



**WMS25: Virtual Congress**  
30th September - 2nd October 2020

**Preliminary Summary Programme**  
All times are in British Summer Time (BST)

**Monday, 28 September 2020**

14:50 – 15:00	<b>President’s Introduction</b>
15:00 – 16:30	<b>WMS Industry symposia 1 and 2</b> <i>(2 x 90 minute parallel sessions incorporating live Q&amp;A)</i>
16:30 – 17:00	<b>Comfort break</b>
17:00 – 18:30	<b>WMS Industry symposia 3 and 4</b> <i>(2 x 90 minute parallel sessions incorporating live Q&amp;A)</i>
18:30 – 19:00	<b>Comfort break</b>
19:00 – 20:30	<b>WMS Industry symposia 5 and 6</b> <i>(2 x 90 minute parallel sessions incorporating live Q&amp;A)</i>
	E-Posters available to view on demand

**Tuesday, 29 September 2020**

15:00 - 22:00	<b>Pre-Congress Teaching Course (congress registration required)</b> Congress Industry Symposia and all E-Posters available to view on demand
---------------	--

**Wednesday, 30 September 2020**

15:00-15:15	<b>President’s Welcome</b>
15:15-15:45	<b>Opening lecture</b> <b>COVID-19 for NMD, telemedicine, changing practice in the pandemic situation</b>
	The COVID-19 pandemic has so far not been the largest, or the most lethal pandemic of the last 100 years, but the global response to it has been unprecedented, both in medical and in societal terms. It has led to a significant change in behaviour, specifically in the way medicine is practised, and its longer- term effects cannot yet be predicted confidently. The concerns for people with neuromuscular disease specifically are significant: in addition to potentially being a risk group for severe infection, they are put at risk by an interruption to their access to treatment. The World Muscle Society is making an effort to help them face these challenges, by providing advice for patients and carers, trying to define best practice parameters, and suggest avenues to manage the future changes in medical practice.

This lecture discusses the approach, its effects on patients, and potential future developments in telemedicine.

*Speaker: Max Damian*

*Session Moderator: Jiri Vajsar*

**15:45-16:00**

**Comfort break**

16:00-16:30

**Panel discussion 1**

**Pre-clinical requirements for novel neuromuscular therapies**

It is a unique time for neuromuscular disorders, as an unprecedented number of therapeutic candidates are entering clinical trial for a range of diseases. With this surge of potential treatments, the importance of pre-clinical models and rigor in pre-clinical drug development has never been more important. It is critical to evaluate study designs, predictive power, reliability and relevance of current animal models in assessing the efficacy and safety of emerging therapies. In this panel, we have assembled four world leading experts on the application of pre-clinical models to therapy development. We will present key ideas related to best practice and discuss important pitfalls that have plagued pre-clinical drug programs. We will also identify current gaps in the field and offer ideas for future improvements.

*Speakers: Annamaria DeLuca, Kanneboyina Nagaraju, Dominic Wells, Jennifer Morgan*

*Session Moderator: Jim Dowling*

*Content Moderator: Grace Yoon*

16:30-16:45

**Panel discussion 1 – Live Q&A**

**16:45-17:15**

**Comfort break, poster viewing, sponsors & exhibition, and chat room**

17:15-17:45

**Panel discussion 2**

**Gene/ advanced/ new therapeutics**

Gene and transcript directed therapies are rapidly becoming a reality and a promise for many neuromuscular disorders. Various such therapies are in the clinic, in clinical trials, or in advanced preclinical development. This panel will discuss opportunities and challenges of these therapies, with a focus on AAV mediated gene therapy. Determinants of success and potential failure will be discussed, touching on issues such as AAV capsids, biodistribution, therapeutic windows of opportunity and durability of expression, seropositivity and redosing, immune mediated toxicity and immunomodulation, as well as pathways and roadblocks for ultrarare applications. This will be an open discussion drawing on recent examples and experiences in the field of clinical neuromuscular gene therapy.

*Speakers: Carsten Bönnemann, Francesco Muntoni, Barry Byrne, Ana Buj Bello*

*Session Moderator: Kathryn Wagner*

*Content Moderator: Chris Weihl*

17:45-18:00

**Panel discussion 2 – Live Q&A**

18:15- 19:15

**WMS 25th Anniversary Quiz and chat room**

## **Thursday, 1 October 2020**

15:00-16:00

**New genes in neuromuscular diseases – Selected oral presentations 1 (O.1-4)**

(4 x 10 mins consecutive presentations plus 20 mins live Q&A)

Muscle researchers have identified novel genes and their variants that may be causative to neuromuscular disorders, assess these with transgenic studies, sequencing, and phenotypic and molecular correlation, etc., and discuss future studies and outlook.

