# TREAT-NMD International Conference 2019

## Monday 9th December

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>17.00 - 19.00 pm</td>
<td>Registration</td>
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<tr>
<td>18.00 – 19.30 pm</td>
<td>Drinks reception</td>
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## Tuesday 10th December

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<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>08.00 am</td>
<td>Registration</td>
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<tr>
<td>09.00 am</td>
<td><strong>WELCOME</strong></td>
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<tr>
<td>09.00 am</td>
<td>Annemieke Aartsma-Rus, Professor of Translational Genetics, Leiden University Medical Center, The Netherlands</td>
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<tr>
<td>09.15 am</td>
<td><strong>KEYNOTE LECTURE</strong></td>
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<tr>
<td>09.15 am</td>
<td>Mayana Zatz, Professor of Genetics, University of São Paulo, Brazil</td>
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<tr>
<td>10.00 am</td>
<td>Discussion</td>
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<tr>
<td>10.15 am</td>
<td>Refreshments</td>
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<tr>
<td>10.45 am</td>
<td><strong>SESSION ONE: Patient engagement</strong></td>
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<tr>
<td>10.45 am</td>
<td><strong>Patient engagement in medicine development</strong></td>
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<tr>
<td>10.45 am</td>
<td>Kees van der Graaf, Chairman of FSHD Unlimited, The Netherlands &amp;</td>
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<td>Ingrid de Groot, Patient Representative, The Netherlands</td>
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<tr>
<td>11.45 am</td>
<td>Panel discussion and questions</td>
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<tr>
<td>12.15 pm</td>
<td>Lunch</td>
</tr>
<tr>
<td>13.15 pm</td>
<td><strong>Lunchtime Symposia</strong></td>
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<tr>
<td>13:15 - 13.35 pm</td>
<td>Sarepta</td>
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<td></td>
<td><strong>DMD: Beyond the wheelchair</strong></td>
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<td></td>
<td>Dr Erik Niks, Consultant Paediatric Neurologist, Leiden University Medical Center</td>
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<tr>
<td>13:35 – 13:50 pm</td>
<td>Biogen</td>
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<tr>
<td></td>
<td><strong>Biogen’s vision to transform the therapeutic landscape of neuromuscular diseases</strong></td>
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<td></td>
<td>Dr Wildon Farwell, VP, Late Stage Unit Head for Pain, Neuromuscular &amp; Rare Disease</td>
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## Session Two: Adults with progressive neuromuscular disease

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>14.00 am</td>
<td>Networking &amp; Posters (Location: Conelis Schuytzaal)</td>
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<tr>
<td>14.15 am</td>
<td><strong>SESSION TWO: Adults with progressive neuromuscular disease</strong></td>
</tr>
<tr>
<td>14.15 am</td>
<td><strong>Sarepta</strong></td>
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<tr>
<td>14.30 am</td>
<td><strong>DMD: Beyond the wheelchair</strong></td>
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<tr>
<td>14.30 am</td>
<td>Dr Erik Niks, Consultant Paediatric Neurologist, Leiden University Medical Center</td>
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<tr>
<td>14.45 am</td>
<td><strong>Biogen</strong></td>
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<tr>
<td>15.00 am</td>
<td><strong>Biogen’s vision to transform the therapeutic landscape of neuromuscular diseases</strong></td>
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<tr>
<td>15.00 am</td>
<td>Dr Wildon Farwell, VP, Late Stage Unit Head for Pain, Neuromuscular &amp; Rare Disease</td>
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<td>Time</td>
<td>Session</td>
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<tr>
<td>14.00 pm</td>
<td><strong>Take a deep breath and cough!</strong> <em>Inspiratory muscle training and cough assistance in NMD</em></td>
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<tr>
<td>14.20 pm</td>
<td><strong>Nutritional assessment and interventions in adult NMD</strong></td>
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<tr>
<td>14.40 pm</td>
<td><strong>Exercise in neuromuscular disease: a moving topic</strong></td>
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<tr>
<td>15.00 pm</td>
<td><strong>Panel discussion and questions</strong></td>
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<tr>
<td>15.30 pm</td>
<td><strong>Refreshments &amp; Posters</strong></td>
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**SESSION THREE: New technologies and platforms**

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<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
<th>Chairpersons</th>
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<tbody>
<tr>
<td>16.00 pm</td>
<td><strong>Deep learning and imaging recognition</strong></td>
<td>Jordi Diaz-Manera, M.D. Associate Professor, Muscular Diseases Unit, Hospital de la Santa Creu i Sant Pau, Barcelona</td>
<td>Volker Straub, Jordi Diaz</td>
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<tr>
<td>16.20 pm</td>
<td><strong>Supporting the collection of post-marketing surveillance data</strong></td>
<td>Craig Campbell, M.D. Head Division of Pediatric Neurology and TGDOC Chair, Western University, Canada</td>
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<tr>
<td>16.40 pm</td>
<td><strong>The EURO-NMD Clinical Patient Management System (CPMS)</strong></td>
<td>Teresinha Evangelista, M.D. Hospital Practitioner, Researcher and EURO-NMD Coordinator, INSERM Institute of myology, France</td>
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<tr>
<td>17.00 pm</td>
<td><strong>Panel discussion and questions</strong></td>
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<tr>
<td>17.30 pm</td>
<td><strong>Evening meal (Buffet style)</strong></td>
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**WEDNESDAY 11TH DECEMBER**

