24th Annual Neuromuscular Study Group Scientific Meeting

Thursday, September 21, 2023
We will have early check in Thursday evening and Friday morning

Day 1: Friday, September 22, 2023

6:30am-8:00am Buffet Breakfast and Check-In

8:00am-8:20am Welcome and State of the Neuromuscular Study Group
Dr. Richard Barohn and Prof Michael Hanna

Session I: Motor Neuron and Neuromuscular junction
Moderator - Heidi Fuller, PhD

8:20am-8:40am Simon Rinaldi, MBChB, PhD University of Oxford
Inflammatory neuropathy

8:45am-9:05am David Hermann, MBBCh University of Rochester Medical Center
Towards Clinical Trial Readiness for Charcot Marie Tooth Neuropathies

9:10am-9:30am Riccardo Zuccarino, MD NeMO Centre, Milan
Resp involvement in neuropathy

9:35am-9:55am Stephan Zuchner, MD, PhD, FAAN University of Miami, Miller School of Medicine
hereditary neuropathies

10:00am-10:15am Refreshment/Exhibitor Break

10:15am-10:35am James Howard, MD University of North Carolina at Chapel Hill
new treatments in MG
10:40am-11:00am Jon Katz, MD
Updates on ALS

Session II – Flash Presentations Moderator - Dr Michael Pulley, MD, PhD

11:05am-11:15am Marwah Almadhi, BSc Biomedical Sciences, MBChB Medicine (current)
University of Manchester
Investing to Save: Evaluation of Unplanned Hospital Admissions of Neuromuscular Patients in Greater Manchester, UK.

11:17am-11:27am Michael Hehir, MD University of Vermont
Measuring Adverse Event Burden in Myasthenia Gravis: Retrospective Validation of the Adverse Event Unit (AEU) with MGTX Trial Data

11:29am-11:39am Elie Naddaf, MD Mayo Clinic
A systematic review and meta-analysis of the placebo

11:41am-11:51am Shaweta Khosa, MD University of Rochester, Center of Health and Technology Outcomes Division (CHeT)
The Inclusion Body Myositis Health-Index (IBM-HI): Development of a Novel, Disease Specific Patient-Reported Outcome Measure for IBM in Clinical Trials

11:53am-12:03pm Zhihao He, University of Florida
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

12:05pm-12:15pm Katy Dodd, MBChB, MRCP University of Manchester
Mycophenolate is better tolerated than azathioprine in myasthenia gravis

12:17pm-12:27pm Iwona Skorupinska, MSc, BSc University College London
A UK experience of symptomatic treatment of myotonia with Lamotrigine

12:30am-1:30pm Lunch

Muscle Study Group Executive Committee Meeting Breakout

Session III Platform Presentations – Moderator Kris Kelly, DPT, MS, Edm

1:30pm – 1:45pm William Macken, MD, PhD University College London
Improving diagnostic rates for mitochondrial diseases using enhanced WGS analysis and RNA-seq.

1:50pm-2:05pm  
**Vinojini Vivekanandam, MBBS(Hons)** University College London, Queen Square Institute of Neurology

MEND: MExiletine versus lamotrigine in Non-Dystrophic Myotonia

2:10pm-2:25pm  
**Giorgia Coratti, PhD** Catholic University of Sacred Heart

Scoliosis progression in type II SMA at the time of treatment: a comparative study with untreated patients

2:30pm-2:45pm  
**Michael Hehir, MD** University of Vermont

Remote Monitoring and Management of Myasthenia Gravis (REMOTE-MG): A Pilot Feasibility Study

2:50pm-3:00pm  
Refreshment/Exhibitor Break

Session IV

3:00pm-5:30pm  
Young Investigator Session/Evaluator Session Breakout; Coordinator Breakout

*ALL NEW*

Session Moderators – Dr W. David Arnold, Dr Karen Suetterlin, Dr Katherine Dodd, Dr Heidi Fuller, Prof Valeria Sansone, Marie Wencel

- How to give an effective elevator pitch
- Tips for successful Grant Writing
- Failing well, overcoming rejections, criticisms and changing direction

6:00pm-8:00pm  
Abstract poster walk through session

8:00pm-9:00pm  
Dinner

9:00pm-11:30pm  
Reception

Day 2: Saturday, September 23, 2023

7:00am-8:00am  
Buffet Breakfast
8:00am-8:15am  Dr Barohn and Prof Hanna Opening

Session V: Biomarkers (new) Moderator Donovan Lott

8:15am-8:35am  Lisa Hobson Webb, MD Duke University
Shear wave electrography/ultrafast ultrasound use for neuromuscular disorders

8:40am-9:00am  Professor Anna Rostedt Punga, MD, PhD Uppsala University, Sweden
Circulating microRNAs as biomarkers in Myasthenia Gravis

9:05am-9:25am  Robert Bowser, PhD Barrow Neurological Institute
Neurofilament as a biomarker for everything

9:30am-9:50am  Eric Wang, PhD University of Florida
Alternative splicing DM1

9:55am-10:15am  Glenn Walter, PhD University of Florida
MRI as a biomarker in MD

10:15am-10:30am  Refreshments/Exhibitor Break

Session VI: Aging Moderator – W. David Arnold

10:30am -10:50am  Thomas Wishart, BSc., M.B.A., PhD University of Edinburgh
proteomic analysis of differentially vulnerable synaptic populations to identify regulators of stability

10:55am - 11:15am  Dr Matthew Birkbeck, PhD Newcastle University/Newcastle Upon Tyne Hospitals NHS Foundation Trust
Changes in Motor Unit MRI with age /sarcopenia

