



# Neuromuscular Study Group

24<sup>TH</sup> ANNUAL SCIENTIFIC MEETING

ORLANDO, FLORIDA | SEPTEMBER 22-24, 2023

## DAY 1: FRIDAY, SEPTEMBER 22

6:30-8 a.m.	Buffet Breakfast and Check-In   <i>Caribbean VI and VII</i>	12:05-12:15 p.m.	<b>Mycophenolate is Better Tolerated Than Azathioprine in Myasthenia Gravis</b> Katy Dodd, MBChB, MRCP   <i>University of Manchester</i>
8-8:20 a.m.	<b>Welcome and State of the Neuromuscular Study Group</b> Dr. Richard Barohn and Prof Michael Hanna <i>Caribbean III and IV</i>	12:17-12:27 p.m.	<b>A UK Experience of Symptomatic Treatment of Myotonia with Lamotrigine</b> Iwona Skorupinska, MSc, BSc <i>University College London</i>
<b>SESSION I: MOTOR NEURON AND NEUROMUSCULAR JUNCTION</b> Heidi Fuller, M.D., Moderator   <i>Caribbean III and IV</i>		12:30-1:30 p.m.	Lunch   <i>Caribbean VI and VII</i>  <b>Neuromuscular Study Group Executive Committee Meeting Breakout Lunch</b>   <i>Governor's Board Room</i>
8:20-8:40 a.m.	<b>Unravelling Inflammatory Neuropathies</b> Simon Rinaldi, MBChB, Ph.D.   <i>University of Oxford</i>	<b>SESSION III: PLATFORM PRESENTATIONS</b> Kris Kelly, DPT, MS, EdM, Moderator   <i>Caribbean III and IV</i>	
8:45-9:05 a.m.	<b>Towards Clinical Trial Readiness for Charcot Marie Tooth Neuropathies</b> David Hermann, MBBCh   <i>University of Oxford</i>	1:30-1:45 p.m.	<b>Improving Diagnostic Rates for Mitochondrial Diseases Using Enhanced WGS Analysis and RNA-seq</b> William Macken, M.D., Ph.D. <i>University College London</i>
9:10-9:30 a.m.	<b>Respiratory Updates in CANVAS</b> Riccardo Zuccarino, M.D.   <i>NEMO Milan</i>	1:50-2:05 p.m.	<b>MEND: MExiletine versus lamotrigine in Non-Dystrophic Myotonia</b> Vinojini Vivekanandam, MBBS(Hons) <i>University College London, Queen Square</i>
9:35-9:55 a.m.	<b>Recent Learnings from Non-Coding Genome Investigations</b> Stephan Zuchner, M.D., Ph.D.   <i>University of Miami</i>	2:10-2:25 p.m.	<b>Scoliosis Progression in Type II SMA at the Time of Treatment: A Comparative Study with Untreated Patients</b> Giorgia Coratti, Ph.D. <i>Catholic University of Sacred Heart</i>
10-10:15 a.m.	Refreshment/Exhibitor Break	2:30-2:45 p.m.	<b>Remote Monitoring and Management of Myasthenia Gravis (REMOTE-MG): A Pilot Feasibility Study</b> Michael Hehir, M.D.   <i>University of Vermont</i>
10:15-10:35 a.m.	<b>Expanding Therapeutic Options in Myasthenia Gravis</b> James Howard, M.D. <i>The University of North Carolina at Chapel Hill</i>	2:50-3 p.m.	Refreshment/Exhibitor Break
10:40-11 a.m.	<b>Updates on ALS</b> Jon Katz, M.D. <i>Sutter Health, California Pacific Medical Center, The Forbes Norris MDA/ALS Research and Treatment Center</i>	<b>SESSION IV: YOUNG INVESTIGATOR/EVALUATOR/COORDINATOR</b> <b>*ALL NEW*</b> Session Moderators: Dr. W. David Arnold, Dr. Karen Suetterlin, Dr. Katherine Dodd, Dr. Heidi Fuller, Prof Valeria Sansone, Marie Wencel, CCRP <i>Caribbean III and IV</i>	
<b>SESSION II: FLASH PRESENTATIONS</b> Dr. Michael Pulley, Moderator   <i>Caribbean III and IV</i>		3-5 p.m.	<b>How to Give an Effective Elevator Pitch</b> <b>Failing Well, Overcoming Rejections, Criticisms and Changing Direction</b> <b>Networking</b>
11:05-11:15 a.m.	<b>Investing to Save: Evaluation of Unplanned Hospital Admissions of Neuromuscular Patients in Greater Manchester, UK</b> Marwah Almadhi, BSc Biomedical Sciences, MBChB Medicine (current)   <i>University of Manchester</i>	<b>POSTER SESSION</b> <i>Caribbean V</i>	
11:17-11:27 a.m.	<b>Measuring Adverse Event Burden in Myasthenia Gravis: Retrospective Validation of the Adverse Event Unit (AEU) with MGTX Trial Data</b> Michael Hehir, M.D.   <i>University of Vermont</i>	6-8 p.m.	Poster Walk Through and Reception  Reception
11:29-11:39 a.m.	<b>A Systematic Review and Meta-Analysis of the Placebo Effect in Inclusion Body Myositis</b> Elie Naddaf, M.D.   <i>Mayo Clinic</i>	8-9 p.m.	Dinner   <i>Caribbean VI and VII</i>
11:41-11:51 a.m.	<b>The Inclusion Body Myositis Health-Index (IBM-HI): Development of a Novel, Disease Specific Patient-Reported Outcome Measure for IBM in Clinical Trials</b> Shaweta Khosa, M.D. <i>University of Rochester, Center of Health and Technology Outcomes Division (CHeT)</i>	9-11:30 p.m.	Reception   <i>Atrium East, Main Hotel Lobby, Lower Level</i>
11:53 a.m.-12:03 p.m.	<b>Relationships of Lower Leg Fat Fraction Among Antagonistic and Synergistic Muscles and a Potential Fat Fraction Threshold for Functional Performance in Myotonic Dystrophy Type 1</b> Zhihao He, MS   <i>University of Florida</i>		