**SESSION FOUR: Personalised medicine**

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<tbody>
<tr>
<td>09.00 am</td>
<td><strong>Case study of personalized medicine</strong></td>
<td>Speaker TBC</td>
<td>Annmieke Aartsma-Rus, Volker Straub</td>
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<td>09.20 am</td>
<td><strong>CRISPR-Based Genome Editing for Duchenne Muscular Dystrophy – Successes</strong></td>
<td>Charles Gersbach, Associate Professor, Duke University, Durham NC, United States</td>
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<tr>
<td>09.40 am</td>
<td><strong>CRISPR-Based Genome Editing for Duchenne Muscular Dystrophy - Challenges</strong></td>
<td>Annemieke Aartsma-Rus, Professor of Translational Genetics, Leiden University Medical Center, The Netherlands</td>
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<td>10.00 am</td>
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<td>10.30 am</td>
<td><strong>Refreshments</strong></td>
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**SESSION FIVE: Access to drug treatment**

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<tbody>
<tr>
<td>11.00 am</td>
<td><strong>EURORDIS vision and proposals to address current challenges of access to new treatments</strong></td>
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<td>Ria Broekgaardhen</td>
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<tr>
<td>11.20 am</td>
<td><strong>Patient and clinician perspective on access to therapy</strong>&lt;br&gt;Ans van der Ploeg, Professor in Pediatrics, Center for Lysosomal and Metabolic Diseases &amp; Pompe Center, Erasmus MC University Medical Center, Rotterdam, The Netherlands &amp;&lt;br&gt;Ria Broekgaarden VSN, Patient Representative, Spierziekten Nederland, The Netherlands</td>
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<td>11.40 am</td>
<td><strong>Parent and patient perspective on access to therapy</strong>&lt;br&gt;Huub van Rijswijck VSN, Member Diagnose group SMA Spierziekten Nederland, The Netherlands</td>
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<td>Lunch</td>
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<tr>
<td>13.15 pm</td>
<td><strong>Lunchtime Symposia</strong>&lt;br&gt;(Location: Grote Zaal)<strong>&lt;br&gt;Santhera 13:15 – 13:30 pm&lt;br&gt;PTC Therapeutics 13:30 – 13:40 pm&lt;br&gt;A science-led, patient-centric approach to treating rare diseases&lt;br&gt;Dr. Christian Werner, Executive Director Medical Affairs, PTC Therapeutics 13:40 – 13:50 pm&lt;br&gt;Roche</strong>&lt;br&gt;How do we set the right expectations when treating SMA&lt;br&gt;Nathalie Goemans M.D. PhD, Paediatric Neurologist, University Hospitals Leuven</td>
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<td>14.00 pm</td>
<td><strong>SESSION SIX: Pregnancy in neuromuscular disorders</strong>&lt;br&gt;Frequent questions raised by patients before pregnancy&lt;br&gt;Zohar Argov, M.D. Professor of Neurology, Hadassah-Hebrew University Medical Center, Israel&lt;br&gt;Personal experience of pregnancy with progressive myopathy&lt;br&gt;Yuriko Oda, Patient Advocate, Patient Association for Distal Myopathies (PADM), Japan&lt;br&gt;Pregnancy in women with muscle weakness – impossible is nothing&lt;br&gt;Sabine Rudnik-Schöneborn, M.D. Senior Clinical Geneticist, Department of Human Genetics, Medical University of Innsbruck, Austria&lt;br&gt;Panel discussion and questions&lt;br&gt;Chairpersons: Zohar Argov, Sabine Rudnik-Schöneborn</td>
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<tr>
<td>15.00 pm</td>
<td>Refreshments</td>
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<tr>
<td>16.00 pm</td>
<td><strong>SESSION SEVEN: Learning from successes of clinical trials</strong>&lt;br&gt;Learning from CMT trials in adults and children&lt;br&gt;David Cornblath, M.D. Professor of Neurology, Johns Hopkins University School of Medicine, USA&lt;br&gt;Challenges and limitations of re-using outcome measures and trial design&lt;br&gt;Chairpersons: Linda Lowes, Stojan Peric</td>
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Lindsay Alfano, Research Physical Therapist, Nationwide Children’s Hospital, USA

**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

**Panel discussion and questions**

**CLOSE OF MEETING**

Vice chair of TREAT-NMD