

## TREAT-NMD Expert Masterclass Week

### (DMD, Gene Therapy and SMA)

Monday 22<sup>nd</sup> – Friday 26<sup>th</sup> May 2023

Amsterdam, Netherlands

Face-to-Face

#### Overview

With the current advances in the field of neuromuscular care and treatments, it is important for all healthcare professionals to be kept abreast of these advances so that they can provide the most up to date information and care to patients. TREAT-NMD is therefore delighted to be hosting a face-to-face Expert Masterclass Week (DMD, Gene Therapy and SMA) in Amsterdam in 2023.

Each 1.5 day masterclass will feature sessions providing the most up to date information on diagnosis, the patient and parent perspective, and standards of care as well as emerging therapies (please see the masterclass programmes below).

Delegates are welcome to spend the week with us in Amsterdam and attend all of the masterclasses or select one or two areas of interest. Please select all of the masterclasses you are planning to attend on the [Expert Masterclass Week Application Form](#).

#### 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), Spinal Muscular Atrophy 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 patients with DMD and SMA.
- Provide the latest information on gene therapy, advances in this field and lessons learned from the clinical landscape past and present.
- Provide an overview of the current clinical trial landscape and the emerging implications for care and therapies.

#### Masterclass Structure

Day 1 22 <sup>nd</sup> May 2023	Day 2 23 <sup>rd</sup> May 2023	Day 3 24 <sup>th</sup> May 2023	Day 4 25 <sup>th</sup> May 2023	Day 5 26 <sup>th</sup> May 2023
	DMD Masterclass	Gene Therapy Masterclass	Gene Therapy	SMA Masterclass
DMD Masterclass			SMA Masterclass	

Draft Programme*		
TIME	PROGRAMME SESSION	SPEAKER DETAILS
12:00 – 13:00	Welcome Lunch and Masterclass Check In	
13:00 – 13:05	Masterclass Welcome	<b>Chairs</b> <b>Annemieke Aartsma-Rus</b> , Leiden University Medical Center, Netherlands <b>Nathalie Goemans</b> , University of Leuven, Belgium
13:05 -13:50	Diagnosis (including clinician and patient/parent perspective)	<b>Michela Guglieri</b> , John Walton Muscular Dystrophy Research Centre, UK  <b>Filippo Buccella</b> , Duchenne Parent Project, Italy
13:50 – 14:00	Q&A	<b>Chairs</b>
14:00 – 14:30	Genetics of DMD	<b>Annemieke Aartsma-Rus</b> , Leiden University Medical Center, Netherlands
14:30 – 14:40	Q&A	<b>Chairs</b>
14:40 – 15:00	Afternoon Break	
15:00 – 15:30	Adult Duchenne and Becker	<b>Ros Quinlivan</b> , University College London Hospitals, UK
15:30 -15:40	Q&A	<b>Chairs</b>
15:40 -16:10	Overview of Therapies in Development and approved	<b>Annemieke Aartsma-Rus</b> , Leiden University Medical Center, Netherlands
16:10 -16:20	Q&A	<b>Chairs</b>
16:20 – 16:50	<b>Patient Registries and Patient Engagement</b> <ul style="list-style-type: none"> <li>• The value of patient registries – why are they so important to patients and the community?</li> <li>• Importance of data collection</li> </ul>	<b>Elizabeth Vroom</b> , World Duchenne Organisation, Netherlands

	<ul style="list-style-type: none"> <li>• What are the success stories surrounding patient engagement?</li> <li>• What are the challenges Patient Organisations face?</li> </ul>	
<b>16:50 – 17:00</b>	<b>Q&amp;A</b>	<b>Chairs</b>
<b>TIME</b>	<b>Day 2 DMD Masterclass Tuesday 23<sup>rd</sup> May 2023 PROGRAMME SESSION</b>	<b>SPEAKER DETAILS</b>
<b>08:30 -09:00</b>	<b>Refreshments and welcome to Day 2</b>	
<b>09:00 – 09:30</b>	<b>Cardiac management</b>	<b>Karim Wahbi</b> , University of Paris, France
<b>09:30 – 09:40</b>	<b>Q&amp;A</b>	
<b>09:40– 10:10</b>	<b>Respiratory care</b>	<b>Nathalie Goemans</b> , University of Leuven, Belgium
<b>10:10 – 10:20</b>	<b>Q&amp;A</b>	
<b>10:20 – 10:40</b>	<b>Morning Break</b>	
<b>10:40 – 11:10</b>	<b>Nutrition and bowel health</b>	<b>Ros Quinlivan</b> , University College London Hospitals, UK
<b>11:10 -11:20</b>	<b>Q&amp;A</b>	
<b>11:20 -11:50</b>	<b>Steroids and bone health</b>	<b>TBC</b>
<b>11:50 -12:00</b>	<b>Q&amp;A</b>	
<b>12:00 – 13:00</b>	<b>Lunch</b>	
<b>13:00 -14:00</b>	<b>Workshop 1: Case Studies</b> Including discussion of interesting/complex cases <b>OR</b> <b>Workshop 2: Physiotherapy</b>	<b>All Clinicians from previous sessions</b>  <b>TBC</b>
<b>14:00 – 14:20</b>	<b>Afternoon Break</b>	
<b>14:20 – 15:20</b>	<b>Workshop 1: Case Studies</b> Including discussion of interesting/complex cases <b>OR</b> <b>Workshop 2: Physiotherapy</b>	<b>All Clinicians from previous sessions</b>  <b>TBC</b>
<b>15:20 – 15:30</b>	<b>Break and Re-group</b>	

