



TREAT-NMD DMD Expert Masterclass Post Event Report 2019

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TREAT-NMD DMD Expert Masterclass

Post Event Report 2019

The TEC (TREAT-NMD Education Committee) set the objectives for the DMD Expert Masterclass, these are listed below. Many of these objectives were met through the programme and titles on which speakers presented and others were met during the course of the event and are examined in this report.

Meeting Objectives

- Provide a high-quality educational and interactive meeting for paediatric neurologists and other related specialities who may be diagnosing and/or managing the care of patients with Duchenne muscular dystrophy (DMD)
- Present the natural history of DMD and review the typical signs and symptoms of the disease
- Explain the genetics of DMD, and underline the importance of obtaining a mutation-specific diagnosis as early as possible
- Highlight the updated standards of care (2018) and discuss their implementation
- Give patient and parent perspectives on diagnosis, care and research in DMD

Day 1 – Thursday 16 May: Chairs: Annemieke Aartsma-Rus (am) and Elizabeth Vroom (pm)

| | | |
|-------------|--|-----------------------------------|
| 10:30-11:00 | Welcome coffee and registration | |
| 11:00–11:20 | Welcome and introduction to TREAT-NMD <ul style="list-style-type: none"> • Aims of the meeting | Annemieke Aartsma-Rus |
| 11:20-11:35 | Parent Perspective – Children <ul style="list-style-type: none"> • Behavioural issues and psychosocial impact | Simon van Boxtel |
| 11:35-12:00 | Parent Perspective – Adult <ul style="list-style-type: none"> • Experience in lower-income countries | Elizabeth Vroom |
| 12:00-12:15 | Introduction to DMD <ul style="list-style-type: none"> • Definitions • Variability of disease | Nathalie Goemans |
| 12:15–12:45 | Diagnosis <ul style="list-style-type: none"> • Clinical presentation • Communication • Neonatal screening • Tools to aid raised awareness | Michela Guglieri and Alex Johnson |
| 12:45–13:00 | Questions and Discussion | |
| 13:00–14:00 | Lunch | |
| 14:00–15:00 | Genetics <ul style="list-style-type: none"> • Explanation of genetics and mutation type Reference document: https://www.ncbi.nlm.nih.gov/pubmed/26754139 <ul style="list-style-type: none"> • Need for sharing information • Explanation of diagnostic tools (NGS) • Genotype phenotype • Mutation specific therapies (brief) • Quiz | Annemieke Aartsma-Rus |
| 15:00–15:30 | Standards of Care (SoC) <ul style="list-style-type: none"> • Introduction • Steroid & management of chronic treatment • Emergency care | Nathalie Goemans |

| | | |
|-------------|---|----------------------------------|
| | <ul style="list-style-type: none"> • Introduction to break out | |
| 15:30–15:50 | Coffee | |
| 15:50–16:30 | Breakout session (SoC) <ul style="list-style-type: none"> • Steroids | 3 groups, facilitated by Faculty |
| 16:30-16:50 | Feedback and discussion | |
| 16:50-17:50 | Communication with parents <ul style="list-style-type: none"> • Identifying fake news from factual news • Delivering diagnosis appropriately • Answering parents' questions effectively and positively • Enabling parents to feel confident making decisions about their child's care • Managing parents' and carers' expectations • Transition of care and autonomy for the child/adult – including the young adult in decision making as appropriate | Andoni Urtizbera |
| 17:50 | End of the day/wrap up | Annemieke Aartsma-Rus |
| 19:00 | Dinner | |

Day 2 – Friday 17 May Chairs: Nathalie Goemans (am) and Ros Quinlivan (pm)

