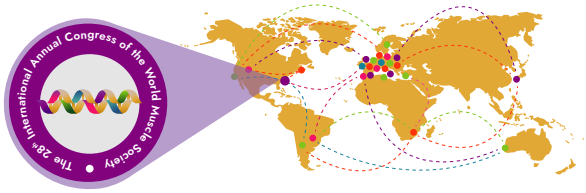

WMS2023
3rd-7th October 2023 Charleston, USA
The 28th Annual Congress of the World Muscle Society

WMS 2023 Summary Programme

Monday 2nd October 2023

08:30-19:00	**Pre-Congress Teaching Course** (separate registration required)

Tuesday 3rd October 2023

08:00-11:00	**Pre-Congress Teaching Course** (separate registration required)	
11:00-16:00	**WMS Executive Board Meeting** 📍 Meeting Room	
15:30-18:00	**Registration** 📍 Ballroom Foyer, **refreshments** 📍 PAC Foyer and Exhibit Hall **and poster set up** 📍 Ballroom Foyer	
16:30-17:30	**Industry Symposium 1** 📍 PAC	**Industry Symposium 2** 📍 Exhibit Hall A1
18:00-18:45	**Opening Ceremony** 📍 PAC INV01: The strength to explore: a review of NASA experience with muscle atrophy in space *Don Thomas, Ohio Astronaut, USA* *Moderators: Lindsay Alfano & Chris Weihl*	
18:45-21:00	Networking Reception 📍 Congress Venue (separate registration required)	

Wednesday 4th October 2023

06:30-19:30	**Registration desk open**	
07:30-08:30	**Industry Symposium 3** 📍 PAC	**Industry Symposium 4** 📍 Exhibit Hall A1
09:00-09:15	**Congress Welcome** 📍 PAC Message from the President	
09:15-10:45	📍 PAC **Topic 1: Understanding phenotypic and genetic diversity in neuromuscular disorders 1** *Moderators: Gina Ravenscroft, University of Western Australia, Australia & Marco Savarese, University of Helsinki, Finland* 09:15-09:45 INV02: High throughput functional assays to improve interpretation of rare variants discovered in Neuromuscular disease genes *Monkol Lek, Yale School of Medicine, USA* 09:45-10:15 INV03: Understanding genetic variants in neuromuscular disorders *Chris Weihl, Washington University in St. Louis, USA*	

	10:15-10:30 **O01: Clinical spectrum and molecular features of asymptomatic and paucisymptomatic DMD mutations** *Stefan Nicolau, Nationwide Children's Hospital, USA* 10:30-10:45 **O02: Unpacking gene expression profile to the single nuclei level in humam muscle Pompe samples** *Jordi Diaz-Manera, Newcastle University, UK*		
10:45-11:15	**Morning refreshments & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom		
10:45-11:15	**Social Media Committee** find out about how to get involved 📍 Myology Café, Exhibit Hall		
11:15-13:15	📍 PAC **Topic 1: Understanding phenotypic and genetic diversity in neuromuscular disorders 2** *Moderators: Gisèle Bonne, Centre de Recherche en Myologie, France & Charlotte Lilien, MDUK Oxford Neuromuscular Centre, UK* 11:15-11:45 **INV04: Accounting for phenotypic variability in clinical outcome assessments** *Lindsay Alfano, Nationwide Children's Hospital, USA* 11:45-12:15 **INV05: Engaging patients from diverse backgrounds in NMD research** *Gita Ramdharry, UCL Queen Square, Institute of Neurology, UK* 12:15-12:30 **O03: In vivo gene therapy for striated muscle laminopathy** *Mariko Okubo, Institut de Myologie, France* 12:30-12:45 **O04: Myoguide.org: a web-based portal supporting the analysis of MRIs for the diagnosis of neuromuscular patients** *Carla Bolaño Diaz, John Walton Muscular Research Centre, UK* 12:45-13:00 **O05: TDP-43 dependent cryptic exon derived neoepitopes as a novel diagnostic biomarker in muscle biopsies of inclusion body myositis patients** *Chiseko Ikenaga, Johns Hopkins University School Of Medicine, USA* 13:00-13:15 **O06: Clinical trial readiness and validation of onsite and remote evaluation in valosin containing protein-associated multisystem proteinopathy: A 24-month longitudinal study** *Natalie Reash, Nationwide Children's Hospital, USA*		
13:15-14:30	**Lunch & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom		
13:45-14:15	**New WMS Members Event** 📍 Myology Café, Exhibit Hall		
14:30-15:30	**Poster session 1** 📍 Ballroom A-C *Refreshments served*		
	P41-P42, VP43, P44-P47, VP48, P49-P54, VP55: Clinical trial highlights **P56-P57, P59-P67: Therapies for neuromuscular disorders** **VP114-VP116, P117, VP118-VP119, P120-P126 Muscle MRI** **P127-P151: DMD - imaging and outcome measures** **P205-P212, VP213, P214-P215, VP216, P217-P225, VP226, P227, VP228, P229-P234 SMA - clinical** **P319-P333, VP334, P335-P338, VP339, P340: Myositis**		
15:30-16:00	**Short Oral Presentations 1** 📍 Ballroom C1 **P319-P322, P126-P127** *Moderator: Tahseen Mozaffar, University of California, USA*	**Short Oral Presentations 2** 📍 Ballroom C2 **P205-210** *Moderator: Laurent Servais, University of Oxford, UK*	**Short Oral Presentations 3** 📍 Ballroom C3 **P211-P212, P56, P57, P59** *Moderator: Jana Haberlová, Motol University Hospital, Czech Republic*

