



TREAT-NMD DMD Expert Masterclass Post Event Report 2021

The main funding to allow the DMD masterclass to take place was provided by **Sarepta Therapeutics**, with additional funding provided by **PTC**. We are grateful to funders for their support, without which this masterclass would not have been possible. Please note: Funders were not involved in any way in determining the content of the masterclass, the speaker selection or the delegates application review process. TREAT-NMD is grateful for all financial support received.

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TREAT-NMD DMD Expert Masterclass 2021

Introduction and Overview

Introduction

The TREAT-NMD DMD Expert Masterclass 2021 was originally planned to take place in Sao Paulo, Brazil in May 2020. Due to the impact of the COVID 19 pandemic, the event was postponed and changed to a live online masterclass which took place on 5th and 6th May 2021.

The location for this masterclass was decided by the TEC (TREAT-NMD Education Committee), who recognised the need for education on DMD in South America and utilised contacts such as Dr Arnaldo Godoy and Edmar Zanoteli to set up this collaboration. The TEC also set the objectives, designed the programme and nominated speakers to present at the masterclass.

Overview

Following the success of a previous masterclass series from 2015 to 2019, the 2021 TREAT-NMD Expert Masterclass on Duchenne Muscular Dystrophy (DMD) was held as a live online event on 5th and 6th May. The focus was to encourage delegates from South America to attend. The event provided high-quality information regarding diagnosis of DMD, genetics of DMD, the latest standards of care as well as information concerning therapies for DMD. There were also sessions which focussed on patient and parent perspectives, which added an extra dimension to the event as attendees gathered experience from both a clinical and a patient and family perspective.

The masterclass opened with co-chairs Annemieke Aartsma-Rus and Arnaldo Godoy introducing the event and its aims, followed by the patient and parent perspectives. The patient and parent presentations were particularly striking as the speakers outlined the impact of COVID 19 on their lives and care and gave an opportunity for questions from the audience about patient care in Brazil. What followed was a range of presentations and Q&A sessions from a panel of speakers who are leading experts in the field of DMD (the list of speakers is included below). These presentations were further supported by interactive breakout sessions which allowed delegates to discuss more sensitive and specific issues relating to standards of care, steroids and emerging therapies. The breakout sessions were an excellent platform to promote more in-depth discussion between colleagues from different centres, countries and professional disciplines where people could share experiences, discuss barriers and possible solutions.

The use of the Whova platform & app provided an excellent arena for delegates to post questions for each session speaker, to connect with other delegates and to share knowledge and ideas. The breakdown of data and usage is discussed later in this document.

The Masterclass Objectives

Masterclass Objectives

1. Provide a high quality educational and interactive opportunity for health care providers diagnosing or managing the care of patients with Duchenne muscular dystrophy (DMD) through information and discussion on the latest in diagnostic pathways, natural history, and internationally recognized standards of care (SoC).
2. Provide a platform for participants to share ideas, challenges, and successes in delivering optimal care for people with DMD.
3. Provide an overview of the current clinical trial landscape and the emerging implications for care and therapies.

Masterclass Programme and Speakers

To see the full programme, see appendix 1 at the end of this document.

For further information on speakers, please refer to the delegate booklet which can be requested by emailing: education@treat-nmd.com

Speakers

Co-Chairs:

- **Annemieke Aartsma-Rus**, Leiden University Medical Center, The Netherlands
- **Arnaldo Godoy**, Sao Caetano do Sul University, Brazil

Speakers:

- **Jose Canto**, Father of a child living with DMD, Brazil
- **Jose Corderi**, Favalaro Foundation, Argentina
- **Michela Guglieri**, John Walton Muscular Dystrophy Research Centre, Newcastle University, UK
- **Juliana Gurgel Gianetti**, Universidade Federal de Minas Gerais, Brazil
- **Anri Human**, Sefako Makgatho Health Sciences University, Pretoria, South Africa
- **Rafael Lellis**, DMD Patient living in Brazil
- **Gustavo Mendes**, General Manager of Medicines of the Brazilian Regulatory Agency (ANVISA).
- **Alexandra Prufer de Queiroz Campos Araújo**, Universidade Federal do Rio de Janeiro, Brazil
- **Edna Pupin**, Mother of an adult living with DMD, Brazil
- **Ros Quinlivan**, MRC Centre for Neuromuscular Disease, London, UK
- **David J Schonfeld**, Children's Hospital Los Angeles, USA
- **Volker Straub**, John Walton Muscular Dystrophy Research Centre, Newcastle University, UK
- **Karim Wahbi**, University of Paris Descartes, France
- **Edmar Zanoteli**, Faculdade de Medicina da Universidade de São Paulo, Brazil

Achieving the Objectives 1

1. Objective: *Provide a high quality educational and interactive opportunity for health care providers diagnosing or managing the care of patients with Duchenne muscular dystrophy (DMD) through information and discussion on the latest in diagnostic pathways, natural history, and internationally recognized standards of care (SoC).*

The TEC (TREAT-NMD Education Committee) agreed on selection criteria which has been applied for all masterclasses to date. As part of the application form, questions are phrased so that applicants can describe how they meet such criteria. Criteria such as: occupation (open to healthcare professionals who work in the diagnosis and care of DMD patients), affiliation, country, amount of patient contact and how the information will be disseminated to a wider team in their organisation following the event. This information was examined on a case-by-case basis and candidates were selected using this information and applying the criteria. It is important to note that prior to the COVID-19 pandemic, only delegates applying from South American countries would have been eligible, but as the programme was switched to an online event, applications were open to those who met all other criteria regardless of location.

