

TREAT-NMD International Conference 2019

Monday 9 – Wednesday 11 December 2019

Leiden Stadsgehoorzaal

Breestraat 60, 2311 CS Leiden, The Netherlands



MONDAY 9TH DECEMBER

17.00 -
19.00 pm **Registration**

18.00 -
19.30 pm **Drinks reception**

TUESDAY 10TH DECEMBER

All main sessions will take place in the Grote Zaal

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 just a scientist is not enough**
Mayana Zatz, Professor of Genetics, University of São Paulo, Brazil

10.00 am **Discussion**

10.15 am **Refreshments & Posters**

SESSION ONE: Patient engagement

10.45 am **Patient engagement in medicine development**
Kees van der Graaf, Chairman of FSHD Unlimited, The Netherlands
&
Ingrid de Groot, Patient Representative, The Netherlands

Chairpersons:
Ria Broekgaarden
Fabiola Bertinotti

11:45 am **Panel discussion and questions**

12.15 pm **Lunch**

13.15 pm **Lunchtime Symposia**
(Location: Grote Zaal)

Networking & Posters
(Location: Conelis Schuytzaal)

13:15 - 13.35 pm **Sarepta**

Evolution of Registries in Neuromuscular Disorders
Dr Michela Guglieri, Consultant Neurologist, John Walton Muscular Dystrophy Research Centre, Newcastle University

13:35 – 13:50 pm **Biogen**

Biogen's vision to transform the therapeutic landscape of neuromuscular diseases
Dr Wildon Farwell, VP, Late Stage Unit Head for Pain, Neuromuscular & Rare Disease

SESSION TWO: Adults with progressive neuromuscular disease

14.00 pm	Take a deep breath and cough! Inspiratory muscle training and cough assistance in NMD Anri Human, Lecturer & Researcher, Physiotherapy Department, Sefako Makgatho Health Sciences University, South Africa	<i>Chairpersons:</i> 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 Nijmegen 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 Díaz-Manera
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 17.30 pm	Panel discussion and questions	
19.30 23:00 pm	Evening meal (Buffet style)	

WEDNESDAY 11TH DECEMBER

All main sessions will take place in the Grote Zaal

SESSION FOUR: Personalised medicine

09.00 am	Patient-customized oligonucleotide therapy for rare genetic diseases Jinkuk Kim, Research Fellow, Boston Children's Hospital and Harvard Medical School, USA	<i>Chairpersons:</i> Annemieke Aartsma-Rus Volker Straub
09.20 am	Opportunities and challenges for personalised medicine Annemieke Aartsma-Rus, Professor of Translational Genetics, Leiden University Medical Center, The Netherlands	
09.40 am	CRISPR-Based Genome Editing for Duchenne Muscular Dystrophy – Successes Charles Gersbach, Associate Professor, Duke University, Durham NC, United States	
10:00am 10.30 am	Panel discussion and questions Refreshments & Posters	

SESSION FIVE: Access to drug treatment

11.00 am **EURORDIS vision and proposals to address current challenges of access to new treatments**
Yann le Cam, Chief Executive Officer, EURORDIS Rare Diseases Europe, Paris, France

Chairpersons:
Ria Broekgaarden
Ans van der Ploeg

11.20 am **Clinicians perspective on access to therapies**
Ans van der Ploeg, Professor in Pediatrics, Center for Lysosomal and Metabolic Diseases & Pompe Center, Erasmus MC University Medical Center, Rotterdam, The Netherlands

11.40 am **Parent and patient perspective on access to therapies**
Huub van Rijswijk VSN, Member Diagnose group SMA 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**

DMD: Beyond the wheelchair

Dr Erik Niks, Consultant Paediatric Neurologist, Leiden University Medical Center

13:30 – 13:40 pm **PTC Therapeutics**

A science-led, patient-centric approach to treating rare diseases

Dr. Christian Werner, Executive Director Medical Affairs, PTC Therapeutics

13:40 – 13:50 pm **Roche**

How do we set the right expectations when treating SMA

Nathalie Goemans M.D. PhD, Paediatric Neurologist, University Hospitals Leuven

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 **Pregnancy in women with muscle weakness – impossible is nothing**
Sabine Rudnik-Schöneborn, M.D. Senior Clinical Geneticist, Department of Human Genetics, Medical University of Innsbruck, Austria

15.00 pm **Panel discussion and questions**

15.30 pm **Refreshments & Posters**

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

Chairpersons:

Linda Lowes

Stojan Peric

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

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

CLOSE OF MEETING

17.30

Volker Straub, TREAT-NMD Executive Committee member, John Walton

17.45 pm

Muscular Dystrophy Research Centre, Newcastle University