WMS 2024 Summary Programme

Version: 041024

Monday 7th October 2024

08:30-19:00	**Pre-Congress Teaching Course** Congress Venue, Club D+E (separate registration required)

Tuesday 8th October 2024

08:00-11:00	**Pre-Congress Teaching Course** Congress Venue, Club D+E (separate registration required)	
11:00-16:00	**WMS Executive Board Meeting** South Boardroom 1	
14:30-18:00	**Registration and poster set up** Congress Venue, Second Floor Foyers	
14:30-16:00	**Meet the Exhibitors** Congress Venue, Second Floor Foyers (Catering provided)	
16:30-17:30	**Industry Symposium 1** South Hall 1	**Industry Symposium 2** South Hall 2 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium
18:00-18:45	**Opening Ceremony** Congress Hall (separate registration required) *Moderators: Jana Haberlová, University Hospital Motol, Czechia & Radim Mazanec, Charles University, Czechia*	
18:45-21:00	**Networking Reception** Congress Venue, Panorama Hall and Zoom Room, First Floor (separate registration required)	

Wednesday 9th October 2024

06:30-19:30	**Congress desk open**	
07:45-08:45	**Industry Symposium 3** South Hall 1 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium	**Industry Symposium 4** South Hall 2 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium
09:00-09:15	**Congress Welcome - Message from the President** Congress Hall	
09:15-10:45	**Topic 1: Acquired Muscle Disorders** Congress Hall *Moderators: Suur Biliciler, Uthealth Science Center Houston, Mcgovern Medical School, USA & Heřman Mann, Institute of Rheumatology, Prague, Czechia* 09:15-09:45 **01INV** Acquired muscle disorders: Introduction to CART cells and Tregs *Armando Villalta, University of California, Irvine, United States of America* *continued on next page*	

	09:45-10:15 02INV Current and prospective uses of CAR and CAAR T-cell therapies in muscle disorders *Tahseen Mozaffar, University of California, Irvine, United States of America* **10:15-10:30 01O** Response to rozanolixizumab across treatment cycles in patients with generalised Myasthenia Gravis: A post hoc analysis *Sabrina Sacconi, Université Côte d'Azur* **10:30-10:45 02O** Characterization of a mouse model for Jo-1, PL-7 and PL-12 associated Anti-synthetase syndrome *Derya Bachir, Heinrich Heine University, Germany*	
10:45-11:15	**Morning refreshments, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	
10:45-11:15	**Myology Café - Meet the WMS EDI Committee** Congress Hall Foyer	
11:15-13:15	**Topic 1: Acquired Muscle Disorders** Congress Hall *Moderators: Ichizo Nishino, National Institute of Neuroscience, Japan & Corinna Preusse, Charité-universitätsmedizin Germany* **11:15-11:45 03INV** Idiopathic inflammatory myopathies: current state of the field, new insights and treatment *Jiří Vencovský, Charles University, Czechia* **11:45-12:15 04INV** Acquired muscle disease - a paediatric perspective *Sithara Ramdas, Oxford Children's Hospital, United Kingdom* **12:15-12:30 03O** Identification of Class I HLA genetic predispositions and prediction of autoantigenic epitopes in dermatomyositis patients of Indian origin *Bandana Jassal, All India Institute of Medical Sciences, India* **12:30-12:45 04O** In-silico Interactomics as a way to elucidate Inclusion Body Myositis *Francia Victoria De Los Reyes, National Institute of Neuroscience, Japan* **12:45-13:00 05O** Unravelling the role of early mitochondrial dysfunction in Inclusion Body Myositis: a chicken or egg dilemma reloaded *Felix Kleefeld, University of Cambridge, UK* **13:00-13:15 06O** NLRP3 inflammasome activation and altered mitophagy are key pathways in Inclusion Body Myositis. *Elie Naddaf, Mayo Clinic, USA*	
13:15-14:30	**Lunch, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	
13:15-14:30	**Meet the Experts Lunch** Zoom and Panorama Rooms, 1st Floor (separate registration required and lunch is provided)	