| | | |
|-------------|---|--------------------------------------|
| 08:30–09:30 | Physiotherapy & Orthopaedics SoC <ul style="list-style-type: none"> • Care & assessments • Adult and emergency • PUL and North Star | Elena Mazzone |
| 09:30-10:15 | Respiratory SoC | Jana Haberlova |
| 10:15–10:45 | Coffee | |
| 10:45–11:25 | Cardiac SoC <ul style="list-style-type: none"> • Becker • Adults and carriers • Emergency | Karim Wahbi |
| 11:25–11:55 | Adult Duchenne Management including Becker <ul style="list-style-type: none"> • Introduction | Ros Quinlivan |
| 11:55-12:45 | Breakout <ul style="list-style-type: none"> • Adult DMD and Becker Management | Facilitated by Faculty |
| 12:45-13:00 | Feedback and discussion | |
| 13:30-13:45 | Lunch | |
| 13:45-14:00 | Reaching a consensus on SoC | Erik Niks |
| 14:00-14:40 | Emerging therapies – Bench to Bedside <ul style="list-style-type: none"> • Biomarkers • Regulatory & HTA • Financial implications | Michela Guglieri and Cecile Ollivier |
| 14:40-15:10 | Emerging therapies - Therapeutics in trials <ul style="list-style-type: none"> • Dystrophin restoration • Secondary pathology | Annemieke Aartsma-Rus |
| 15:10-15:25 | Clinical trial burden – patient perspective | Maxime Arras |
| 15:25 | Meeting wrap up and close | Annemieke Aartsma-Rus |
| 15:30 | Coffee available | |

Overview

Following the success of a previous masterclass series from 2015 to 2018, the 2019 Expert Masterclass on Duchenne Muscular Dystrophy (DMD) was held in Amsterdam, Netherlands on 16th and 17th May. The event provided high-quality information regarding diagnosis of DMD, the 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 and this 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 Elizabeth Vroom introducing the event and its aims. 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 breakout sessions which allowed delegates to discuss more sensitive and specific issues relating to: standards of care, steroids, adult DMD and Becker muscular dystrophy (BMD) management. 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, barriers and their solutions. The dinner on the evening of 16th provided an opportunity for delegates to network socially too during the event.

The masterclass was drawn to a close with talks from Maxime Arras; a DMD patient who took part in an exon-skipping clinical trial for 7 years, and then the closing summary was given Professor Annemieke Aartsma-Rus.

Meeting the Objectives: One of the meeting objectives stated in the masterclass programme was to:

- Give patient and parent perspectives on diagnosis, care and research in DMD

This was achieved through the inclusion of Simon Van Boxtel and Phillip and Maxime Arras' presentations as well as the presentation given by Elizabeth Vroom.

Speakers:

Co-Chairs:

- Annemieke Aartsma-Rus, Leiden University Medical Center, the Netherlands, and John Walton Muscular Dystrophy Research Centre, Newcastle University, UK
- Nathalie Goemans, University of Leuven, Belgium
- Ros Quinlivan, MRC Centre for Neuromuscular Diseases, University College London, UK
- Elizabeth Vroom, World Duchenne Organization, the Netherlands

Additional speakers

- Maxime Arras, patient representative, Belgium
- Michela Guglieri, John Walton Muscular Dystrophy Research Centre, Newcastle University, UK
- Jana Haberlova, Charles University, Czech Republic
- Alex Johnson, Duchenne UK
- Elena Mazzone, Fondazione Telethon, Italy
- Erik Niks, Leiden University Medical Center, the Netherlands
- Cécile Ollivier, Aparito, the Netherlands
- Andoni Urtizbera, Paris Summer School of Myology, France
- Simon van Boxtel, patient representative, the Netherlands
- Karim Wahbi, Cochin Hospital Paris, France

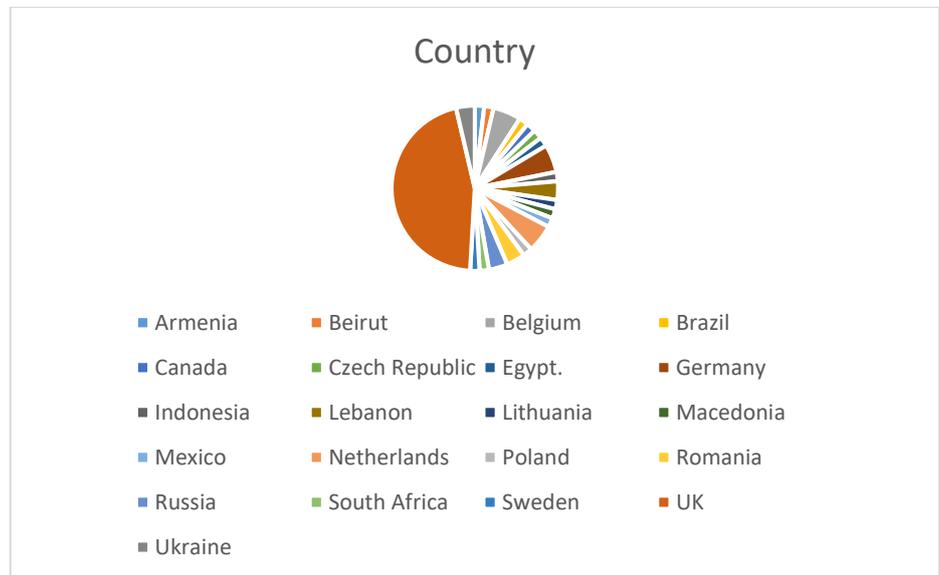
Meeting the objectives:

One of the meeting objectives stated in the in the masterclass programme was to:

- Provide a high-quality educational and interactive meeting for paediatric neurologists and other related specialities who may be diagnosing and/or managing the care of patients with Duchenne muscular dystrophy (DMD)

The masterclass attracted 57 delegates from 21 different countries and a range of professional backgrounds, thus achieving the aims set out above. A breakdown of these figures can be found below:

| Country | Number of Attendees |
|----------------|---------------------|
| Armenia | 1 |
| Beirut | 1 |
| Belgium | 3 |
| Brazil | 1 |
| Canada | 1 |
| Czech Republic | 1 |
| Egypt | 1 |
| Germany | 3 |
| Indonesia | 1 |
| Lebanon | 2 |
| Lithuania | 1 |
| Macedonia | 1 |
| Mexico | 2 |
| Netherlands | 3 |
| Poland | 1 |
| Romania | 2 |
| Russia | 2 |
| South Africa | 1 |
| Sweden | 1 |
| UK | 26 |
| Ukraine | 2 |
| Total | 57 |



In order to qualify for selection the TEC agreed on criteria and applicants were asked about how they would meet such criteria. Criteria such as: occupation, affiliation, country and how the information would be disseminated following the event. This information was examined on a case by case basis and candidates were selected using this information and applying the criteria.

There is a significant UK bias in the data. There are several reasons which could go towards explaining this: perhaps the location of Amsterdam was more convenient for UK applicants, perhaps the networking within these areas and support regarding training was more prevalent, there were a greater number of applications from UK based clinicians. No geographical areas were given preference over another. All application criteria was applied to each case fairly.

Below is a further breakdown of the applications by country and the percentage success rate for applications.

| Country | Number of eligible applications | Percentage of successful applications | Number of ineligible applications * |
|----------------|---------------------------------|---------------------------------------|-------------------------------------|
| Armenia | 1 | 100% | 0 |
| Belgium | 4 | 100% | 0 |
| Brazil | 1 | 100% | 0 |
| Canada | 1 | 100% | 0 |
| Czech Republic | 1 | 100% | 0 |
| Egypt | 1 | 100% | 0 |
| Germany | 3 | 100% | 0 |
| India* | 0 | 0% | 1 |
| Indonesia | 1 | 100% | 0 |
| Lebanon | 2 | 100% | 0 |
| Lithuania | 1 | 100% | 0 |
| Macedonia* | 1 | 20% | 4 |
| Mexico | 2 | 100% | 0 |
| Netherlands | 4 | 100% | 0 |
| Poland | 1 | 100% | 0 |
| Romania* | 3 | 60% | 2 |
| Russia* | 2 | 66% | 1 |
| South Africa | 1 | 100% | 0 |
| Sweden | 1 | 100% | 0 |
| UK * | 24 | 80% | 6 |
| Ukraine | 2 | 100% | 0 |
| Total | 57 | | 16 |

* Those countries where the amount of successful applications is lower is due to these applications being ineligible for selection according to the selection criteria.

Those applicants who were ineligible were from either individual parents or patient organisations rather than clinicians or from clinicians who did not work with DMD clinical teams and patients.

This, along with the ability to cascade learning to wider teams, was part of the selection criteria, so these applicants were not awarded places.

Each application for the TREAT-NMD Masterclass 2020 will be assessed on a case by case basis once again and the selection criteria applied by the TREAT-NMD Education Committee.