15:30 -15:50	Q&A and Discussion	Chairs
15:50 – 16:00	Close of masterclass	Chairs

<b>TREAT-NMD Expert Masterclass Week</b> <b>Gene Therapy Masterclass</b> <b>24<sup>th</sup>- 25<sup>th</sup> May 2023</b>		
<b>Draft Programme*</b>		
TIME	PROGRAMME SESSION	SPEAKER DETAILS
08:30 - 09:00	Masterclass Check-In & Morning Refreshments	
09:00 - 09:05	Masterclass Welcome	Chair <b>Liesbeth De Waele</b> , University of Leuven, Belgium
09:05 – 09:50	Gene Therapy: The background and the basics	<b>Annemieke Aartsma-Rus</b> , Leiden University Medical Center, Netherlands
09:50 – 10:00	Q&A	Chair
10:00 -10:45	<b>The Patient Perspective</b> <ul style="list-style-type: none"> <li>• Gene therapy</li> <li>• Participating in clinical trials</li> <li>• Using gene therapy product</li> </ul>	<b>Marie-Christine Ouillade</b> , AFM Telethon, France and SMA Europe  <b>Sebastian Pańczyk</b>
10:45 – 10:55	Q&A	Chair
10:55 – 11:15	Morning Break	
11:15 – 12:15	<b>Clinical Experience:</b> <ul style="list-style-type: none"> <li>• Overview of ongoing clinical trials in DMD, MTM and NMDs.</li> </ul> <b>Pre-clinical Experience:</b> <ul style="list-style-type: none"> <li>• Trial design and trial readiness</li> </ul>	<b>Andoni Uritzberrea</b> , Maladies Orphelines Sans Frontières, France  <b>Ana Buj-Bello</b> , Genethon, France
12:15 – 12:25	Q&A	Chair

12:25 – 13:25	Lunch	
13:25 -14:25	<b>Running a Clinical Trial</b> <ul style="list-style-type: none"> <li>Logistics of running a gene therapy trial</li> <li>Communication to patients (clinician and patient perspective)</li> </ul>	TBC  Sebastian Pańczyk
14:25 – 14:35	Q&A	Chair
14:35 – 15:05	<b>Safety Considerations</b> <ul style="list-style-type: none"> <li>Safety issues in AAV Gene Therapy</li> <li>Management of safety issues</li> </ul>	Giovanni Baranello, University College London, UK
15:05 – 15:15	Q&A	Chair
15:15 – 15:35	Afternoon Break	
15:35 – 16:10	<b>Approved products:</b> <ul style="list-style-type: none"> <li>State of the art for zolgensma</li> <li>Prescribing gene therapy products</li> <li>How to monitor for side effects</li> </ul>	Liesbeth De Waele, University of Leuven, Belgium
16:10 – 16:20	Q&A	Chair
16:20 – 16:55	<b>Emerging Therapies:</b> <ul style="list-style-type: none"> <li>The pipeline</li> <li>Early access programs</li> <li>Pricing and access</li> </ul>	TBC  TBC
16:55 – 17:05	Q&A	Chair
	<b>Day 2 Gene Therapy Masterclass</b> <b>Thursday 25<sup>th</sup> May 2023</b> <b>Programme Session</b>	
TIME	PROGRAMME SESSION	SPEAKER DETAILS
08:30 – 09:00	Refreshments and welcome to Day 2	

<b>09:00 – 10:00</b>	<b>Workshop 1 -Experiences of safety, efficacy of long-term effects.</b> <ul style="list-style-type: none"> <li>Experiences of Adverse Effects of Gene Therapy</li> <li>Short and long term outcomes of Gene Therapy</li> <li>Long term follow-up of patients after Gene Therapy treatment.</li> </ul>	<b>All speakers</b>
<b>10:00 – 10:20</b>	<b>Morning Break</b>	
<b>10:20 – 11:20</b>	<b>Workshop 2 -Managing clinician &amp; patient expectations.</b> <ul style="list-style-type: none"> <li>Decisions surrounding the selection of the right candidates for Gene Therapy</li> <li>Managing clinical expectations of Gene Therapy</li> <li>Managing patient/family's expectations of Gene Therapy</li> </ul>	<b>All speakers</b>
<b>11:20 – 12:00</b>	<b>Preparing for the future</b> <ul style="list-style-type: none"> <li>Ethical aspects</li> <li>Patient perception for benefit and risk</li> <li>Inflammatory response and potential for redosing</li> <li>Preclinical development/what to expect in the future?</li> </ul>	<b>Andoni Urtizberea</b> , Maladies Orphelines Sans Frontières, France
<b>12:00 – 12:10</b>	<b>Q&amp;A</b>	<b>Chair</b>
<b>12:10 – 12:20</b>	<b>Close of masterclass</b>	<b>Chair</b>

