
(no label)	0.00%	0.00%	0.00%	8.41%	49.53%	42.06%	107	4.8
	0	0	0	9	53	45		

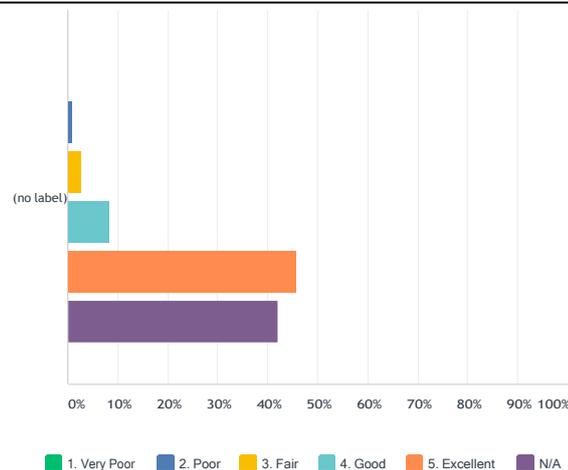
Delegate comments about the Exemplary Clinical Cases workshop:

"I particularly liked the discussion on critical cases and how the speakers run through the various differentials and phenotypes."

"The clinical cases workshop was brilliant at getting you to think through differentials."

"The workshop with clinical cases. Really liked the interaction and try to apply what we have learned these two days."

Physiotherapy and other clinical care considerations. Ranked 4.71 (out of 5)



	1. VERY POOR	2. POOR	3. FAIR	4. GOOD	5. EXCELLENT	N/A	TOTAL	WEIGHTED AVERAGE
(no label)	0.00%	0.93%	2.80%	8.41%	45.79%	42.06%	107	4.7
	0	1	3	9	49	45		

Delegate comments about the Physiotherapy and other clinical care consideration include:

"The workshop and the physio cases were really useful to get you thinking what could be applied to appointments with patients."

“The Physiotherapy workshop {was the most useful for} applying examples to practice, case studies and useful links/tips in treatment/MDT assessment etc.”

“The Orthopaedic and rehabilitation management {workshop was the most useful as it was} useful for the clinician to know and try to prevent complications related to pathology/hypermobility.”

Delegate perception of Commercial bias.

As part of the accreditation board standards, a question about delegate’s perception of bias was mandatory as part of the event survey. The table below indicated the outcome from 107 responses. Only 3 delegates recorded negative or uncertain answers and would be considered as outliers in this data.

Q) Do you agree that the information overall was free of commercial bias?	
Strongly Agree	71%
Agree	26%
Disagree	1%
Strongly Disagree	1%
Not sure/Do not know	1%

Delegates Formal Reviews of the Masterclass (in publication)

The next two items of feedback are masterclass reviews written by delegates from whom permission was obtained for TREAT-NMD to publish in the members newsletters to promote future masterclasses.

“In April 2021 I attended the virtual TREAT-NMD LGMD European Expert Masterclass. At a time when the COVID 19 pandemic has grounded thousands of planes, it was fascinating to go on an online tour around the world (with the sights and sounds of Spain!) to learn about the updates in the fascinating field of genetic neuromuscular disorders.

The Masterclass began by covering the impact of LGMD on patient's lives. These opening sessions were presented by both patients and parents of patients living with LGMD. Their stories really hit home and allowed us insight into the practical challenges patients face each day. This led nicely into the clinical lectures where the subspecialty ‘best practice’ series of live talks regarding cardiological and respiratory care management were all very practical, nicely presented and emphasized the role of the neuromuscular clinician in coordinating the care of these patients. There was also a live talk about the role of Muscle Biopsy in the diagnostic process of LGMD which was very interesting. We were asked which lecture we found most enjoyable, and being a clinician, I mostly enjoyed the lecture presented by Dr Giorgio Tasca about the uses and applications of MRI in diagnosis of LGMD. MRI is likely to play a central role in the diagnosis of LGMDs as more and more patterns are being realized in different genetic forms of LGMD, so this talk was particularly interesting for me.

What was also interesting was the opportunity, after each live presentation, for a dedicated 'Question & Answer' session where delegates were able to post live, moderated questions to experts and these questions were discussed and answered during the masterclass which was an added bonus.

I would like to congratulate the Masterclass co-chairs, Dr Michela Guglieri and Professor Jordi Diaz-Manera, on such an impressive event. This Masterclass has provided me with an enhanced understanding of LGMD and broadened my horizons and was overall a very rich experience. I highly recommend the LGMD Masterclass to any future neuromuscular trainees as it brings together superb clinicians in neuromuscular diseases with a wealth of expertise in the field."

Dr Maha Elseed, Specialist Doctor in Clinical Neuromuscular Diseases from the John Walton Muscular Dystrophy Research Centre, UK.