11:20am-11:40am  Roger Fielding, PhD Tufts University
Clinical aspects of sarcopenia

11:45am-1:00pm  Lunch

Session VII: Exercise – Moderator Miguel Chuquilin, MD
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<tr>
<th>Time</th>
<th>Speaker and Affiliation</th>
<th>Title/Summary</th>
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<tbody>
<tr>
<td>1:00pm-1:20pm</td>
<td>Elisa Duchesne, PHT, PhD Université du Québec à Chicoutimi</td>
<td>Effect of exercise on functional outcomes and disease pathophysiology in DM1</td>
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<td>1:25pm-1:45pm</td>
<td>Jackie Montes, PT, EdD, NCS Columbia University Irving Medical</td>
<td>Exercise in SMA: More than just an intervention</td>
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<td>1:50pm-2:10pm</td>
<td>Tanja Taivassalo, PhD University of Florida</td>
<td>has multiple ongoing projects on exercise in DMD, impacts on functional</td>
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<td>outcomes and disease biomarkers</td>
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<td>2:15pm-2:35pm</td>
<td>Gita Ramdharry, PhD University College London</td>
<td>Exercise, rehabilitation and physical activity in Charcot-Marie-Tooth disease</td>
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<td>2:40pm-3:10pm</td>
<td>Refreshments/Exhibitor Break</td>
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<td>3:10pm-3:30pm</td>
<td>UCB Update</td>
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<td>3:35pm-3:55pm</td>
<td>Argenx Update</td>
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<td>4:00pm-4:20pm</td>
<td>Sarepta Therapeutics Update</td>
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<td>4:25pm-4:45pm</td>
<td>Fulcrum Therapeutics Update</td>
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<td>4:50pm-5:10pm</td>
<td>Catalyst Pharmaceuticals Update</td>
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<td>5:15pm-5:35pm</td>
<td>Pfizer Update</td>
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<td>7:30pm-8:30pm</td>
<td>Robert C. Griggs Annual NMSG Keynote Speaker</td>
<td>The Circle of Translation</td>
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<td>Professor Mary M Reilly</td>
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<td>UCL Queen Square Institute of Neurology</td>
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<td>8:30pm-9:30pm</td>
<td>Dinner</td>
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<td>9:30pm-11:30pm</td>
<td>Evening Reception</td>
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**Day 3: Sunday, September 24, 2023**

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<th>Time</th>
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<tr>
<td>7:00am-8:00am</td>
<td>Breakfast</td>
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<td>8:00am-8:10am</td>
<td>Dr Barohn and Prof Hanna opening</td>
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<td>Session IX – NMSG Young investigators projects</td>
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<td>Moderator - Karen Suetterlin, MBBS, MRCP, PhD</td>
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8:10am-8:25am 2022 NMSG Fellow Tyler Rehbein, MD University of Rochester

Neuromuscular ultrasound as a biomarker to improve clinical trial readiness in Charcot Marie Tooth Neuropathies

8:30am-8:45am 2023 NMSG Fellow Dr Natalie Katz, MD, PhD Duke University

Development of novel imaging biomarkers for use in pediatric facioscapulohumeral muscular dystrophy

8:50am-9:05am 2022 Shark Tank Award update Alex Oldroyd MBChB, PhD, MSc, MRCP University of Manchester

Quantifying Idiopathic Inflammatory Myopathy-Associated Cancer Risk via Comprehensive Phenotyping of a Large UK-Wide Cohort

9:10am-9:25am 2022 Shark Tank Award update Giorgia Coratti, PhD Pediatric Neurology and Centro Clinico Nemo Rome

Predictive models in spinal muscular atrophy treated patients using machine learning

9:30am-10:45am Shark Tank Session

Moderator: Aziz Shaibani, MD, FACP, FAAN, FANA
Nerve and Muscle Center of Texas

Shark Panel: Mazen Dimachkie, MD, Amanda Guidon, MD and Laurie Gutmann, MD

Presentations:

Determination of the unique genotypic/phenotypic characteristics of South East Asia FSHD1 patients in Singapore Dr Zhiyong Chen, UCL Queen Square Centre

Perceived Fatigability Tracker: Improving Assessment to Enhance Spinal Muscular Atrophy (SMA) Patient Outcomes Rafael Rodriguez-Torres DPT, Columbia University Irving Medical Center

Markers of respiratory progression in Duchenne Muscular Dystrophy – systemic inflammation and diaphragm thickness and motion (“DMD-InflUS”) Federica Trucco MD, PhD, University of Milan

LITEBulb SMA - Lingual Intervention of Tongue Exercises for BULBar SMA Carla Zingariello, DO University of Florida College of Medicine, Gainesville, FL
10:45am-11:00am  Refreshments/Exhibitor Break

Session X Emerging Phenotypes - Moderator Dr. Katy Dodd, MBChB MRCP

11:00am-11:20am  Aziz Shaibani, MD, FACP, FAAN, FANA  Nerve and Muscle Center of Texas
Neuromuscular implications of placebo research

11:25am-11:40pm  James Lilleker, MBChB, PhD  University of Manchester
Adult SMA

11:45am-12:05pm  Amanda Guidon, MD, MPH  Mass General Hospital/Harvard
checkpoint inhibitors

12:10pm-12:30pm  Katherine Mathews, MD  University of Iowa Carver College of Medicine
Peds FSHD

12:35pm-12:55PM  Valeria Sansone, MD, PhD  University of Milan
CNS and cognitive in SMA across the lifespan

1:00pm-2:00pm  Lunch

Closing