Delegate Data and Analysis

Overall Application Data

Category	Number
Total number of applications received	151
Number of eligible applications	128
Number of ineligible applications	23

151 applications were received, which is very positive and to have 128 that were eligible is an excellent number considering in 2019 for a face-to-face masterclass there were 60 places available and for an online meeting we were able to offer all 128 a place.

The 23 ineligible applications were rejected on the ground that these were not healthcare professionals. Even though the audience is clearly stated on every newsletter article, on the website and on the application form, we still receive applications from a range of people who are not clinicians. These applications came from: parents, laboratory researchers, herbalists, journalists, marketing directors and patient organisations.

Attendance Data

Category	Number	Percentage
Number of eligible applications	128	100%
Number of delegates in attendance day 1	96	75%
Number of delegates in attendance day 2	88	69%

From previous experience in running online programmes, we would estimate between 10-20% drop out rate, so 25% is slightly more than expected.

Following some investigation, whilst there were 98 logins to the masterclass, some attendees were in meeting rooms in groups working together, so the audience was in fact larger than 98 and 88 on both days.

As part of the masterclass process all applicants were contacted in the lead up to the DMD Masterclass with information about the dates and times and general logistics to boost numbers and ensure delegates had all the necessary information to attend. There were also 3 drop-in sessions on Whova in preparation for the event so that delegates could login, test the app and meet the organisers to ask any questions. Out of 128 delegates only 1 attended a drop-in session.

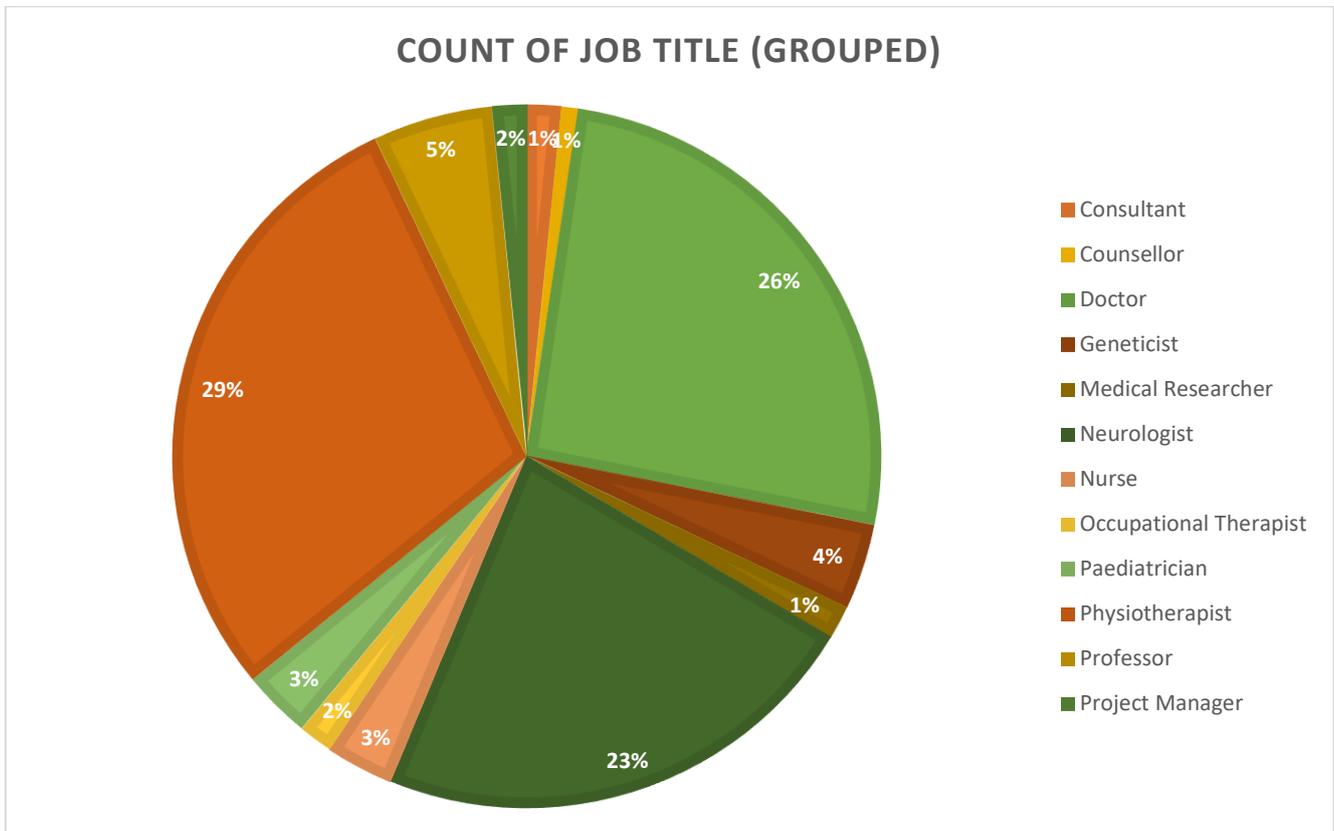
Research into those delegates who did not attend at all, and the drop from 96 to 88 on day two revealed several reasons behind these numbers. These are outlined below.

- Delegates stated that the time difference meant that they could not attend
- Delegates stated that due to COVID 19 they were working extra
- Delegates were under the impression that they would be able to watch all the content at their leisure (even though it is explained that this is a live and interactive masterclass in all of the documentation).
- Delegates stated that poor wi-fi connection or technology issues prevented them from attending

On reflection, and to prepare better for future masterclass events, we will:

- Examine the time differences more closely and communicate this to individuals
- Include in the acceptance email clear information on logistics and technology requirements
- Accredite the masterclasses so that delegates earn CME credits for each session they attend.