*Session Moderator: Gisèle Bonne*

*Assistant Moderator: Hernán Gonorazky*

**16:00-16:30**

**Comfort break, poster viewing, sponsors & exhibition, and chat room**

16:30-17:30	<p><b>OMICs and AI approaches for muscle diseases - Selected oral presentations 2 (O.5-8)</b>  (4 x 10 mins consecutive presentations plus 20 mins live Q&amp;A)  <i>Session Moderator: Werner Stenzel</i>  <i>Assistant Moderator: Louise Benarroch</i></p>
17:30-19:30	<p><b>Comfort break, poster viewing and sponsors &amp; exhibition</b></p>
17:30-19:30	<p><b>Poster session 1</b>  Autophagic myopathies / Myofibrillar Myopathies / Distal myopathies / Pompe disease (P.1-17)  Congenital myopathies 1 – Nemaline (P.18-30)  Myasthenia &amp; related disorders (P.31-39)  DMD &amp; BMD – Clinical (P.40-60)  Muscle function &amp; homeostasis / Molecular therapeutic approaches (P.62-77)  SMA - Clinical (P.78-92)  Late Breaking Posters (LBP 1–14)</p> <p><b>Poster session 2</b>  Congenital myopathies 2 (P.93-104)  Hereditary neuropathies &amp; ALS (P.105-117)  DMD - Biomarkers &amp; Outcomes measures (P.118-134)  Limb Girdle Muscular Dystrophies (P.135-156)  Muscle Imaging – MRI (P.157-170)  SMA: Registries, Biomarkers &amp; Outcome measures (P.171-191)  Late Submitted Posters (LSP 1-26)</p> <p><b>Poster session 3</b>  Congenital muscular dystrophies (P.192-203)  DMD - Animal models &amp; Preclinical treatment (P.204-222)  FSHD / OPMD / Myotonic dystrophy (P.224-241)  Outcome measures (P.242-252)  SMA – Therapy (P.253-279)</p> <p><b>Poster session 4</b>  DMD - Therapy (P.280-294)  Autoimmune myopathies (P.295-312)  Mitochondrial diseases &amp; Metabolic myopathies (P.313-324)  New genes and diseases / NGS &amp; related techniques (P.325-334)  Registries, Care, Quality of Life, management of NMD (P.335-349)</p>
19:30-20:30	<p><b>From the spinal cord to the muscle - Selected oral presentations 3 (O.9-12)</b>  (4 x 10 mins consecutive presentations plus 20 mins live Q&amp;A)</p> <p>Molecular and clinical assessments of spinal cord disease, sarcoglycanopathy, and dysferlinopathy are discussed, along with genetic therapy for the latter.</p> <p><i>Session Moderator: John Vissing</i>  <i>Assistant Moderator: Mo Zhao</i></p>
20:30-21:00	<p><b>Comfort break, poster viewing, sponsors &amp; exhibition, and chat room</b></p>
21:00–22:00	<p><b>Highlights across Myology - Selected oral presentations 4 (O.13-16)</b>  (4 x 10 mins consecutive presentations plus 20 mins live Q&amp;A)</p> <p>From basic science to clinical research, investigators use a variety of approaches to assess NM disease, including calcium studies, gene editing, large-scale electron microscopy, and pre-conception carrier screening.</p> <p><i>Session Moderator: Mariz Vainzof</i>  <i>Assistant Moderator: Nicolas Chrestian</i></p>

## Friday, 2 October 2020

15:00-15:45

### **Poster highlights (PH1-6)**

(6 x 5 mins consecutive presentations plus 15 mins live Q&A)

This is one of the most popular sessions of the annual WMS Congress. The session moderators, with support from the programme committee, select 6 posters for short oral presentations that they feel are of particular interest to the neuromuscular community. The poster may address controversial or polarising topics or topics that aren't regularly covered in plenary lectures, but are nevertheless scientifically sound and relevant.

*Session Moderators: Nicol Voermans and Ichizo Nishino*

15:45-16:15

### **Comfort break, poster viewing, sponsors & exhibition, and chat room**

16:15-17:00

### **Late Breaking News Session (LBO 1-3)**

(3 x 10 mins consecutive presentations plus 15 mins live Q&A)

One of the most exciting sessions of the annual WMS Congress, this sessions provides a platform for still unpublished, high quality and high impact research findings that are of great interest to all congress participants. Presentations often focus on new disease causes, pathomechanisms and therapies.

*Session Moderators: Laurent Servais and Kevin Flanigan*

17:00-17:15

### **Comfort break, poster viewing and sponsors & exhibition**

17:15-18:15

**Prize presentations:** *Nicol Voermans*

**Close of conference:** *Volker Straub*

**Introduce 2021 and presentation of flag:** *Jana Haberlová*