14:30-15:30	**Poster Session 1** Forum Hall (refreshments provided) **113P-146P, 147VP:** SMA Clinical **204P-231P, 232VP-233VP:** Clinical trials, access to health care and outcome measures **267P-279P:** Cell insights, muscle homeostasis **361P-400P:** DMD - imaging and outcome measures **538P-577P, 578VP:** Acquired, inflammatory, myositis **644P-655P:** Muscle MRI & new imaging techniques **688P-704P:** Therapies for NMD		
15:30-16:00	**Short Oral Presentations 1** North Hall **146P, 145P, 231P, 576P, 577P** *Moderator: Werner Stenzel, Charité University Hospital*	**Short Oral Presentations 2** Terrace 2A **278P, 703P, 702P, 279P, 704P** *Moderator: Perry Shieh, University of California*	**Short Oral Presentations 3** Terrace 2B **400P, 399P, 654P, 655P, 653P** *Moderator: Krista Vandenborne, University Of Florida*
16:15-17:00	**Debate: Can the costs of gene therapies in neuromuscular disorders be justified?** Congress Hall *Moderators: Francesco Muntoni, University College London, Great Ormond Street Hospital, UK & Teresinha Evangelista, Institut de Myologie, France* **14INV** *Olga Germanenko, SMA Family Foundation, Russia* **15INV** *Josie Godfrey, JG Zebra Consulting, United Kingdom*		
17:15-18:15	**Poster Session 2** Forum Hall (refreshments provided) **01P-51P, 52VP-54VP:** CM - CMD **79P-97P:** LGMD **401P-436P, 437VP:** DMD – treatments **472P-496P:** Genetics of NMD (new genes and NGS, diagnostic etc.) **579P - 592P, 593VP, 594P, 595P, 596VP, 597VP:** EDMD, OPDM, autophagic, extramuscular **619P-642P, 643VP:** FSHD **677P-686P, 687P:** RNA in NMD: clinical insights, pathomechanisms and treatments		
18:15-18:45	**Short Oral Presentations 4** North Hall **641P, 630P, 638P, 639P, 640P** *Moderator: Nicol Voermans, Radboud University Medical Centre*	**Short Oral Presentations 5** Terrace 2A **586P, 50P, 97P, 96P, 51P** *Moderator: Piera Smeriglio. Institut de Myologie*	**Short Oral Presentations 6** Terrace 2B **496P, 686P, 594P, 595P, 403P** *Moderator: Nigel Laing, Harry Perkins Institute of Medical Research*
19:00-20:00	**Industry Symposium 5** South Hall 1 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium	**Industry Symposium 6** South Hall 2 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium	

Thursday 10th October 2024

07:00-15:00	**Congress desk open**	
07:15-08:15	**NMD Board Meeting** Club B & C (separate registration required)	
08:15-09:15	**Industry Symposium 7** South Hall 1 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium	**Industry Symposium 8** South Hall 2 **Catering provided by the sponsor for this session**, available from 30 minutes prior to the start of the symposium
09:30-11:00	**Topic 2: NMD Around the World** Congress Hall *Moderators: Hernan Gonorazky, The Hospital for Sick Children, Canada & Rasha El Sherif, Newgiza University, Egypt* Panellists: **06INV** What about management of neuromuscular diseases in Latin America? *Soledad Monges, Hospital de Pediatría J.P. Garrahan, Argentina* **07INV** Neuromuscular disorders in India: New World meets the Old *Venugopalan Y Vishnu, All India Institute of Medical Sciences, India* **05INV** Advancing neuromuscular disorders in Senegal, West Africa, through collaborative networks and available resources *Pedro M Rodriguez Cruz, Centro Nacional de Análisis Genómico (CNAG), Spain* **08INV** An African perspective on muscle diseases *Jeannine Heckmann, University of Cape Town, South Africa*	
11:00-11:30	**Morning refreshments, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	
11:00-11:30	**Myology Café - Meet the WMS Myology Developments Across the World Committee** Congress Hall Foyer	
11:30-13:30	**Topic 2: NMD Around the World** Congress Hall *Moderators: Sharon Aharoni, Schneider Childrens' Medical Center, Israel & Jorge Bevilacqua, Hospital Clínico Universidad De Chile & Clinica Dávila, Chile* **11:30-11:45 070** Rare neuromuscular disease specific mega clinics: a proposal to bridge the gap in resource limited settings *Ramya Ramesh Babu, Bangalore Baptist Hospital, Bengaluru, India* **11:45-12:00 080** To manually or mechanically cough: that is the question. Cough augmentation in children with neuromuscular disorders: a feasibility study *Anri Human, Sefako Makgatho Health Sciences University, South Africa* **12:00-12:15 090** The clinical and molecular landscape of genetic neuromuscular disorders in Senegal, West Africa *Pedro M Rodriguez Cruz, Centro Nacional de Análisis Genómico (CNAG), Spain*	