Application Breakdown by Occupation



Row Labels	Count of Job Title (Grouped)	% of Total
Consultant	2	2%
Counsellor	1	1%
Doctor	33	26%
Geneticist	5	4%
Medical Researcher	2	2%
Neurologist	29	23%
Nurse	4	3%
Occupational Therapist	2	2%
Paediatrician	4	3%
Physiotherapist	37	29%
Professor	7	5%
Project Manager	2	2%
Grand Total	128	100%

The occupation spread here is interesting. The Medical Researchers and Project Managers were all heavily involved in either patient registries and in emerging treatments for patients and were granted places on this basis. There was a large group of physiotherapists, and they were highly active on Whova and set up their own meeting to network and share expertise beyond the realms of the Masterclass.

With this, next time we should consider doing something similar to the LGMD format and run parallel workshops and have one which focusses on physiotherapist's needs and one on clinical cases. This was mentioned as a possible improvement by several delegates in the feedback in question 4 which was concerned with how to improve the programme.

Feedback from Delegate Survey Question 4: What would you also like to have seen included in the agenda?

Responses:

'Occupational therapy and physiotherapy strategies we can use with limited resources (creative alternatives).'

'PT session or PT specific'

'Clinical trials in countries of low income and clinical cases'

'Clinical cases discussion'

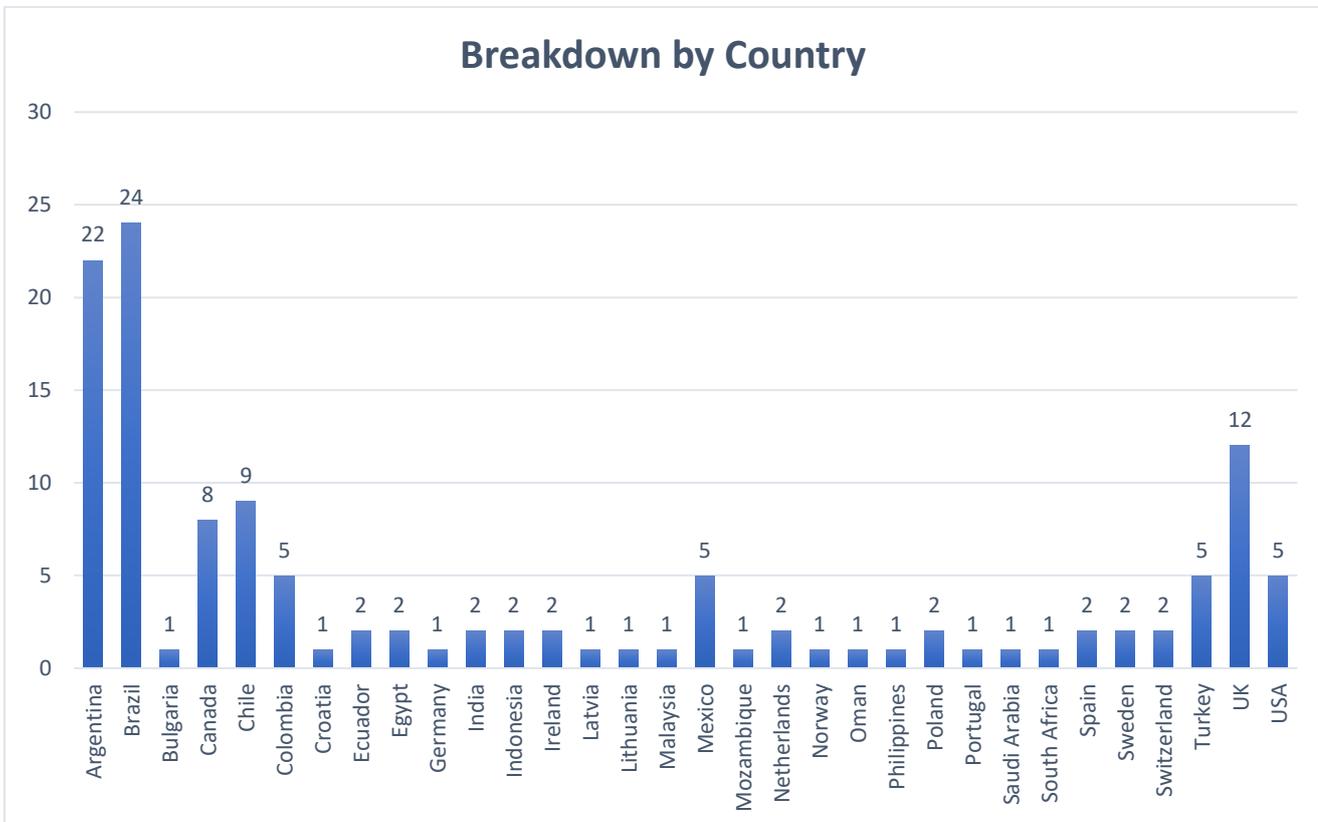
'I would include Physiotherapy assessment'

'More workshops, specially about physical Therapy'

'Physiotherapy treatment and functional assessment scales'

'Discussion of clinical cases and genetic reports'

Application Breakdown by Country



The eligible applications from South America make up the largest proportion of applications totalling 67 out of 128 or 52%. This is because this was the target area when the event was first planned as a face-to-face event. The application range was extended to other regions following the event changing to online delivery.

The UK and Canada are those countries with the second largest presence. TREAT-NMD has a strong presence in these areas which explains these larger numbers. We promoted the event through our contacts in a variety of European countries. Whilst these other areas were not the focus of this event, perhaps there is still some work to do in terms of reaching audiences in some areas of Europe and Asia.