Below is a further breakdown of the applications by professional category.



In terms of the spread of professional categories there is an interesting spread. The two main categories of doctor and neurologist are to be expected given the nature of DMD these clinicians are at the forefront of multidisciplinary treatment teams.

This could be used to our advantage for future masterclasses as these people could become part of the TREAT-NMD audience and be contacted regarding future events and pass this information to members of their networks.

Meeting the Objectives:

- Provide a high-quality educational and interactive meeting

This was achieved through a range of Q and A sessions, breakout groups and the delegate networking dinner, but also through the use of Vevox interactive voting and polling app which was used during presentations by some of the speakers, but also to gather evaluation responses, the results of which are examined in the next section of the report.

Vevox Evaluation Responses

The evaluation is comprised of 24 questions; some with 1 selection response and some with room for comment. There were 56 respondents to the evaluation. There were 57 attendees and 6 industry observers totalling 63 potential participants, so 88% of those who attended have given feedback in the evaluation.

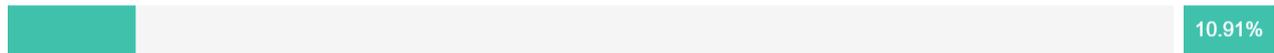
Question 1

How useful did you find this masterclass for your professional activity?

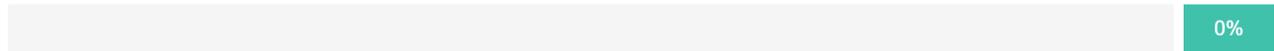
Very useful



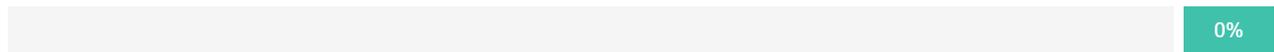
Somewhat useful



Not sure



Not useful



Question 2

If the masterclass was not as useful as you hoped, please say why

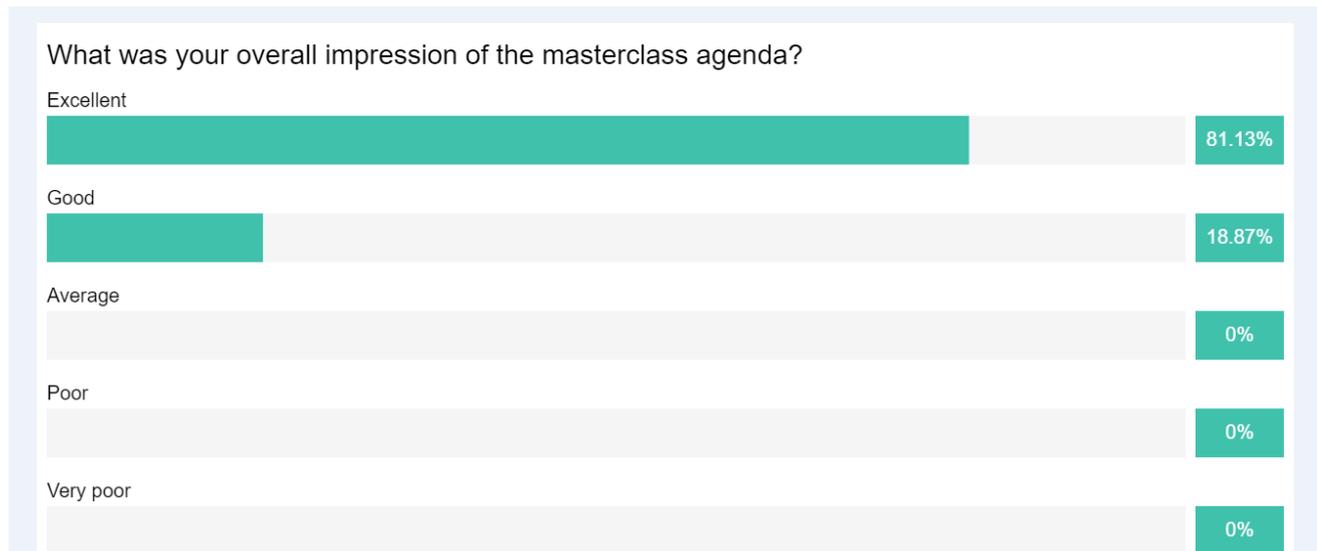
- 23 respondents
- 22 to confirm event was as expected
- 1 comment to expand upon:

‘there are always more details to be discussed: endocrinology problems nutritional psych and PT access in different countries etc.’