	12:15-12:30 100 The neonatal screening of SMA in Ukraine: the 18 months of experience *Nataliia Olkhovych, Expert Centre of Neonatal Screening, Ukraine* **12:30-12:45 110** Genetic profile of Brazilian patients with LAMA2-related dystrophies *Clara Camelo, University Of Sao Paulo, Brazil* **12:45-13:00 120** Clinical, paraclinical features and classification of myositis in Vietnam *Si Le, University Medical Centre of Hochiminh, Vietnam* **13:00-13:15 130** Genomic neuromuscular disorders in Turkey originate from a vast background *Haluk Topaloglu, Yeditepe University, Turkey* **13:15-13:30 140** Inherited Neuromuscular disorders in India: Outcomes of 1000 probands in the ICGNMD study at AIIMS New Delhi *Venugopalan Y Vishnu, All India Institute of Medical Sciences, India*	
13:30-14:45	**Lunch, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	
13:30-14:45	**Career Development Session - Engagement with industry** Zoom and Panorama Rooms (separate registration required and lunch is provided)	
15:00-17:30	**Poster viewing / Group activity** (separate registration required) Please refer to your joining instructions for further details of group activities and schedules.	
17:30-20:00	**Group activity networking reception** Kaiserstein Palace (separate registration required)	

Friday 11th October 2024

06:45-17:15	**Congress desk open**	
07:00	**Light refreshments available**	
07:30-08:30	**Interesting Case Discussions** South Hall 2 *Moderators: A. Reghan Foley, National Institutes of Health, USA & Hans-Hilmar Goebel, Charité – Universitätsmedizin Berlin, Germany* **Case 1:** HIV-associated inclusion body myositis in a young woman *Jeannine Heckmann, University of Cape Town, South Africa* **Case 2:** A muscle disorder presenting with Resistant Hypercalcaemia *Ashirwad Merve, University College London Hospitals, UK* **Case 3**: TUBA4A associated congenital myopathy with centronuclear findings and protein aggregates *Cristiane Moreno, Universidade de São Paulo, Brazil* **Case 4**: Proximal and facial weakness: Beyond FSHD *Carmen Paradas Lopez, Virgen del Rocío Hospital, Spain* **Case 5**: ADSSL1 -associated myopathy presented with EDMD like phenotype *Hacer Durmus, Istanbul Faculty of Medicine, Turkey*	
08:30-08:45	**Comfort break**	
08:45-10:15	**Topic 3 - Session 1 - RNA in NMD: clinical insights, patho-mechanisms and treatments** Congress Hall *Moderators: Liubov Gushchina, The Abigail Wexner Research Institute, Nationwide Children's Hospital, USA & Emma Rybalka, Victoria University, Australia*	

	08:45-09:15 09INV An overview of the RNA pathomechanisms in neuromuscular diseases *Maurice Swanson, University of Florida College of Medicine, United States of America* **09:15-09:45 10INV** Clinical and diagnostic utility of RNA/transcriptome: An overview of role of RNA in disease gene discovery and diagnostics *Grace Yoon, the Hospital for Sick Children, Toronto, Canada* **09:45-10:00 15O** Spatial transcriptomics analysis of Becker and Duchenne skeletal muscle to decipher histopathological alterations in dystrophinopathies *Laura Heezen, Leiden University Medical Centre, Netherlands* **10:00-10:15 16O** Lost in translation: pathogenic translation of GGC repeats in novel and toxic proteins in Oculopharyngodistal myopathy (OPDM) *Manon Boivin, Institute of Genetics and Molecular and Cellular Biology, France*	
10:15-10:45	**Morning refreshments, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	
10:15-10:45	**Myology Café - Meet the WMS Sustainability Committee** Congress Hall Foyer	
10:45-12:15	**Topic 3 - Session 2 - RNA in NMD: clinical insights, patho-mechanisms and treatments** Congress Hall *Moderators: Gisèle Bonne, Institut de Myologie, France & Silvère Van Der Maarel, Leiden University Medical Center, The Netherlands* **10:45-11:15 11INV** RNA as biomarkers in neuromuscular diseases, including monitoring progression, in clinical trial design, as outcomes *Pietro Spitali, Leiden University Medical Center, The Netherlands* **11:15-11:45 12INV** RNA therapeutics in neuromuscular diseases, including new developments, and challenges *Virginia Arechavala-Gomeza, Biobizkaia Health Research Institute, Spain* **11:45-12:00 17O** Schwann cell transduction and PMP22 target engagement in non-human primates supports translation of RNAi-based gene therapy for CMT1A *Lindsay Wallace, Nationwide Children's Hospital, USA* **12:00-12:15 18O** Breaking ground in CMT1B treatment: AAV9-mediated dual RNAi and gene replacement therapy targeting schwann cells improves myelination and peripheral nerve function in mice *Mary McCulloch, Nationwide Children's Hospital, USA*	
12:30-13:30	**WMS General Assembly / Poster viewing for non-members** South Hall 2	
12:30-14:00	**Lunch, exhibition and posters** Congress Hall Foyer, Forum Hall and Forum Foyer	