Achieving the Objectives 2

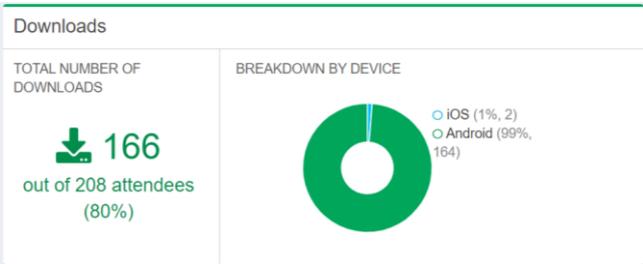
- Objective: *Provide a platform for participants to share ideas, challenges, and successes in delivering optimal care for people with DMD.*

Whova was the platform which was used to deliver the live online masterclass. We have used Whova for masgterclasses in the past and it has proven effective as it integrates with Zoom very well. For this masterclass it was important for us to cater to the needs of clinicians in South America and to ensure that the language barrier was not an issue so that they could follow lectures given by English speakers and interact in breakout groups.

We achieved this by utilising the language interpretation channel on Zoom and employed interpreters to provide live simultaneous translation during the whole live online event. In addition, we connected with a translations company and had pre-recorded lectures subtitled in English and/or Portuguese and we had all delegate documentation translated. During the live online event, breakout rooms were divided by language and lead by members of the faculty and questions submitted by delegates on Whova and were moderated and translated by the chairs.

Each delegate was given access to Whova two weeks prior to the masterclass so that they could access the reading materials and the pre-recorded content. Whova also gives delegates the facility to post questions and comments about lectures/sessions before, during and after the event. The questions and comments are available for the whole audience to see along with any responses.

What was particularly striking about the use of Whova for this DMD Masterclass was the level to which it was adopted by delegates. The delegates were active on the app throughout the event asking questions, leaving comments, messaging one another and even organising networking events on Whova to discuss matters further. Below is a breakdown of some of the key data from Whova.

 <p>The screenshot shows a 'Downloads' dashboard. On the left, it states 'TOTAL NUMBER OF DOWNLOADS' with a green arrow icon and the number '166' in large green text, followed by 'out of 208 attendees (80%)'. On the right, under 'BREAKDOWN BY DEVICE', there is a donut chart. The legend indicates 'iOS (1%, 2)' in light blue and 'Android (99%, 164)' in green.</p>	<p>Including the TREAT-NMD staff and the speakers there were 208 attendee places allocated on Whova.</p> <p>166 (80%) downloaded the app which meant they could interact on the move and could access all of the content on their phones, which is what we recommend for the best experience.</p> <p>Those who did not download the app could only access through their desktop or laptop.</p>

<table border="1"> <thead> <tr> <th colspan="2">Networking Activities</th> </tr> </thead> <tbody> <tr> <td>TOTAL PROFILE VIEWS</td> <td>TOTAL MESSAGES SENT/RECEIVED</td> </tr> <tr> <td> 566</td> <td> 685</td> </tr> </tbody> </table>	Networking Activities		TOTAL PROFILE VIEWS	TOTAL MESSAGES SENT/RECEIVED	 566	 685	<p>On the day of the masterclass there were 96 delegates in attendance and 685 direct messages were sent during the two days.</p> <p>On average each of the 96 delegates sent 7 direct messages to connect with other delegates and speakers. Whilst this does not replace face-to-face networking, it does suggest that Whova gives delegates the space and capacity to connect with one another.</p>
Networking Activities							
TOTAL PROFILE VIEWS	TOTAL MESSAGES SENT/RECEIVED						
 566	 685						
<table border="1"> <thead> <tr> <th colspan="2">Event Community Board</th> </tr> </thead> <tbody> <tr> <td>NUMBER OF MESSAGES IN COMMUNITY</td> <td>NUMBER OF ATTENDEES ACTIVE IN COMMUNITY</td> </tr> <tr> <td> 677</td> <td> 80</td> </tr> </tbody> </table>	Event Community Board		NUMBER OF MESSAGES IN COMMUNITY	NUMBER OF ATTENDEES ACTIVE IN COMMUNITY	 677	 80	<p>The delegates were also very active on the community boards where there are open chat threads and discussion boards. Delegates started their own chat threads on a variety of topics including physiotherapy, equipment and recent articles to share.</p> <p>A total of 677 messages were posted across all of these chat threads throughout the DMD Masterclass.</p>
Event Community Board							
NUMBER OF MESSAGES IN COMMUNITY	NUMBER OF ATTENDEES ACTIVE IN COMMUNITY						
 677	 80						
<p style="text-align: center;">Number of Questions Asked</p> <p style="text-align: center;">119</p>	<p>Across the two days of the masterclass there were a total of 16 different sessions. Across all sessions a total of 119 questions were posted during the live lectures by the delegates to the speakers.</p> <p>These questions were answered live during the event by the speakers.</p>						
	<p>The feedback from delegates about the use of Whova for the DMD Masterclass has been overwhelmingly positive.</p> <p>Delegates commented that Whova gave them the opportunity to connect with people and to feel as if they were ‘part of something’.</p>						

<p>Love Whova</p> <hr/> <p>TOTAL NUMBER WHO REPORTED THEY FOUND WHOVA USEFUL</p> <p> 94% of your app users.</p>	<p>Perhaps this was due to the target region of Brazil and South American countries</p> <p>See section on 'Feedback from Delegates' for more details.</p>
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This was such a popular topic, several delegates mentioned this in the feedback survey in question 4; including other topics they would like to have seen included in the programme.

Feedback from Delegate Survey Question 4: Would you also have liked to see included in the agenda?

‘More on clinical trial DMD with sponsorship, therapy DMD with achievable price, safe, can be delivered’

‘Extra information on clinical trials in countries of low income.’