Actions/comments:

Perhaps something which can be addressed through more breakout sessions, pairing up by location and/or job or affiliation to share information and encourage networking to encourage working links to address this issue.

Question 3



Question 4 : 32 responses

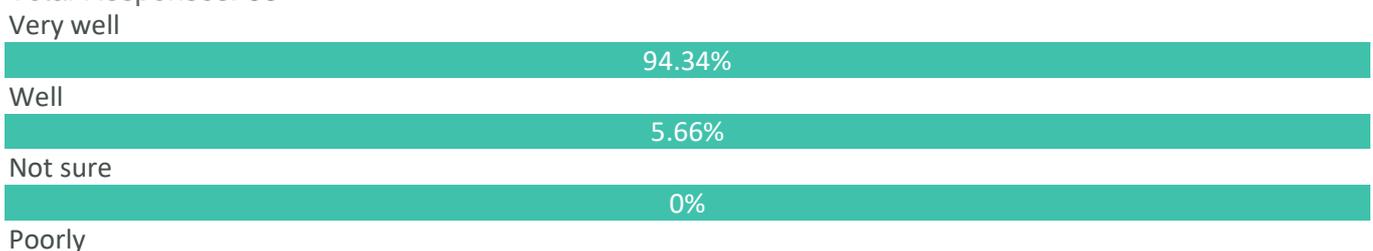
What would you have liked to see also included on the agenda? (amount of responses in brackets):

1. Breakout sessions for more discussion and collaboration (more with nurse or physio perspective) (10)
2. Agenda complete/ n/a (8)
3. Workshops tailored to disciplines or Info about newer treatments (6)
4. More info on clinical trials and which centres are participating (2)
5. Information about psychosocial and psychological issues (2)
6. Nutrition assessment/impact (2)

Question 5

How well do you think the masterclass was organised?

Total Responses: 53



| | |
|-------------|----|
| | 0% |
| Very poorly | |
| | 0% |

Question 6

How could we improve the organisation next time?

31 responses

Other responses were (amount of responses in brackets):

1. Confirmation that organisation was very good and no changes (19)
2. More breakout sessions (3)
3. Share participant list (2)
4. Transfer from airport to hotel was long (1)
5. Masterclass should be each year (1)
6. Formal letter of invitation for visa applications (1)
7. Include other countries like Guatemala and Bolivia (1)
8. Earlier finish on first day (1)
9. Warning or cover cost of £50 refundable deposit for rooms (1)
10. Standing banner to take pictures for own twitter account (1)

Actions/comments:

Formal letters were given on request to individuals who needed them for visa applications

Sharing of participant info was done post masterclass due to GDPR restrictions-this could be addressed in the application process for 2020.

Question 7

As a result of participating, I have a better understanding of the family perspective

Total Responses: 53

Strongly agree

| | |
|-------------------|--------|
| | 52.83% |
| Agree | |
| | 47.17% |
| Not sure | |
| | 0% |
| Disagree | |
| | 0% |
| Strongly disagree | |
| | 0% |

Question 8

As a result of participating, I have a better understanding of the patient perspective

Total Responses: 53

Strongly agree

| | |
|-------------------|--------|
| | 54.72% |
| Agree | |
| | 43.40% |
| Not sure | |
| | 1.89% |
| Disagree | |
| | 0% |
| Strongly disagree | |
| | 0% |