16:15-17:00	PAC **Debate: Is the muscle biopsy still indicated?** *Moderators: Teerin Liewluck, Mayo Clinic-Rochester, USA & Edoardo Malfatti, Paris Est University/INSERM U955, France* **INV06:** *Teresinha Evangelista, Institut de Myologie, France* **INV07:** *Baziel van Engelen, Radboud University Medical Centre, The Netherlands*		
17:15-18:15	**Poster session 2** Ballroom A-C *Refreshments served*		
	P12-P18, P20-P37, VP38, P39-P40: DMD - treatments **P168-P177, VP178, P179-P187, VP188, P189-P190: Genetics of neuromuscular disorders** **P281-P285, VP286, P287-P288, VP289, P290, VP291, P292-P305, VP306, P307: Limb-girdle muscular dystrophies** **P308-P316, VP317, P318: Facioscapulohumeral muscular dystrophy** **P398-P413, VP414, P415, VP416-VP417, P418-P420: Congenital myopathies**		
18:15-18:45	**Short Oral Presentations 4** Ballroom C1 **P398-P403** *Moderator: Anna Sarkozy, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital, UK*	**Short Oral Presentations 5** Ballroom C2 **P168, P281-P285** *Moderator: Meredith James, John Walton Muscular Dystrophy Research Centre, UK*	**Short Oral Presentations 6** Ballroom C3 **P169-P172, P308-P309** *Moderator: Vishnu Venugopalan Thampy Yamuna, All India Institute of Medical Sciences, India*
19:15-20:15	**Industry Symposium 5** PAC	**Industry Symposium 6** Exhibit Hall A1	

Thursday 5th October 2023

07:00-15:00	**Registration desk open**	
08:00-09:00	**Industry Symposium 7** PAC	**Industry Symposium 8** Exhibit Hall A1
09:30-11:00	PAC **Topic 2: Pathobiology of neuromuscular expansion disorders 1** *Moderators: Ichizo Nishino, National Institute of Neuroscience, NCNP, Japan & Louise Benarroch, Centre De Recherche En Myologie, France* 09:30-10:00 **INV08: RAN translation in C9orf72 ALS/FTD and other repeat opportunities** *Laura Ranum,University of Florida, USA* 10:00-10:30 **INV09: Novel repeat disorders in muscle disease: the emergence of OPDM** *Zhaoxia Wang, Peking University First Hospital, China* 10:30-10:45 **O07: RAN translation of expanded CGG repeat in LRP12 may contribute to oculopharyngodistal myopathy,** *Chengcheng Li, Washington University in St. Louis, USA* 10:45-11:00 **O08: Single-nucleus RNA sequencing reveals characteristic gene expression in pathologically-specific myofibers in oculopharyngodistal myopathy** *Nobuyuki Eura, Nara Medical University, Japan*	