‘If a longer meeting, more on the different sub-specialities of SoC’

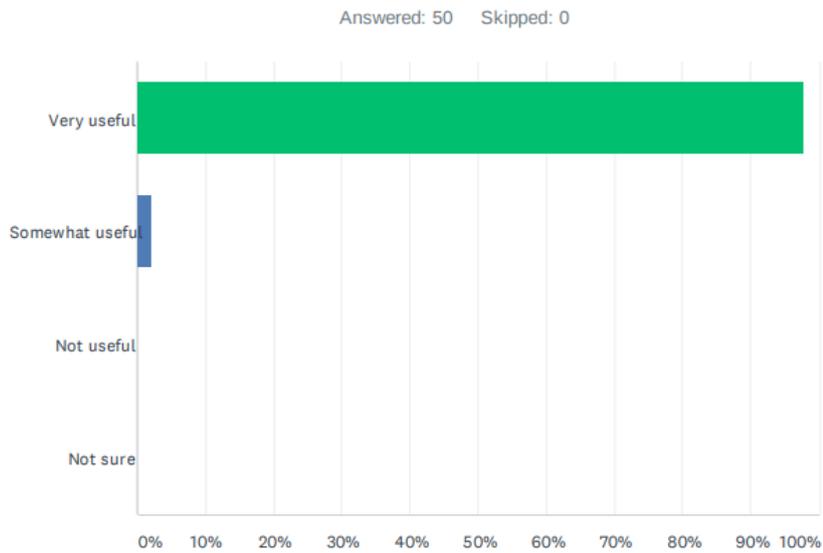
‘Just a whole session (more time) with questions and answer so everybody could learn from each other about standard of care’

Delegate Feedback Results

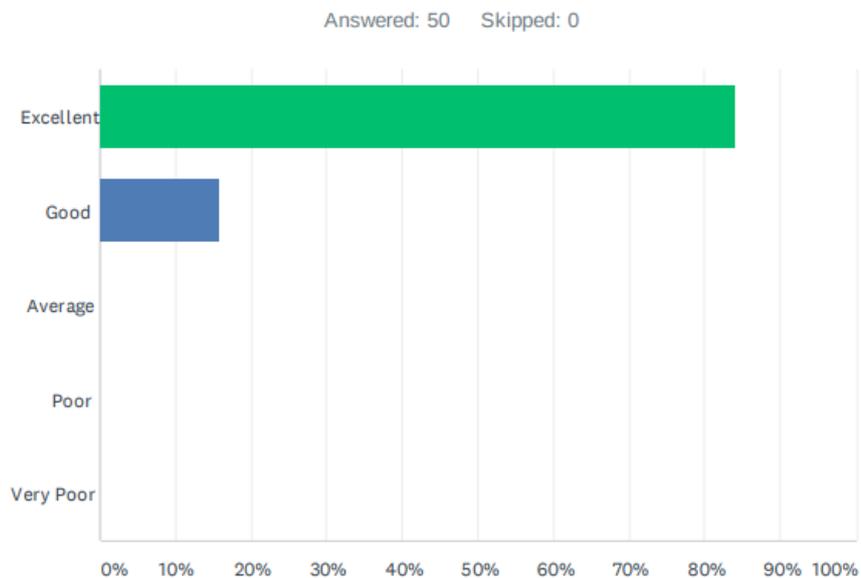
The Post Masterclass Delegate Survey Results

We collected feedback from delegates in the form of a survey at the end of the masterclass. The highlights of this survey are presented below.

Q1 How useful did you find the masterclass for your professional activity?

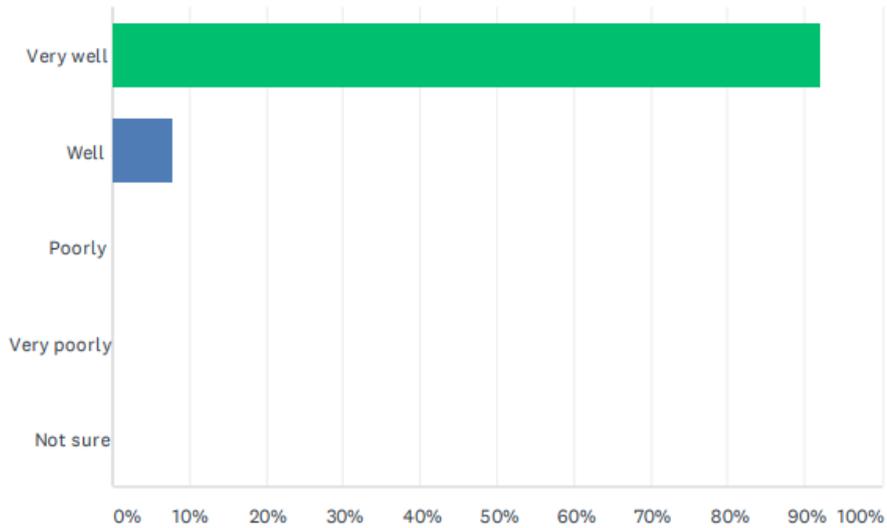


Q3 What was your overall impression of the masterclass agenda?