Question 9

I now have a better understanding of the variability of DMD

Total Responses: 53

Strongly agree

| | |
|--|--------|
| | 52.83% |
|--|--------|

Agree

| | |
|--|--------|
| | 43.40% |
|--|--------|

Not sure

| | |
|--|-------|
| | 3.77% |
|--|-------|

Disagree

| | |
|--|----|
| | 0% |
|--|----|

Strongly disagree

| | |
|--|----|
| | 0% |
|--|----|

Question 10

I now have a better understanding of the recommended diagnostic pathway for DMD

Total Responses: 52

Strongly agree

| | |
|--|--------|
| | 59.62% |
|--|--------|

Agree

| | |
|--|--------|
| | 36.54% |
|--|--------|

Not sure

| | |
|--|-------|
| | 3.85% |
|--|-------|

Disagree

| | |
|--|----|
| | 0% |
|--|----|

Strongly disagree

| | |
|--|----|
| | 0% |
|--|----|

Question 11

I now have a better understanding of the genetics of DMD

Total Responses: 52

Strongly agree

67.31%

Agree

23.08%

Not sure

9.62%

Disagree

0%

Strongly disagree

0%

Question 12

I now have a better understanding of steroid use and management according to the latest standards of care for DMD

Total Responses: 52

Strongly agree

44.23%

Agree

48.08%

Not sure

5.77%

Disagree

1.92%

Strongly disagree

0%

Question 13

I now have a better understanding of best practice when communicating with families about DMD

Total Responses: 52

Strongly agree

50.00%

Agree

42.31%

Not sure

3.85%

Disagree

3.85%

Strongly disagree

0%

Question 14

I now have a better understanding of the physiotherapy recommendations in the latest standards of care

Total Responses: 52

| | |
|-------------------|--------|
| Strongly agree | 46.15% |
| Agree | 40.38% |
| Not sure | 11.54% |
| Disagree | 1.92% |
| Strongly disagree | 0% |

Question 15

I now have a better understanding of respiratory management according to the latest standards of care

Total Responses: 52

Strongly agree

| | |
|-------------------|--------|
| Strongly agree | 44.23% |
| Agree | 48.08% |
| Not sure | 5.77% |
| Disagree | 1.92% |
| Strongly disagree | 0% |

Question 16

I now have a better understanding of cardiac management according to the latest standards of care

Total Responses: 53

Strongly agree

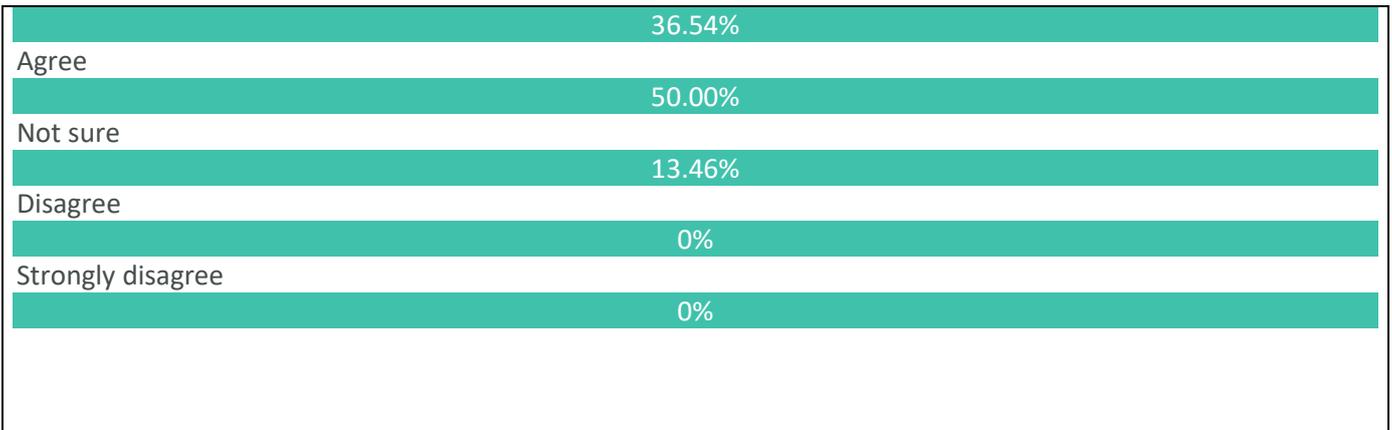
| | |
|-------------------|--------|
| Strongly agree | 45.28% |
| Agree | 49.06% |
| Not sure | 5.66% |
| Disagree | 0% |
| Strongly disagree | 0% |

Question 17

I now have a better understanding of best practice in management of care of adults living with DMD

Total Responses: 52

Strongly agree



Question 18

I now have a better understanding of emerging therapies for DMD

Total Responses: 51

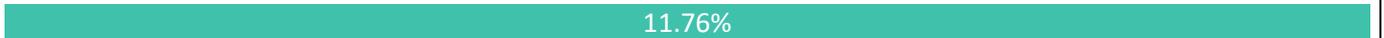
Strongly agree



Agree



Not sure



Disagree



Strongly disagree



Question 19

Please say 1-3 ways you will change your clinical practice based on this masterclass (number of responses in brackets).