"It can be challenging to find the latest knowledge on the topic of muscular dystrophies and as a physiotherapist, I am often relying on my own experience and that of my colleagues. Since there are not so many patients with LGMD disease, and every case is different, it is challenging to capture the whole picture of the patient's symptoms and keep up to date with current, evidence based, practice. Therefore, it was a great pleasure to take part in the TREAT-NMD LGMD Masterclass in April 2021 and a unique opportunity to hear live talks from LGMD patients and expert physiotherapists, doctors, and professors. Also, the opportunity to ask live, moderated questions to expert speakers, and have them answered on the spot by them was exceptional.

It is now crucial to put into practice the knowledge gained from attending the Masterclass event - and there is a lot of knowledge! Fortunately, the available pre-recorded and post-event video recordings make it easy to assimilate and revisit this knowledge.

It was mentioned during the event that no one person in the world can manage dystrophy treatment alone; we need cooperation. In my opinion, the Masterclass is perfect proof that cooperation is possible across the world, and I would recommend anyone who comes in to contact with people living with LGMD to attend this Masterclass."

Joanna Tokarska, Physiotherapist from the Polish Chamber of Physiotherapists.

Achievement of Masterclass Objectives

As detailed previously the LGMD Masterclass Objectives were as follows:

Objectives:

- Deliver a 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.

It is important to review whether these objectives were achieved.

Given the high delegate satisfaction scores regarding the Masterclass programme, which included specific sessions detailing the diagnosis, natural history, clinical trials, and emerging therapies, the first and last objectives above appear to have been met. The average weighted scores where delegates have rated the quality of speaker sessions indicates that overall, sessions were useful to delegates and were of good quality. There are no recorded results in which session themes or speakers were rated with an overall majority rating of 'poor' or other negative score.

Delegate feedback around the use of 'discussion' of clinical cases during workshops is identified as being one of the most useful/best elements of the masterclass and indicates that delegates agree that opportunities have been embedded into the programme to provide them with a platform to share their ideas, challenges, and successes. The Whova platform, and overall satisfaction with this digital platform, indicates that this has been used to encourage interaction and networking opportunities between delegates and speakers and as a platform for Q&A dialogues to take place. This indicates that the objective to create an interactive platform for healthcare providers has been met.

Delegate surveys and comments are largely positive. Some issues reported in the comments may not be within the control of TREAT-NMD as they relate to individual circumstances regarding technology however, there are some meaningful comments that should be considered and explored for future workshops such as more time for breaks during the event as well as reviewing the need for possibly more physiotherapy content across the 2 days.

The next section captures the positives of this masterclass and how some suggestions/improvements could be considered for future events.

Masterclass Reflection and Lessons Learned - PMI

Positives	Minus	Interesting
<ul style="list-style-type: none"> -Largest attendance rate at a Masterclass to date. -Smaller than anticipated drop-out rate from expected confirmed attendees. -Good retention rate of delegates across Day 1 and Day 2. -Good return rate of delegate surveys. -Interpretation features can be implemented on Zoom & can therefore widen the reach of audience. -EACCME accreditation application process was efficient and provides added value to delegates. -High rates of delegate satisfaction relating to event quality and organisation. 	<ul style="list-style-type: none"> -Not all applicants were offered a place due to high interest in the event and several applications were from the same institution providing smaller geographical spread within a country. -Large proportion of applications were made by UK hospitals/universities and may be due to small geographical reach of networking and promotional activities. -Poor take-up rates of 'Delegate Online Drop-In' sessions for delegates to test their Whoova connection and contact the TREAT-NMD Education Team. -Some delegates mentioned an overlap in some content across day 1 of the event programme. 	<ul style="list-style-type: none"> -Interest in attending LGMD Masterclasses has been received from across Australasia/Asia and African regions and could be worth exploring for future masterclass locations. -Some application forms were received within a 24-hour period after the weblinks had been removed from the TREAT-NMD website. This could be due to previously promoted links being copied and circulated amongst external networks or a delay in the technology in closing the link. - Some Physiotherapist feedback requests that more Masterclass content is targeted towards them outside of the Workshops.
<p>Action Points & Areas for improvement</p> <ul style="list-style-type: none"> -Consider increasing target audience size for future events if greater interest from suitable candidates is expressed. -Ensure programme session themes are planned clearly and relayed to expert speakers to minimise overlaps in content/topics (where feasible). -Shorten application timeframes or close applications earlier to limit the number of 'reserve' delegates. 		

-Collate a promotions database of useful organisations and contacts around the world in order to widen the reach of masterclass promotions where possible and attract a wider spread of applications across different affiliations.

-Consider longer or more frequent breaks during Masterclasses to improve delegate comfort and engagement levels.

-Make a copy of the TREAT-NMD Whova webinar video available to all delegates at the beginning of delegate communications so that all delegates receive this guidance on understanding the platform better.

-Promote the Delegate Online Drop-In sessions at an earlier stage to delegates explaining the benefits of attending to improve take-up. Continue to monitor value and attendance of this provision.