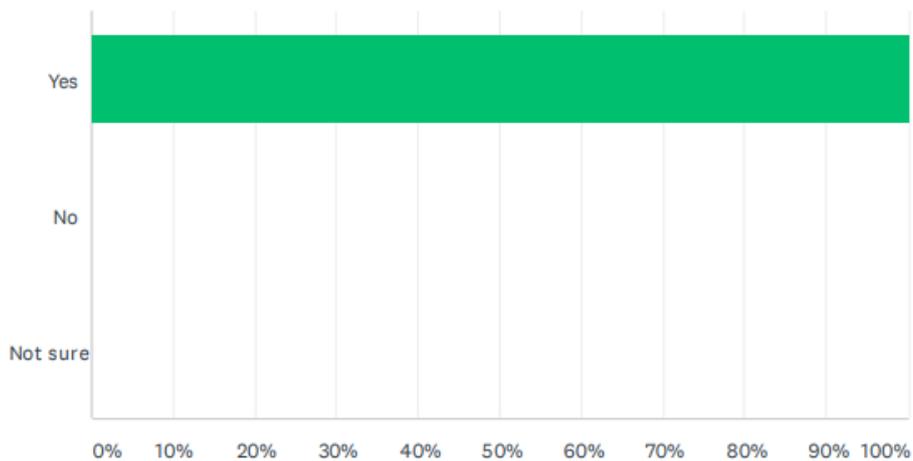
Q5 How well do you think the masterclass was organised?

Answered: 50 Skipped: 0



Q16 Would you recommend this workshop to a colleague?

Answered: 50 Skipped: 0



Full survey results are available here:

<https://documentcloud.adobe.com/link/track?uri=urn:aaid:scds:US:e3136d80-6aca-4312-b48e-f1c5303e6051>

Other Feedback and Comments

Some of the delegates chose to send direct messages at the end of the event to express their gratitude. These messages are displayed here.



It has been a very well organised event. Learnt a lot! Great platform to interact on. Congratulations from India!

Vineeth Jaison

Christian Medical College, India



Thanks for a fantastic Masterclass. It's truly amazing work.

Fatima Vido-Vecchio

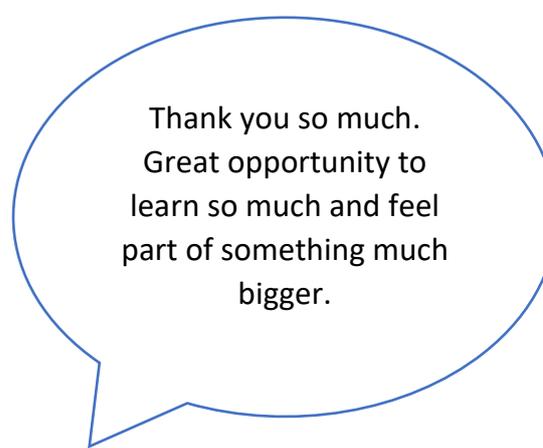
BC Children's Hospital, Canada



It has been a tremendous opportunity to meet other experts around the world. Thank you again.

Doug Levine

Cure Duchenne, USA



Thank you so much. Great opportunity to learn so much and feel part of something much bigger.

Paula Fenty

NHS Trust Nottingham UK

Other Feedback and Comments (continued)

Organizer Appreciation

APPRECIATION MESSAGES



ATTENDEES APPRECIATE YOUR EFFORTS



Lianne Solis
Hospital Pediátrico Juan Pablo II
Thank you very much!



Sandra Ramirez
Neuroimagenes. Clínica Sagrada Familia. Universidad Von Humbolth
thank you. Excellent event



Poorani Anandakrishnan
Clare, Thank you for organizing this event. You did a really good job:)



Camila Speltz Perussolo
Instituto de Neurologia de Curitiba
Clare, Thank you for organizing this event. You did a really good job:)



Ana Teresa Quissico
Healthy Ministry
its been great



Julian Murcia
Arcoiris S.A.
amazing!!



Vineeth Jaison
Christian Medical College, Ludhiana
It has been a very well organized event. Learnt a lot! Great platform to interact on. Congratulations from India!



Fatima Vido-Vecchio
BCCB
Thanks for a fantastic Masterclass. It's truly amazing work.



Paula Fenty
NHS
thank you so much. great opportunity to learn so much and feel part of something much bigger.

SWANS Analysis

Once the Masterclass was delivered, representatives from the TREAT-NMD organising committee undertook a SWANs Analysis

Strengths	Weaknesses	And Next Steps
<p>Strong programme which delegates appreciated</p> <p>Whova was received positively by delegates</p> <p>Whova numbers were very positive as delegates were keen to use this medium to connect with one another</p> <p>The simultaneous translation feature on Zoom and use of translators was very effective</p>	<p>Large number of delegates did not attend:</p> <p>Time zones (as we expanded the audience range)</p> <p>Some working due to COVID</p> <p>Difficult to judge true numbers as some people were online in groups</p>	<p>Ensure that next DMD event is EACCME Accredited to increase uptake</p> <p>Possibly use Whova even if face to face due to advantages for communication, article sharing and using as a platform to upload masterclass content for viewing post-masterclass</p> <p>Examine the time differences more closely and communicate this to individuals</p> <p>Include in the acceptance email clear information on logistics and technology requirements</p>

Appendix 1: Masterclass Programme

Expert Masterclass on Duchenne Muscular Dystrophy

5th and 6th May 2021, Virtual Event

Masterclass Programme

Masterclass Objectives

- Provide a high quality educational and interactive opportunity for health care providers diagnosing or managing the care of patients with Duchenne muscular dystrophy (DMD) through information and discussion on the latest in diagnostic pathways, natural history, and internationally recognized standards of care (SoC).
- Provide a platform for participants to share ideas, challenges, and successes in delivering optimal care for people with DMD.
- Provide an overview of the current clinical trial landscape and the emerging implications for care and therapies.

Masterclass Delivery Format

Due to the COVID-19 pandemic, was 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, with breakout sessions and opportunities for Q&A throughout. The content will match that which was planned for the face-to-face masterclass.

Day 1 is comprised of a range of lectures/talks from speakers with time allocated for questions and discussion. Questions will be posted on Whova (the masterclass app), moderated by the team and posed live to the speaker at the end of each presentation. There will be breakout sessions following some lectures to provide a space for discussion of certain topics.