13:45-14:15	**Sponsor Meeting** South Boardroom 1		
14:15-15:15	**Poster Session 3** Forum Hall (refreshments provided) **55P-75P, 76VP-78VP: Distal myopathies, MFM** **182P-203P: SMA outcome measures and registries** **247P-265P, 266VP: Neuromuscular disorders around the world** **280P-315P, 316VP-317VP: Dystrophinopathies (animals models, biomarkers, brain, genetics)** **438P-440P, 442P-471P: Myotonic dystrophy** **598P-618P: Registries, networks and care of NMD**		
15:15-15:45	**Short Oral Presentations 7** North Hall **69P, 70P, 74P, 71P, 72P** *Moderator: Duygu Selcen, The Mayo Clinic*	**Short Oral Presentations 8** Terrace 2A **469P, 470P, 471P, 468P, 203P** *Moderator: Nicholas Johnson, Virginia Commonwealth University*	**Short Oral Presentations 9** Terrace 2B **315P, 314P, 312P, 265P, 262P** *Moderator: Anna Sarkozy, Great Ormond Street Hospital*
15:45-16:45	**Poster Session 4** Forum Hall (refreshments provided) **98P-109P, 110VP-112VP: ALS/neuropathy** **148P-181P: SMA Therapies** **234P-246P, 497P-513P, 514VP-515VP: Myasthenia Gravis, NMJ1-2, Periodic paralysis** **318P-358P, 343VP, 359VP-360VP: DMD - clinical care and cases reports, BMD** **516P-536P, 537VP: Metabolic and mitochondrial myopathies** **656P-676P: Pompe disease** **705LBP-710LBP, 711VP, 712LBP-717LBP, 719LBP-733LBP, 735LBVP, 736LP-738LBP: Late Breaking**		
16:45-17:15	**Short Oral Presentations 10** North Hall **181P, 177P, 178P, 180P, 179P** *Moderator: Laurent Servais, University of Oxford, UK*	**Short Oral Presentations 11** Terrace 2A **656P, 675P, 673P, 674P, 358P** *Moderator: Pascal Laforêt, Hôpital Raymond Poincaré*	**Short Oral Presentations 12** Terrace 2B **536P, 109P, 513P, 512P, 241P** *Moderator: Heike Kölbel, Universitätsmedizin Essen,*
19:15-01:00	**Networking Dinner** Municipal House (separate registration required)		

Saturday 12th October 2024

07:15-14:00	**Congress desk open**
07:15-14:00	**Myology Café open** Congress Hall Foyer
07:45-08:45	**Clinical Trial Updates** Congress Hall *Moderators: Francesco Muntoni, University College London, Great Ormond Street Hospital, UK & Tina Duong, Stanford University, USA* **07:45-08:00 190** Muscle MRI outcomes in patients with Duchenne Muscular Dystrophy treated with delandistrogene moxeparvovec: Findings from EMBARK Part 1 *Krista Vandenborne, University of Florida, USA* **08:00-08:15 200** Preliminary results from a Phase 1-2 gene therapy study of ATA-100, AAV9 vector encoding FKRP, in patients with Limb Girdle Muscular Dystrophy R9 *Sophie Olivier, Atamyo Therapeutics, France* **08:15-08:30 220** MExiletine versus lamotrigine in Non-Dystrophic myotonias – a randomised, double-blinded, cross-over trial *Vinojini Vivekanandam, National Hospital for Neurology and Neurosurgery, UK* **08:30-08:45 210** Rainbowfish: 2-year efficacy and safety data of risdiplam in infants with presymptomatic SMA *Laurent Servais, University of Oxford, UK*
08:45-09:00	**Comfort Break**