<b>TREAT-NMD Expert Masterclass Week</b> <b>SMA Masterclass</b> <b>25<sup>th</sup> - 26<sup>th</sup> May 2023</b>		
<b>Draft Programme*</b>		
TIME	PROGRAMME SESSION	SPEAKER DETAILS
<b>12:20 – 13:20</b>	<b>Welcome Lunch and Masterclass Check In</b>	
<b>13:30 – 13:35</b>	<b>Masterclass Welcome</b>	<b>Chair</b> <b>Eduardo Tizzano</b> , Vall d'Hebron Hospital, Spain

<b>13:35 – 14:20</b>	<b>Patient Perspective</b>	<b>Yasemin Erbas, SMA Europe, Belgium</b>
<b>14:20 – 14:30</b>	<b>Q&amp;A</b>	<b>Chair</b>
<b>14:30 – 15:00</b>	<b>Communicating with Patients and Families</b>	<b>Stefan Bos, SMA Europe, Netherlands</b>
<b>15:00 – 15:10</b>	<b>Q&amp;A</b>	<b>Chair</b>
<b>15.10 – 15:30</b>	<b>Afternoon Break</b>	
<b>15:30 – 16:15</b>	<b>Genetics of SMA</b>	<b>Eduardo Tizzano, Vall d’Hebron Hospital, Spain</b>
<b>16:15 – 16:25</b>	<b>Q&amp;A</b>	<b>Chair</b>
<b>16:25 - 17:05</b>	<b>Newborn Screening</b>	<b>Damjan Osredkar, University Medical Centre Ljubljana, Slovenia</b>
<b>17:05 – 17:15</b>	<b>Q&amp;A</b>	<b>Chair</b>
	<b>Day 2 SMA Masterclass Friday 26<sup>th</sup> May 2023 Programme Session</b>	
<b>TIME</b>	<b>PROGRAMME SESSION</b>	<b>SPEAKER DETAILS</b>
<b>08:30 – 09:00</b>	<b>Refreshments and welcome to Day 2</b>	
<b>09:00 – 09:40</b>	<b>Current treatments for SMA</b>	<b>TBC</b>
<b>09:40 – 09:50</b>	<b>Q&amp;A</b>	
<b>09:50 – 10.20</b>	<b>Changing natural history in SMA</b>	<b>Nathalie Goemans, University of Leuven, Belgium</b>
<b>10:20 – 10:30</b>	<b>Q&amp;A</b>	

<b>10:30 – 10:50</b>	<b>Morning Break</b>	
<b>10:50 – 11:30</b>	<b>Respiratory Care</b>	<b>Anita Simonds</b> , Royal Brompton Hospital, UK
<b>11:30 – 11:40</b>	<b>Q&amp;A</b>	<b>Chair</b>
<b>11:40 – 12:20</b>	<b>Nutritional management in patients with SMA</b>	<b>TBC</b>
<b>12:20 – 12:30</b>	<b>Q&amp;A</b>	
<b>12:30 – 13:30</b>	<b>Lunch</b>	
<b>13:30 – 14:00</b>	<b>Patient Registries</b> <ul style="list-style-type: none"> <li>• The value of patient registries – why are they so important to patients and the community?</li> <li>• Importance of data collection</li> <li>• What are the success stories surrounding patient engagement?</li> <li>• What challenges do Patient Organisations face and how can they be overcome?</li> </ul>	<b>TBC</b>
<b>14:00 – 14:10</b>	<b>Q&amp;A</b>	
<b>14:10 – 15:10</b>	<b>Workshop 1: Physiotherapy in SMA Patients: Motor Development and physiotherapy in SMA</b>  <b>OR</b>  <b>Workshop 2: Clinical Cases</b> <b>Including discussion of interesting/complex cases</b>	<b>TBC</b>   <b>All Clinicians from previous sessions</b>
<b>15:10 – 15:30</b>	<b>Afternoon Break</b>	
<b>15:30 – 16:30</b>	<b>Workshop 1: Physiotherapy in SMA Patients: Motor Development and physiotherapy in SMA</b>  <b>OR</b>  <b>Workshop 2: Clinical Cases</b>	<b>TBC</b>   <b>Clinicians from earlier sessions</b>



	<b>Including discussion of interesting/complex cases</b>	
<b>16:30 – 16:50</b>	<b>Q+A and Discussion</b>	<b>Chair</b>
<b>16:50 – 17:00</b>	<b>Close of Masterclass</b>	<b>Chair</b>

DRAFT