## DAY 2: SATURDAY, SEPTEMBER 23

7-8 a.m.	<b>Buffet Breakfast</b>   <i>Caribbean VI and VII</i>
8-8:15 a.m.	<b>Opening</b> Dr. Richard Barohn and Prof Michael Hanna <i>Caribbean III and IV</i>
<b>SESSION V: BIOMARKERS</b> Donovan Lott, PT, Ph.D., CSCS, Moderator   <i>Caribbean III and IV</i>	
8:15-8:35 a.m.	<b>Shear Wave Elastography in Neuromuscular Disease</b> Lisa Hobson-Webb, M.D.   <i>Duke University</i>
8:40-9 a.m.	<b>Unveiling the Hidden Regulators: Circulating MicroRNAs as Biomarkers in Myasthenia Gravis</b> Professor Anna Rostedt Punga, M.D., Ph.D. <i>Uppsala University</i>
9:05-9:25 a.m.	<b>Neurofilament as a biomarker in ALS clinical trials</b> Robert Bowser, Ph.D. <i>Barrow Neurological Institute</i>
9:30-9:50 a.m.	<b>Alternative Splicing DM</b> Eric Wang, Ph.D.   <i>University of Florida</i>
9:55-10:15 a.m.	<b>MRI as a Biomarker in the Muscular Dystrophies</b> Glenn Walter, Ph.D.   <i>University of Florida</i>
10:15-10:30 a.m.	<b>Refreshments/Exhibitor Break</b>
<b>SESSION VI: AGING</b> W. David Arnold, M.D., Moderator   <i>Caribbean III and IV</i>	
10:30-10:50am	<b>Proteomic Analysis of Differentially Vulnerable Synaptic Populations to Identify Regulators of Stability</b> Thomas Wishart, BSc., M.B.A., Ph.D. <i>University of Edinburgh</i>
10:55-11:15am	<b>Motor Unit Magnetic Resonance Imaging (MUMRI) in Ageing Skeletal Muscle</b> Matthew Birkbeck, Ph.D. <i>Newcastle University/Newcastle Upon Tyne Hospitals NHS Foundation Trust</i>
11:20-11:40am	<b>Clinical Aspects of Sarcopenia</b> Roger Fielding, Ph.D.   <i>Tufts University</i>
11:45 a.m.-1 p.m.	<b>Lunch</b>   <i>Caribbean VI and VII</i>
<b>SESSION VII: EXERCISE</b> Dr. Miguel Chuquilin, Moderator   <i>Caribbean III and IV</i>	
1-1:20 p.m.	<b>Effect of exercise on functional outcomes and disease pathophysiology in DM1</b> Elisa Duchesne, PHT, Ph.D. <i>Université du Québec à Chicoutimi</i>
1:25-1:45 p.m.	<b>Exercise in SMA: More Than Just an Intervention</b> Jackie Montes, PT, EdD, NCS <i>Columbia University Irving Medical Center</i>
1:50-2:10 p.m.	<b>Exercise as medicine for DMD</b> Tanja Taivassalo, Ph.D.   <i>University of Florida</i>
2:15-2:35 p.m.	<b>Exercise, Rehabilitation and Physical Activity in Charcot-Marie-Tooth Disease</b> Gita Ramdharry, Ph.D. <i>University College London</i>
2:40-3:10 p.m.	<b>Refreshments/Exhibitor Break</b>