09:00-09:30	**Victor Dubowitz Lecture** Congress Hall *Moderators: Volker Straub, Newcastle University, UK & Jana Haberlová,University Hospital Motol, Czechia* **INV13** AAV delivery of mini-and full-length dystrophins *Jeffrey S. Chamberlain, University of Washington School of Medicine, United States of America*
09:30-11:00	**Poster Highlights** *Moderators: Edoardo Malfatti, Université Paris Est, France & Tamara Dangouloff, University Of Liege, Belgium* **09:30- 09:45 23O** Spinal Muscular Atrophy is also a disorder of spermatogenesis *Armelle Magot, Centre de Référence des Maladies Neuromusculaires AOC, CHU de Nantes, Filnemus, Euro-NMD, France* **09:45-10:00 24O** Temporal requirement of dystroglycan glycosylation during brain development and rescue of cortical dysplasia via gene delivery in the fetal stage *Atsushi Sudo, The University of Tokyo, Japan* **10:00-10:15 25O** Development of a CRISPR/CasX 4q telomeric region ablation strategy for FSHD1 using an isogenic hiPSC line and a FSHD1 fibroblast cell line *Cheryane Lama, Université Paris Est Créteil, INSERM, IMRB - Hôpital Henri-Mondor, France* **10:15-10:30 26O** Understanding the clinical heterogeneity in myotonic dystrophy type 1: identifying clinical phenotypes using unsupervised clustering *Leandre la Fontaine, Maastricht University Medical Centre, The Netherlands* **10:30-10:45 27O** Mass spectrometry as a technique for robust quantification of titin and other large muscle disease-associated proteins *Andrei Smolnikov, University Of New South Wales, Australia* **10:45-11:00 28O** Minimal detectable change of the Revised Hammersmith Scale in patients with Spinal Muscular Atrophy *Emer O'Reilly, Great Ormond Street Hospital for Children NHS Foundation Trust, UK*
11:00-11:30	**Morning refreshments, exhibition and posters**
11:30-13:30	**Late Breaking News** Congress Hall *Moderators: Ana Topf, John Walton Muscular Dystrophy Research Centre, UK & Alan H. Beggs, Boston Children's Hospital, Harvard Medical School, USA* **11:30 - 11:45 01LBO** Biallelic variants affecting the DST-b isoform cause a severe congenital myopathy presenting with arthrogryposis, muscular hypotonia and dilated cardiomyopathy *M Jacob, Institute of Human Genetics, Technical University of Munich, Germany* **11:45 - 12:00 02LBO** Expanding the genetic and phenotypic landscape of replication factor C complex-related disorders: RFC4 deficiency is linked to a multisystemic disorder *Y Saito, Department of Neuromuscular Research, National Institute of Neuroscience, National Center of Neurology and Psychiatry, Japan* **12:00 - 12:15 03LBO** Decoy gene therapy for myotonic dystrophy *D Furling, Centre De Recherche En Myologie / Institut De Myologie, France* **12:15 - 12:30 04LBO** Interim Clinical Data Summary: A Phase 1b/2a Open-label, Dose Escalation Study to Evaluate the Safety and Clinical Activity of Intramuscular Doses of an AAV9-based gene therapy (BB-301) Administered to Subjects with Oculopharyngeal Muscular Dystrophy (OPMD) with Dysphagia *M Amin, Nyu Grossman School of Medicine, USA* **12:30 - 12:45 05LBO** A phase 1 study of antisense oligonucleotide NS-035 in patients with Fukuyama Congenital Muscular Dystrophy *G Fujino, The University of Tokyo Hospital, Japan*

	12:45 - 13:00 06LBO CIFFREO, a phase 3, randomized, double-blind, placebo-controlled study of fordadistrogene movaparvovec (FM) in ambulatory participants with Duchenne Muscular Dystrophy (DMD) *F Muntoni, University College London, Great Ormond Street Institute of Child Health, UK* **Prize Giving Ceremony** *Moderator: Marco Savarese, University of Helsinki, Finland* **Introduction to the WMS 2025 Congress, Vienna, Austria** **Handover of the WMS flag and close of Congress** *Moderator: Volker Straub*
13:30-14:30	**Homeward lunch**