

TREAT-NMD International Conference 2019

Monday 9 – Wednesday 11 December 2019

Leiden – The Netherlands



MONDAY 9TH DECEMBER

15.00 -
17.00 pm

Registration

17.30 –
19.30 pm

Drinks reception

TUESDAY 10TH DECEMBER

08.00 am

Registration

WELCOME

09.00 am

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

KEYNOTE LECTURE

09.15 am

When being a scientist is not enough

Mayana Zatz, Professor of Genetics, University of São Paulo, Brazil

10.00 am

Discussion

10.15 am

Refreshments

SESSION ONE: Patient engagement

Patient advocacy

Kees van der Graaf, Chairman of FSHD Unlimited, The Netherlands
&
Ingrid de Groot, Patient representative, The Netherlands

Chairpersons:

Ria Broekgaarden
Fabiola Bertinotti

12.15 pm

Lunch

13.15 pm

Lunchtime Symposia
(Location: Grote Zaal)

Networking & Posters
(Location: Conelis Schuytzaal)

13:15 - 13.35 pm **Sarepta**

13:35 – 13:50 pm **Biogen**

SESSION TWO: Adults with progressive neuromuscular disease

14.00 pm

Respiratory muscle training

Anri Human, Lecturer & Researcher, Physiotherapy department, Sefako Makgatho Health Sciences University, South Africa

Chairpersons:

Anri Human
Zohar Argov

14.20 pm

Nutritional assessment and interventions in adult NMD

Heidi Zweers, Dietitian & Researcher, Radboud University Nijmegen Medical Centre, The Netherlands

14.40 pm

Exercise in neuromuscular disease: a moving topic

Nicole Voet, Rehabilitation Physician & Researcher, Radboud University Medical Center, The Netherlands

15.00 pm	Panel discussion and questions	
15.30 pm	Refreshments & Posters	
SESSION THREE: New technologies and platforms		
16.00 pm	Deep learning and imaging recognition Jordi Díaz-Manera, M.D. Associate Professor, Muscular Diseases Unit, Hospital de la Santa Creu I Sant Pau, Barcelona	<i>Chairpersons:</i> Volker Straub Jordi Diaz
16.20 pm	Supporting the collection of post-marketing surveillance data Craig Campbell, M.D. Head Division of Pediatric Neurology and TGDOC Chair, Western University, Canada	
16.40 pm	The EURO-NMD Clinical Patient Management System (CPMS) Teresinha Evangelista, M.D. Hospital Practitioner, Researcher and EURO-NMD Coordinator, INSERM Institute of myology, France	
17.00 pm	Panel discussion and questions	
19.30 pm	Evening meal (Buffet style)	

WEDNESDAY 11TH DECEMBER

SESSION FOUR: Personalised medicine		
09.00 am	Case study of personalized medicine	<i>Chairpersons:</i> Annemieke Aartsma-Rus Volker Straub
09.20 am	CRISPR-Based Genome Editing for Duchenne Muscular Dystrophy – Successes Charles Gersbach, Associate Professor, Duke University, Durham NC, United States	
09.40 am	CRISPR-Based Genome Editing for Duchenne Muscular Dystrophy - Challenges Annemieke Aartsma-Rus, Professor of Translational Genetics, Leiden University Medical Center, The Netherlands	
10.00 am	Panel discussion and questions	
10.30 am	Refreshments	
SESSION FIVE: Access to drug treatment		
11.00 am	EURORDIS Yann le Cam, Chief Executive Officer, EURORDIS Rare Diseases Europe, Paris France	<i>Chairpersons:</i> Ria Broekgaarden Ans van der Ploeg
11.20 am	Title – TBC	
11.40 am	Perspectives of a Clinician and Patients Ans van der Ploeg, Medical Doctor, Erasmus MC University Centre, The Netherlands & Ria Broekgaarden, Patient Representative, Spierziekten Nederland, The Netherlands	
12.00 pm	Panel discussion and questions	
12.30 pm	Lunch	
13.15 pm	Lunchtime Symposia (Location: Grote Zaal)	Networking & Posters (Location: Conelis Schuytzaal)
13:15 – 13:30 pm Santhera		

13:30 – 13:40 pm **PTC**
13:40 – 13:50 pm **Roche**

SESSION SIX: **Pregnancy in neuromuscular disorders**

14.00 pm

Frequent questions raised by patients before pregnancy

Zohar Argov, M.D. Professor of Neurology, Hadassah-Hebrew University Medical Center, Israel

Chairpersons:
Zohar Argov
Sabine Rudnik-Schöneborn

14.20 pm

Personal experience of pregnancy with progressive myopathy

Yuriko Oda, Patient advocate, Patient Association for Distal Myopathies (PADM), Japan

14.40 pm

Lessons learned from pregnancies in myotonic dystrophies

Sabine Rudnik-Schöneborn, M.D. Professor of Clinical Genetics, Department of Human Genetics, Medical University of Innsbruck, Austria

15.00 pm

Panel discussion and questions

15.30 pm

Refreshments

SESSION SEVEN: **Learning from successes of clinical trials**

16.00 pm

Learning from CMT trials in adults and children

David Cornblath, M.D. Professor of Neurology, Johns Hopkins University School of Medicine, USA

Chairpersons:
Linda Lowes
Stojan Peric

16.20 pm

Challenges and limitations of re-using outcome measures and trial design

Lindsay Alfano, Research Physical Therapist, Nationwide Children's Hospital, USA

16.40 pm

Patient reported outcome measures in neuromuscular diseases: obstacles and perspectives

Stojan Peric, M.D. Assistant Professor of Neurology, Neurology Clinic, Clinical Centre of Serbia, Faculty of Medicine, University of Belgrade, Serbia

17.00 pm

Panel discussion and questions

17.30 pm

CLOSE OF MEETING