

# Expert Masterclass on Duchenne Muscular Dystrophy

Thursday 18 – Friday 19 May 2017

DoubleTree by Hilton Hotel, Lisbon, Portugal

## 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
- Examine the current treatment guidelines and available therapy options
- Analyze the pharmacological therapies that are currently being used in the clinic for optimal patient management in the different regions
- Assess the effectiveness of different measurements for accurately determining the progression of disease in the clinic, and discuss regional differences and challenges in the management of patients with DMD
- Consider the importance of physiotherapy in the management of DMD
- Provide an overview of therapies that are currently in late stage clinical development
- Review the market access options for patients eligible for treatment for DMD in Europe and understand the patient advocacy group burden and the support they can offer to families

## Meeting format

- The masterclass will be a mixture of lectures and workshop sessions, with a significant amount of time allocated to discussion
- Every delegate will be asked to download a messaging and polling app onto their phone or computer, with the aim of increasing engagement and interactivity at the meeting. The app will ensure that discussions revolve around the topics of most interest to you and your colleagues, as well as allowing results of key questions taken at the meeting to be displayed

## Co-chairs

- Annemieke Aartsma-Rus, Leiden University Medical Center, Leiden, the Netherlands, and John Walton Muscular Dystrophy Research Centre, Newcastle University, Newcastle, UK
- Manuela Santos, Centro Hospitalar do Porto, Porto, Portugal

**Day 1 – Thursday 18 May**

09:00–09:10	<b>Welcome and programme overview</b>	Annemieke Aartsma-Rus
09:10–09:20	<b>Overview of TREAT-NMD</b>	<b>Presentation</b> by Annemieke Aartsma-Rus (10 minutes)
09:20–09:50	<b>The true impact of DMD: A parent’s outlook</b>	<b>Presentation</b> by Filippo Buccella (15 minutes) <b>Discussion</b> (15 minutes)
09:50–10:35	<b>Introduction to DMD</b>	<b>Presentation</b> by Nathalie Goemans (25 minutes) <b>Discussion</b> (20 minutes)
10:35–11:00	<b>Coffee</b>	
11:00–12:00	<b>The genetics of DMD and the importance of genetic testing in obtaining a mutation-specific diagnosis of DMD</b>	<b>Presentation</b> by Annemieke Aartsma-Rus (30 minutes) <b>Discussion</b> (30 minutes)
12:00–13:00	<b>Workshop session: Diagnostics in practice</b>	<b>Discussion</b> (60 minutes)
13:00–14:00	<b>Lunch</b>	
14:00–15:00	<b>Management of DMD</b>	<b>Presentation</b> by Nathalie Goemans (25 minutes) <b>Discussion</b> (35 minutes)
15:00–15:30	<b>Coffee</b>	
15:30–17:00	<b>Workshop session: Patient management practices in different regions</b>	<b>Presentation</b> by Imelda de Groot (20 minutes) <b>Discussion</b> (70 minutes)
17:00–17:15	<b>Day 1 summary and close</b>	Annemieke Aartsma-Rus
Evening	<b>Dinner</b>	

**Day 2 – Friday 19 May**

08:30–08:40	<b>Welcome back</b>	Manuela Santos
08:40–10:10	<b>Workshop session: Measurements to determine progression of DMD in clinical practice</b>	<b>Case study presentations</b> by Imelda de Groot (30 minutes) <b>Discussion</b> (60 minutes)
10:10–10:30	<b>Coffee</b>	
10:30–11:30	<b>Workshop session: Practical physiotherapy session</b>	<b>Presentation</b> by Marina di Marco (30 minutes) <b>Discussion</b> (30 minutes)
11:30–12:15	<b>Lunch</b>	
12:15–13:00	<b>Therapies in clinical development</b>	<b>Presentation</b> by Annemieke Aartsma-Rus (30 minutes) <b>Discussion</b> (15 minutes)
13:00–14:00	<b>Ensuring access for patients to treatments for DMD in Europe</b>  <b>Fighting for the patient with DMD: A patient advocacy group outlook</b>	<b>Presentation</b> by Dimitros Athanasiou (30 minutes) <b>Discussion</b> (30 minutes)
14:00–14:15	<b>Day 2 summary and meeting close</b>	Manuela Santos