<b>SPONSOR PRESENTATIONS</b> W. David Arnold, M.D., Moderator   <i>Caribbean III and IV</i>	
3:10-3:30pm	<b>NMJ damage in MG: Point of no return or potential for repair?</b> James F. Howard, Jr., M.D., FAAN <i>The University of North Carolina at Chapel Hill</i>
3:35p-3:55pm	<b>Innovation in the Development of Treatments for Neuromuscular Diseases</b> Jeffrey Guptill, M.D., MA, MHS, FAAN <i>Efgartigimod Neuromuscular Franchise Lead Clinical Development, Argenx</i>
4:00-4:20pm	<b>Advancing Precision Genetic Medicine Through Innovative Technologies for Neuromuscular Diseases</b> Teji Singh, M.D.   <i>Sarepta Therapeutics</i>
4:25-4:45pm	<b>Addressing unmet needs in FSHD: Data from the Iosmapimod Phase 2 trial</b> Olga Mitelman, M.D. <i>Head of Medical Affairs, Fulcrum Therapeutics</i>
4:50-5:10pm	<b>Lambert-Eaton Myasthenic Syndrome is Underrecognized in Small Cell Lung Cancer: An Analysis of Real-World Data</b> Nicholas Streicher, M.D., MPH <i>MedStar Georgetown University Hospital</i>
5:15-5:35pm	<b>At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development, and manufacture of health care products, including innovative medicines and vaccines</b>
<b>ROBERT C. GRIGGS ANNUAL NMSG KEYNOTE SPEAKER</b> <i>Caribbean III and IV</i>	
7:30-8:30 p.m.	<b>The Circle of Translation</b> Professor Mary M. Reilly <i>UCL Queen Square Institute of Neurology</i>
<b>8:30-9:30 p.m. Dinner</b>   <i>Caribbean VI and VII</i>	
<b>9:30-11 p.m. Evening Reception</b>   <i>Boca Patio</i>	



## DAY 3: SUNDAY, SEPTEMBER 24

7-8 a.m.	<b>Buffet Breakfast</b>   <i>Caribbean VI and VII</i>
8-8:10 a.m.	<b>Opening</b> Dr. Richard Barohn and Prof Michael Hanna   <i>Caribbean III and IV</i>
<b>SESSION VIII: NMSG YOUNG INVESTIGATORS PROJECTS</b> Dr. Karen Suetterlin, Moderator   <i>Caribbean III and IV</i>	
8:10-8:25 a.m.	<b>Neuromuscular Ultrasound as a Biomarker to Improve Clinical Trial Readiness in Charcot Marie Tooth Neuropathies</b> Tyler Rehbein, M.D., 2022 NMSG Fellow   <i>University of Rochester</i>
8:30-8:45 a.m.	<b>Development of Novel Imaging Biomarkers for use in Pediatric Facioscapulohumeral Muscular Dystrophy</b> Natalie Katz, M.D., 2023 NMSG Fellow   <i>Duke University</i>
8:50-9:05 a.m.	<b>2022 Shark Tank Award Update: Quantifying Idiopathic Inflammatory Myopathy-Associated Cancer Risk via Comprehensive Phenotyping of a Large UK-Wide Cohort</b> Alex Oldroyd, MBChB, Ph.D., MSc, MRCP   <i>University of Manchester</i>
9:10-9:25 a.m.	<b>2022 Shark Tank Award Update: Predictive Models in Spinal Muscular Atrophy Treated Patients Using Machine Learning</b> Giorgia Coratti, Ph.D.   <i>Pediatric Neurology and Centro Clinico Nemo Rome</i>
<b>SHARK TANK SESSION</b> Aziz Shaibani, M.D., FACP, FAAN, FANA, Moderator Shark Panel: Mazen Dimachkie, M.D., Amanda Guidon, M.D., MPH, Laurie Gutmann, M.D. <i>Caribbean III and IV</i>	
9:30-10:40 a.m.	<b>Perceived Fatigability Tracker: Improving Assessment to Enhance Spinal Muscular Atrophy (SMA) Patient Outcomes</b> Rafael Rodriguez-Torres DPT   <i>Columbia University Irving Medical Center</i>
	<b>Are patients with IIM at a greater risk of developing PCC than healthy controls at six months after COVID infection</b> Tulika Chatterjee, M.D.   <i>University of Illinois College of Medicine at Peoria, IL</i>
	<b>LITEBulb SMA - Lingual Intervention of Tongue Exercises for BULBar SMA</b> Carla Zingariello, DO   <i>University of Florida College of Medicine</i>
10:40-10:55 a.m.	<b>Refreshments/Exhibitor Break</b>
<b>SESSION IX: EMERGING PHENOTYPES</b> Dr. Katy Dodd, Moderator   <i>Caribbean III and IV</i>	
10:55-11:20 a.m.	<b>Neuromuscular Implications of Placebo Research</b> Aziz Shaibani, M.D., FACP, FAAN, FANA   <i>Nerve and Muscle Center of Texas</i>
11:25-11:45 a.m.	<b>Treating Adults with SMA in the UK</b> James Lilleker, MBChB, Ph.D.   <i>University of Manchester</i>
11:50 a.m.-12:10 p.m.	<b>Neuromuscular Complications of Cancer Immunotherapy</b> Amanda Guidon, M.D., MPH   <i>Mass General Hospital, Harvard University</i>
12:15-12:35 p.m.	<b>Pediatric FSHD, overview and gaps in knowledge</b> Katherine Mathews, M.D.   <i>University of Iowa Carver College of Medicine</i>
12:35-1:45 p.m.	<b>Closing</b>   <i>Caribbean III and IV</i>
	<b>Lunch</b>   <i>Caribbean VI and VII</i>