-Review the applications received from Latin American delegates and invite suitable applicants to attend the LGMD Latin America Masterclass in November 2021.

Thanks to our Sponsors.

We would like to take this opportunity to thank our funding partners for supporting the development and delivery of this Masterclass. We are grateful to Sarepta Inc, Edgewise Therapeutics, ML Bio Solutions and the Jain Foundation. Without their generosity this successful event would not have been possible.

Appendix

Appendix 1

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
35 minutes	LGMD Overview, Definitions & Classification	Dr J. Andoni Urtizberea
30 minutes	Frequency and distribution of LGMDs across the world. Insights from the NGS screening focus in LATAM. Recently published papers in USA	Dr Giacomo Comi
40 minutes	Clinical trials landscape & emerging therapies	Prof. Jordi Diaz Manera
60 minutes	Natural history of the most frequent LGMDs FKRP and CAPN3 Dysferlinopathies	Dr John Vissing Dr Meredith James This pre-recorded webinar will be jointly delivered by both speakers
30 minutes	Best practice for care management Orthopaedics Care	Dr Linda Lowes Dr Meredith James This pre-recorded webinar will be jointly delivered by both speakers

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 29TH APRIL 2021				
DURATION	BST	CEST	PROGRAMME SESSION	SPEAKER DETAILS
5 minutes	09:00-09:05	10:00-10:05	Welcome, programme overview and introduction to TREAT-NMD	Professor Jordi Diaz Manera Dr Michela Guglieri

25 minutes	09:05 – 09:15	10:05 – 10:15	Patient perspectives <ul style="list-style-type: none"> Impact of LGMD on their lives and journey 	Luis Garcia Pareras
	09:15 – 09:25	10:15 – 10:25	Patient perspectives <ul style="list-style-type: none"> Impact of LGMD on their lives and journey 	Jéssica Martín
	09:25-09:30	10:25-10:30	Questions and Answers	The speakers will answer a selection of moderated questions
30 minutes	09:30-09:55	10:30-10:55	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) 	Dr Carmen Paradas
	09:55-10:00	10:55-11:00	Questions and Answers	The speakers will answer a selection of moderated questions
30 minutes	10:00-10:25	11:00-11:25	Most frequent recessive forms: DYSF, ANO5 and SGCA <ul style="list-style-type: none"> Including an introduction to Pathophysiology 	Dr Elena Pegoraro
	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	
30 minutes	10:45-11:10	11:45-12:10	Most frequent recessive forms II CAPN3 and FKRP and dystroglycanopathies <ul style="list-style-type: none"> Including an introduction to Pathophysiology 	Dr. Adolfo Lopez de Munain
	11:10-11:15	12:10-12:15	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	11:15-11:40	12:15-12:40	LGMD phenotypes caused by titinopathies and the dominant LGMDs <ul style="list-style-type: none"> Including an introduction to Pathophysiology 	Dr Bjarne Udd
	11:40-11:45	12:40-12:45	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	11:45-12:10	12:45-13:10	Muscle biopsy as a tool to diagnose patients with LGMD	Dr Montse Olive

	12:10-12:15	13:10-13:15	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	12:15-12:40	13:15-13:40	Uses and applications of MRI in LGMD's	Dr Giorgio Tasca
	12:40-12:45	13:40-13:45	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	BST	CET	PROGRAMME SESSION	SPEAKER
1 minute	09:00-09:01	10:00 – 10:01	Welcome to Day 2.	Dr Michela Guglieri Professor Jordi Diaz Manera
29 minutes	09:01-09:25	10:01-10:25	Best practice for care management - Cardiological	Dr Karim Wahbi
	09:25-09:30	10:25-10:30	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	09:30-09:55	10:30-10:55	Best practice for care management - Respiratory	Dr Stephan Wenninger
	09:55-10:00	10:55-11:00	Questions and Answers	The speaker will answer a selection of moderated questions
30 minutes	10:00-10:25	11:00-11:25	Registries. 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	

WE HAVE ARRANGED TWO INTERACTIVE WORKSHOPS; THESE WORKSHOPS WILL BE DELIVERED CONCURRENTLY. WHERE POSSIBLE DELEGATES WILL BE ASSIGNED ONTO A WORKSHOP OF THEIR CHOICE.

3 hours	10:45 – 13:45	11:45 – 14:45	<p style="text-align: center;">Workshop 1</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 Mayhew • 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;">Workshop 2</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 <ul style="list-style-type: none"> • Demonstration and group discussions around 4 clinical cases. Group interaction, feedback, Q&A
5 Minutes	13:45 – 13:50	14:45 – 14:50	Masterclass close and sum up	Professor Jordi Diaz Manera Dr Michela Guglieri

Appendix 2

Full Delegate Survey Results can be found in the Adobe document link below.

<https://documentcloud.adobe.com/link/track?uri=urn:aaid:scds:US:c161fa0d-9cbe-48e0-b22b-e1a8fc6249e4>