Day 2 is comprised of a range of lectures/talks from speakers with time allocated for questions and discussion. Questions will be posted on Whova (the masterclass app), moderated by the team and posed live to the speaker at the end of each presentation. There will be breakout sessions following some lectures to provide a space for discussion of certain topics.

Chairs

Professor Annemieke Aartsma-Rus, Leiden University Medical Center, The Netherlands

Professor Arnaldo Godoy, Sao Caetano do Sul University, Brazil

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. It is vitally important and a condition of your place at the masterclass that these sessions are viewed in advance of the live meeting

PRE-RECORDED VIDEO – DURATRION	PROGRAMME SESSION	SPEAKER DETAILS
30 minutes	Introduction to DMD <ul style="list-style-type: none"> • Definitions • Variability of disease 	Edmar Zanoteli
30 minutes	Respiratory SoC	Anri Human
60 minutes	Physiotherapy and Orthopaedics SoC <ul style="list-style-type: none"> • Routine care and assessments • Adult care considerations • Emergency care • Performance of Upper Limb (PUL) and North Star assessments 	Jose Corderi
30 minutes	Adult Duchenne and Becker	Ros Quinlivan
30 minutes	Communication with parents	David Schonfeld
30 minutes	Cardiac SoC <ul style="list-style-type: none"> • Adults and carriers • Emergency 	Karim Wahbi

DMD Virtual Masterclass (Day 1)

- British Summer Time (BST) 17:00– 21:35 (4 hours 35 minutes)
- Sao Paulo, Brazil 13:00 –17:35

DAY 1 Wednesday 5 th May 2021				
DURATION	BST	Sao Paulo, Brazil	PROGRAMME SESSION	SPEAKER DETAILS
5 minutes	17:00 - 17:05	13:00- 13:05	Welcome, programme overview and introduction to TREAT-NMD	Annemieke Aartsma-Rus
3x 10 minute slots minutes	17:05- 17:15	13:05- 13:15	Patient Perspectives-(children) <ul style="list-style-type: none"> • Impact of DMD 	Jose Canto
	17:15- 17:25	13:15- 13:25	Patient Perspectives-(adult) <ul style="list-style-type: none"> • Impact of DMD 	Edna Pupin
	17:25- 17:35	13:25- 13:35	Patient / Parent perspectives <ul style="list-style-type: none"> • Impact of DMD 	Rafael Lellis
30 minutes	17:35- 18:05	13:35- 14:05	Diagnosis <ul style="list-style-type: none"> • Clinical presentation • Communication • Neonatal screening • Tools to aid raise awareness 	Michela Guglieri
10 minutes	18:05- 18:15	14:05- 14:15	Questions and Answers	The speakers will answer a selection of moderated questions posted on Whova by delegates
40 minutes (30 minutes in breakout rooms for discussion and 10 mins feedback in larger group)	18:15- 18:55	14:15- 14:55	Workshop on Diagnosis <ul style="list-style-type: none"> • Clinical Presentation (with examples) • Difficulties in Diagnosis 	Discussion Groups led by faculty members. Reading materials will be provided prior to the event so that delegates can prepare. The workshop will provide a chance for delegates to answer questions and discuss difficulties in diagnosing DMD patients.

25 minutes	18:55-19:20	14:55-15:20	Genetics: • The basics DMD vs BMD Mutations types and frequencies Genotype/phenotype Working mechanism for mutation specific therapies	Annemieke Aartsma-Rus
10 minutes	19:20-19:30	15:20-15:30	Questions and Answers	The speaker will answer a selection of moderated questions posted on Whova by delegates
15 minutes	19:30 – 19:45	15:30-15:45	BREAK	
30 minutes	19:45-20:15	15:45-16:15	Genetic diagnosis of DMD Reference document: https://www.ncbi.nlm.nih.gov/pubmed/26754139 <ul style="list-style-type: none"> • Diagnostic tools to detect DMD (MLPA, NGS, Sanger sequencing) • Interpreting diagnostic report • Need for sharing information • Quiz 	<ul style="list-style-type: none"> • Annemieke Aartsma-Rus
10 minutes	20:15-20:25	16:15-16:25	Questions and Answers	The speaker will answer a selection of moderated questions posted on Whova by delegates
25 minutes	20:25-20:50	16:25-16:50	Genetics in Brazil	Juliana Gurgel Gianetti
40 minutes	20:50 - 21:30	16:50-17:30	Genetics Discussion Groups Opportunity to discuss challenges you face (e.g. genetic tools not available, difficult to interpret genetic results, late age of diagnosis etc)	Breakout Groups led by Faculty to discuss issues surrounding genetics.
5 minutes	21:30-21:35	17:30-17:35	Close Day 1	Annemieke Aartsma-Rus

DMD Virtual Masterclass (Day 2)				
<ul style="list-style-type: none"> British Summer Time (BST) 17:00 – 21:35 (4hours 35 minutes) Sao Paulo, Brazil 13:00- 17:35 				
DAY 2				
Thursday 6th May 2021				
DURATION	GMT	Sao Paulo, Brazil	PROGRAMME SESSION	SPEAKER
5 minutes	17:00-17:05	13:00-13:05	Welcome to Day 2	Arnaldo Godoy
45 minutes	17:05-17:50	13:05-13:50	Standards of Care (SoC) <ul style="list-style-type: none"> Introduction to 2018 SoC Steroid & management of chronic treatment Emergency care 	Volker Straub
10 minutes	17:50-18:00	13:50-14:00	Question and Answer	The speaker will answer a selection of moderated questions posted on Whova by delegates
65 minutes	18:00-19:05	14:00-15:05	SoC and Steroids <ul style="list-style-type: none"> Implementation of SoC in Brazil (15 Mins) Use of steroids Discussion in breakout groups (15 mins) Reaching a consensus on SoC (15 mins) <ul style="list-style-type: none"> Methods to reach consensus in Brazil What are the issues? Discussion in breakout groups (20 mins)	Introduction given by Alexandra Prufer de Queiroz Campos Araújo Breakout discussion groups facilitated by faculty Arnaldo Godoy/Edmar Zanoteli