Total Responses: 33

1. Communicating messages with patients and families more effectively (8)
2. Share information regarding Standards of Care (8)
3. Understanding and sharing information regarding genetics (5)
4. Share information with communities about DMD (3)
5. Share information and understanding regarding child-adult transition (3)
6. Cascade information about steroid treatment/management (3)
7. Join the TREAT-NMD network/ mailing list (3)

Question 20

Please say what was the most useful/best thing about this masterclass (number of responses in brackets)

Total Responses: 35

1. Genetics talks and quiz (9)
2. Patient/parent and family perspectives (8)
3. Networking and collaboration (6)
4. All of the sessions were useful (4)
5. Standards of Care (4)
6. Physio lectures (2)
7. Emerging therapies (1)
8. Q and A and discussionbs (1)

Question 21

Please say what was the least useful thing about this masterclass (amount of responses in brackets):

Total Responses: 26

Other responses were:

1. All useful (15)
2. Add workshops that people can sign up to/breakout sessions (4)
3. Discussions were hard to follow in breakouts as groups were large (2)
4. At times difficult to discuss adult or child specific treatment if some people do not work in this age bracket (1)
5. Emerging therapies (1)
6. Drug regulations (1)
7. Reaching a consensus on SoC (1)
8. EMA (1)

Question 22

Was there adequate time for discussion/networking?

Total Responses: 48

There was just the right amount of time for discussion

58.33%

Not enough time for discussion

39.58%

Too much time for discussion

0%

Not sure

2.08%

Question 23

Would you recommend this masterclass to a colleague?

Total Responses: 49

Yes



No



Not sure



Question 24

Thank you for your feedback! Is there anything else you would like to add or comment on?

Total Responses: 39 of these, 29 responses were of thanks for the event, how much participants had enjoyed the masterclass and the excellent organisation from TREAT-NMD.

Other comments were:

1. Enjoyed the event/thanks (29)
2. Time built in for people to share a 2 minute presentation about their area of expertise to help with networking (3)
3. Introductions in some way to meet and make links for collaboration (2)
4. Excellent speakers (1)
5. Hotel was great and meals lovely (1)
6. More interaction during presentations (1)
7. Could easily be 3 days (1)
8. Please hold a masterclass in South America (1)

PMI Analysis

| <u>Plus</u> | <u>Minus</u> | <u>Interesting</u> |
|--|---|--|
| <p>A great deal of positive feedback for every question.</p> <p>Many of the ideas put forward by delegates are practical and some can be implemented easily.</p> | <p>Some comments regarding long days (especially first day if people travelled that morning). This is necessary to keep costs down and to ensure all information is covered.</p> <p>One comment regarding the hotel venue being far from the airport (NB: it was close to Amsterdam Centraal station and all travel information was sent out in advance).</p> | <p>More breakout sessions needed was the most common feedback. This was to allow for collaboration, networking within professional categories, sharing experiences etc. Perhaps facilitate this by groupings as above and/or ask people to prepare something short which they will share in a carousel activity at the start or on first day of the event. Build in more breakout sessions or networking evening activities.</p> <p>More demand for sessions/masterclasses and longer-3 day events</p> |

Actions/Comments:

1. Consult TEC and Cathy about the breakout sessions.
2. Consult TEC and Cathy about the groupings/facilitating collaboration by range of categories so that links are made between delegates.
3. Link by age range, professional category, country, affiliation/type of organisation etc.
4. Consult TEC re workshop ideas-could people sign up? Or have a choice?
5. Sharing of information made clearer at the start-will be shared post MC due to GDPR.
6. Letters for visas-made clear that these are available on request.
7. Consider an alternative sharing platform-Dropbox or??
8. Use contacts from this masterclass to crate larger netowkr for promoting and recruiting for future events-feedback was so posiotive this would be a good way to promote.