11:00-11:30	**Morning refreshments & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom
11:00-11:30	**Guidelines Committee** find out about how to get involved 📍 Myology Café, Exhibit Hall
11:30-13:30	📍 PAC **Topic 2: Pathobiology of neuromuscular expansion disorders 2** *Moderators: Gauthier Remiche, Hopital Erasme, Belgium & Nicol Voermans, Radboud University Medical Center, The Netherlands* 11:30-12:00 **INV10: Genetic discovery and pathomechanism of repeat disorders in neuromuscular diseases: lessons from RFC1** *Henry Houlden, UCL Queen Square, Institute of Neurology, UK* 12:00-12:30 **INV11: Motor neuron and muscle involvement in SBMA: therapeutic implications** *Kenneth Fischbeck, National Institutes of Health (NIH), USA* 12:30-12:45 **O09: Bi-allelic variants of FILIP1 cause congenital myopathy, dysmorphism and neurological defects** *Andreas Roos, University Medicine Essen, Germany* 12:45-13:00 **O10: Universal genomic newborn screening for early, treatable, and severe conditions- including 33 genes of NMD: Baby Detect** *Tamara Dangouloff, University Of Liege, Belgium* 13:00-13:15 **O11: Long-read nanopore sequencing in FSHD patients reveals CpG methylation patterns including methylation gradients in contracted D4Z4 arrays** *Russell Butterfield, University of Utah, USA* 13:15-13:30 **O12: Muscle imaging in natural history of FSHD: quantitative MRI and ultrasound results compared head-to-head** *Sanne Vincenten, Radboudumc, The Netherlands*
13:30-14:45	**Lunch & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom
13:45-14:45	**Career Development Workshop** 📍 Ballroom C1 (Lunch available in the room) *Moderator: Chris Weihl, Washington University in St. Louis, USA* *Panel: Meredith James, John Walton, Muscular Dystrophy Research Centre, UK,* *Mike Lawlor, Medical College of Wisconsin, USA,* *Coen Ottenheijm, Amsterdam UMC, The Netherlands* *and Carmen Paradas, Hospital Virgen del Rocío, Spain*
14:45-18:00	Poster viewing / Group Activity (separate registration required)
18:00-21:00	Group Activity Reception (separate registration required)

Friday 6th October 2023

06:45-18:00	**Registration desk open**
07:30-08:30	**Interesting Case Discussions** 📍 PAC (Refreshments available) *Cases presented by delegates* *Moderators: Reghan Foley, National Institute of Health, USA and Riyad El-Khoury, Genethon, France*
08:30-08:45	Comfort break
08:45-10:00	📍 PAC **Topic 3: The effect of lifestyle, exercise and nutrition on neuromuscular pathology and outcomes 1** *Moderators: Salman Bhai, UT Southwestern, USA & Jean-Yves Hogrel, Association Institut de Myologie, France*

	08:45-09:15 **INV12: The exposome in neuromuscular disorders** *Eva Feldman, The University of Michigan, USA* 09:15-09:45 **INV13: Development of a cycle training paradigm to improve exercise capacity and pathophysiology in boys with Duchenne muscular dystrophy,** *Tanja Taivassalo, University of Florida, USA* 09:45-10:00 **O13: Promoting an active lifestyle; use of an in-home body weight support system to increase exercise dosage for children with neuromuscular disease** *Megan Iammarino, Nationwide Children's Hospital, USA*
10:00-10:30	**Morning refreshments & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom
10:00-10:30	**Myology Developments Across the World and Education Committees** find out about how to get involved 📍 Myology Café, Exhibit Hall
10:30-12:00	📍 PAC **Topic 3: The effect of lifestyle, exercise and nutrition on neuromuscular pathology and outcomes 2** *Moderators: Linda Lowes, Nationwide Children's Hospital, USA & John Vissing, Rigshospitalet, Denmark* 10:30-11:00 **INV14: Physical activity and exercise are more than medicine for neuromuscular disorders** *Nicole Voet, Radboud University Medical Centre, The Netherlands* 11:00-11:15 **O14: Experiences with pregnancy and pregnancy-related physiotherapy in women with Charcot-Marie-Tooth disease. A qualitative interview study** *Andreas Rosenberger, National Neuromuscular Centre, Norway* 11:15-11:30 **O15: Large-scale proteomics profiling of peripheral blood of DM1 patients identifies biomarkers for disease severity and physical activity** *Peter-Bram 't Hoen, Radboud University Medical Center, The Netherlands* 11:30-11:45 **O16: New FDX2-loss of function phenotype presenting with blindness and myopathy with potential responsiveness to Co-enzyme Q10 analogs** *Reghan Foley, National Institute of Health, USA* 11:45-12:00 **O17: 6'-sialyllactose supplementation in GNE myopathy: a pilot and subsequent placebo-controlled study** *Young-eun Park, Pusan National University Hospital, South Korea*
12:15-13:15	**WMS General Assembly/Poster viewing for non-members** 📍 Exhibit Hall A1
13:00-14:00	**Lunch & exhibition** 📍 Exhibit Hall **and posters** 📍 Ballroom
13:30-14:00	**Sponsor Meeting** 📍 Meeting Room 10
14:00-15:00	**Poster session 3** 📍 Ballroom A-C *Refreshments served*
	P68-P70, VP71, P72-P81: SMA - outcome measures **P152-P162, VP163, P164-P166, VP167: Distal myopathies** **P191-P204: Registries** **P252-P258, VP259, P260, P262-P268: Dystrophinopathies** **P368, VP369, P370-P380, VP381, P382: Pompe disease**