- Edna Pupin, Mother of an adult living with DMD, Brazil
- Ros Quinlivan, MRC Centre for Neuromuscular Disease, London, UK
- David J Schonfeld, Children's Hospital Los Angeles, USA
- Volker Straub, John Walton Muscular Dystrophy Research Centre, Newcastle University, UK
- Karim Wahbi, University of Paris Descartes, France
- Edmar Zanoteli, Faculdade de Medicina da Universidade de São Paulo, Brazil

Appendix 2: Post Masterclass Articles

Follow Up Articles:

The following articles were published following the DMD Masterclass 2021.

Published in the May 2021 Newsletter:

Double Masterclass Success!

It has been a busy few weeks at TREAT-NMD Headquarters as the Masterclasses have taken centre stage as not one, but two very successful TREAT-NMD Expert Masterclasses took place online this month.

First came the LGMD European Masterclass chaired by Prof Jordi Diaz-Manera and Dr Michela Guglieri. The event was delivered to just over 150 delegates from 37 different countries who tuned in online to hear expert speakers present a variety of topics inclusive of patient perspectives of LGMD, the most frequent recessive forms and the best practice for care management. The event rounded off with a choice of two interactive and engaging workshops for delegates to participate in relating to either Physiotherapy and other clinical care considerations or Exemplary Clinical Cases.

Then four days later the DMD Expert Masterclass took place online with the majority of participants from South American countries, but with others from Canada, India, Mozambique to name a few. The event was chaired by Prof Annemieke Aartsma-Rus and Prof Arnaldo Godoy and featured a range of lectures and discussion groups on a range of subjects including diagnosis, genetics and standards of care.

There will be more detailed reviews of the events from the participants themselves in the next newsletters, so watch out for those.

We would like to thank all of the participants and the speakers for creating such a great platform for learning, networking and sharing expertise. We are looking forward to the next in the series of TREAT-NMD events and all things education and with this in mind we need your input. There are some plans in the pipeline for a masterclass on the subject of gene therapy. We want to plan the agenda using your input. So, please fill in our survey here and let us know what you would like to see on the programme.

In addition to this, we are pleased to announce that there will be another LGMD Masterclass delivered to delegates from South American countries on Wednesday 3rd and Thursday 4th of November 2021. More information about this programme of events and when to apply will be released on the TREAT-NMD Website accordingly but for now, don't forget to save this date in your diary!

As always, if you have any questions about TREAT-NMD Masterclasses or any education projects, contact us at: education@treat-nmd.com

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TREAT-NMD Masterclass Success!

This piece was written by Fatima Vido-Vecchio following her attendance at the most recent DMD Masterclass. It details her experience at this masterclass and her journey since connecting with TREAT-NMD.

Fatima is a Physiotherapist working at BC Children's Hospital in Vancouver, Canada. She is originally from São Paulo, Brazil.

In May 2021 I had the pleasure of attending the virtual TREAT-NMD DMD Expert Masterclass. The event had been planned as a face-to face event due to take place in São Paulo, but like many other events this changed to virtual. We are all used to the world of online meetings and know what to expect, but this was no ordinary Zoom meeting!

This TREAT-NMD DMD Masterclass was a great success in many ways. There was the exceptional line-up of expert speakers delivering a range of sessions from diagnosis to genetics and standards of care to the all-important patient perspectives. This combined with the breakout groups where there was a chance for discussion, debate and networking the virtual masterclass struck the perfect balance. But what was particularly thrilling for me personally, as I am originally from Brazil and São Paulo to be exact, was to see so many people from Latin America joining forces with others from many other corners of the world to learn more about DMD.

Much of the focus of this masterclass was on patient perspective. The talks from each of the patients and parents of patients were inspirational in terms of the insight into life with DMD. I found these sessions incredibly helpful, particularly at this unusual time in which we are living with COVID-19 and we are adapting our practice. During the discussion sessions, I learned that in some circumstances the Pandemic has helped with access to services; some people who live far from specialized centres now have easier and more frequent access to services via virtual consultations.

There was also plenty of time and space for more discussion by using the masterclass platform and app Whova. This made it simple for us to connect, discuss and post questions to the speakers on any topic before, during and after the masterclass. We also had time to create a virtual DMD community-where we arranged a follow up focus meeting with other physiotherapists! All in all, a great success! But it doesn't end, or should I say start there for me.

I have had the honour of connecting with TREAT-NMD as a member and I have attended other masterclasses, in SMA and LGMD, in the past. These opportunities have contributed to my professional growth as well as the improvement of the quality of services to the families we serve at our hospital. Following each masterclass or TREAT-NMD event I have shared the resources and knowledge I have learned during these wonderful learning and networking opportunities. I learned how to best educate boys and their families about DMD. I was able to show community therapists how to access resources and how to spread their learning to their colleagues and co-workers.



Attending TREAT-NMD Masterclasses has given me the knowledge and confidence to apply for and be hired for the position of Physiotherapist at the NM Clinic at my hospital. Attending masterclasses also gave me confidence to present at our Paediatric Symposium and at Jesse's Journey Defeat Duchenne Family Forum on the topic of DMD and I became a member of the Advocacy Committee with the Muscular Dystrophy Canada. All because of attending TREAT-NMD Masterclasses! All that remains to say is a huge thank you to TREAT-NMD for the hard work and commitment in bringing us together.