	P383-P395, VP396, P397: Myotonic dystrophy P421-P426, VP427, P428, VP429, P430-P434, VP435-436: Congenital muscular dystrophies		
15:00- 15:30	**Short Oral Presentations 7** Ballroom C1 P152-157 *Moderator: Bjarne Udd, Tampere Neuromuscular Center, Finland*	**Short Oral Presentations 8** Ballroom C2 P158, P421-424 *Moderator: Payam Mohassel, Johns Hopkins University, USA*	**Short Oral Presentations 9** Ballroom C3 P159, P368, P383-384, P191-192 *Moderator: Carolina Tesi Rocha, Stanford University, USA*
15:30-16:30	**Poster session 4** Ballroom A-C *Refreshments served*		
	P01-P05,VP06-VP07, P08-P11: SMA - therapies P82-P113: Outcome measures P235-P250: DMD - clinical care and cases VP270, P271-P273, VP274, P275-P278, VP279, P280: Myasthenia gravis VP341, P342,VP343, P344-P348, VP349, P350-P356, VP357, P358-P367: Metabolic and mitochondrial myopathies P437-P446, VP447, P448: Motor neuron disease and neuropathies LBP01-LBP21, LBVP01-LBVP03: Late Breaking		
16:30-16:45	**Short Oral Presentations 10** Ballroom C1 P82-P87 *Moderator: Tina Duong, Stanford University, USA*	**Short Oral Presentations 11** Ballroom C2 P235, P01-P02 *Moderator: Jorge Alfredo Bevilacqua, Universidad de Chile & Clínica Dávila, Chile*	
18:00-18:30	Transport to Networking Pre-Dinner Drinks (separate registration required)		
18:30-19:30	Pre-Networking Dinner Sharehouse, Downtown Charleston (separate registration required)		
19:30-23:00	Networking Dinner The Bus Shed, Downtown Charleston (separate registration required)		

Saturday 7th October 2023

07:30-15:00	**Registration desk open**
07:30-09:00	**Arrival refreshments** PAC Foyer
07:45-08:45	**Clinical Trial Updates** PAC *Moderators: Kristl Claeys, Universitaire Ziekenhuizen Leuven, Belgium & Ulrike Schara Schmidt, University of Essen, Germany* O18: Topline Safety and Efficacy Data Analysis of Phase 1/2 Clinical Trial Evaluating AOC 1001 in Adults with Myotonic Dystrophy Type 1: MARINA™ *Nicholas Johnson, Virginia Commonwealth University, USA* O19: Preliminary Results from MLB-01-003: An Open Label Phase 2 Study of BBP-418 in Patients with Limb-girdle Muscular Dystrophy Type 2I/R9 *Amy Harper, Virginia Commonwealth University, USA* O20: Safety and efficacy of intravenous onasemnogene abeparvovec in patients with spinal muscular atrophy: interim findings from the phase 3 SMART study *Hugh McMillan, Children's Hospital of Eastern Ontario, USA* O21: 104-week efficacy and safety of cipaglucosidase alfa+miglustat in patients with late-onset Pompe disease previously treated with alglucosidase alfa *Tahseen Mozaffar, University of California, USA*

09:00-11:00	**The Victor Dubowitz Lecture** 📍 PAC *Moderators: Volker Straub, Newcastle University, UK & Chris Weihl, Washington University in St. Louis, USA* 09:00-09:30 **INV15 RNA-targeted therapy for ALS** *Tim Miller, Washington University, USA* **Poster Highlights** 📍 PAC *Moderators: Alan Beggs, Boston Childrens Hospital / Harvard Medical School, USA & Svetlana Gorokhova, National Institute of Health, USA* **O22: P81 Gastrointestinal assessment in Spinal Muscular Atrophy (SMA): the experience of SMA healthcare professionals in France** *Marta Gomez Garcia, APHP Raymond Poincare University Hospital, Child Neurology and Paediatric ICU Department Pediatrique, France* **O23: P161 Natural history of distal and myofibrillar myopathies assessed by clinical and technological outcome measures (Dista-Myo): baseline results** *Giorgio Tasca, Newcastle University, UK* **O24: P266 Gene expression profiles and spatial localisation of dystrophin isoforms in developing and adult human brain** *Francesco Catapano, University College London, UK* **O25: P325 A comparative single nuclei transcriptomics approach to evaluating the terminally differentiated lymphocytes in autoimmune Myositis** *Francia Victoria De Los Reyes, National Center of Neurology and Psychiatry (NCNP), Japan* **O26: P350 Clinical characteristics and therapeutic response of patients with adult-onset Multiple Acyl-CoA-Dehydrogenase Deficiency (MADD)** *Sofie Sunebo, Linköping University Hospital. Sweden* **O27: P425 Inhibition of TGFβ signaling pathway as a therapeutic approach in collagen VI-related muscular dystrophy** *Hailey Hearn, Johns Hopkins University, USA*
11:00-11:30	**Morning refreshments** 📍 PAC Foyer **and posters** 📍 Ballroom
11:30-13:30	**Late Breaking News** 📍 PAC *Moderator: Lindsay Wallace, Nationwide Children's Hospital, USA and Michele Yang, Children's Hospital Colorado, USA* **LBO01: Impaired iron-sulfur cluster assembly due to biallelic variants in CIAO1 leads to a novel muscle disease** *Rotem Or Bach, National Institute of Health, USA* **LBO02: Ablation of the Carboxiterminal end of MAMDC2 causesa distinctmuscular dystrophy** *Carmen, Paradas, Hospital Virgen del Rocío, Spain* **LBO03: A novel class of Tubulinopathies - Mutations in TUBA4A cause primary skeletal muscle disorders** *Mridul Johari, Harry Perkins Institute of Medical Research - UWA, Australia* **LBO04: CGG repeat expansion in LRP12 causes both amyotrophic lateral sclerosis and oculopharyngodistal myopathy type 1** *Takashi Kurashige, Nho Kure Medical Center and Chugoku Cancer Center, Japan* **LBO05: Proteomic serum profiling identifies ITIH3 as a new biomarker for Myasthenia gravis disease activity** *Tobias Ruck, Heinrich Heine University Düsseldorf, Germany* **LBO06: Functional improvements and decreased aggregate burden in TgT57I Mice following AAVrh74.tMCK.hBAG3 gene therapy** *Burcak Ozes, Nationwide Children's Hospital, USA* **LBO07: RNA-based CRISPRoff silencing to target DUX4 in Facioscapulohumeral muscular dystrophy** *Junjie He, Center For iPS Cell Research And Application (CiRA), Kyoto University, Japan* **LBO08: Identification of AAV variants with enhanced skeletal muscle and muscle stem cell transduction** *Melissa Spencer, UCLA David Geffen School of Medicine, USA* **LBO09: scAAV9.U7-ACCA treatment of DMD exon 2 duplication leads to significant dystrophin expression and evidence of clinical benefit, particularly following treatment as an infant** *Kevin Flanigan, The Research Institute at Nationwide Children's Hospital, USA*

	Prize Giving Ceremony PAC *Moderator: Johann Böhm* **Introduction to the WMS 2024 Congress, Prague, Czech Republic** *Jana Haberlová* **Handover of the WMS flag and close of congress** *Moderator: Volker Straub*
13:30-14:30	Homeward lunch PAC Foyer
13:30-15:00	**NMD Board Meeting** Meeting room 6 & 7 (separate registration required)