

# 25<sup>th</sup> Annual Neuromuscular Study Group Scientific Meeting

**Thursday, September 19, 2024**

Dinner and check in at the Winter Palace 6pm – 9pm

## **Day 1: Friday, September 20, 2024**

- 7:00am-8:00am **Buffet Breakfast (Winter Palace)**
- 7:00am-8:00am **Check in Duke Mansion**
- 8:00am-8:20am **Welcome and State of the Neuromuscular Study Group**  
**Dr. Richard Barohn and Prof Michael Hanna**
- Session I: Genetics***
- Moderator - TBD***
- 8:20am-8:40am *Gene Therapy for DMD and SMA: milestones, lessons learned and current challenges – Emma Ciafaloni, MD, University of Rochester*
- 8:45am-9:05am *Overview of potential genetic treatments for FSHD – Scott Harper, PhD, Nationwide Children’s Hospital*
- 9:10am-9:30am *Genetic Therapeutics in neuropathies/CMT - Mario Saporta, MD, PhD – University of Miami*
- 9:35am-9:55am *Genetic Therapeutics in Myotonic Dystrophy – Charles Thornton, MD, University of Rochester*
- 10:00am-10:20am *Neuromuscular genetic therapies COL6, HSN1 -Dr. Haiyan Zhou, University College London*
- 10:25am-10:40am **Refreshment/Exhibitor Break**
- Session II: Flash Presentations***
- Moderator - TBD***
- 10:40am-10:50am *Refractory myasthenia gravis characterised by widespread innate and adaptive immune system changes, Dr. Katy Dodd*

- 10:52am-11:02am *Remote monitoring to improve adherence to physical exercise: pilot experience at the NeMO site, Michela Nani*
- 11:05am-11:15am *Trial of Oxaloacetate in ALS, TOALS, Katie Lillig*
- 11:17am-11:27am *Outcome Measures to Quantify Longitudinal Changes in Motor Function in FSHD, Lawrence Hayward, MD*
- 11:30am-11:40am *Treatment effects on ambulation loss in Spinal Muscular Atrophy Type III: insights from the Italian ISMAC registry, Dr. Giorgia Coratti*
- 11:42am-11:52am *The Myasthenia Gravis Patient Registry: Characteristics, Insights, and Learnings After a Decade (2013-23), Kelly Graham Gwathmey, MD*
- 12:00-1:00pm **Lunch buffet at Winter Palace**
- 12:00pm – 1:00pm **NMSG Executive Committee Meeting Breakout lunch - Music Room**

**Session III: Platform Presentations**

**Moderator- TBD**

- 1:00pm –1:15pm *Co-designing a Strategy to Engage People with Neuromuscular Diseases from Racially Minoritized Backgrounds in Research, Gita Ramdharry*
- 1:20pm-1:35pm *A Study of the Common Factors that Influence Fatigue in Myasthenia Gravis, Kelly Graham Gwathmey*
- 1:40pm-1:55pm *Combined personalized home-based aerobic exercise and coaching to improve physical fitness in neuromuscular diseases - a multicenter, single-blind, randomized controlled trial, Eric Voorn*
- 2:00pm-2:15pm *An analysis of Mortality Rates and Causes of Death in an Oxford Cohort of Adult Myasthenia Gravis Patients, Pietro Zara*

**2:15pm-2:30pm Refreshment/Exhibitor Break**

**2:30pm-4:30pm Session IV:  
Young Investigator/Evaluator/Coordinator Session**

**Moderators - Dr Michael Hehir, Dr Vino Vivekanandam, and Prof Valeria Sansone**

**Dr Lauren Reoma-** NINDS Associate Director Clinical Trials and Leader of Experimental Therapeutics Program

NMSG Resources - NMSG representative

Fellowships

MDUK

**5:30pm-7:30pm** Abstract poster session (Carriage House)

**7:30pm-9:00pm** Buffet Dinner (Winter Palace)

**9:00pm-11:00pm** Reception (West Terrace)

## **Day 2: Saturday, September 21, 2024**

**7:00am-8:00am** Buffet Breakfast (Winter Palace) Meet the experts breakfast

**8:00am-8:15am** Dr Barohn and Prof Hanna Opening (Mary Duke Ballroom)

### ***Session V: NMS and the body***

***Moderator - TBD***

**8:20am-8:40am** Overview – Julie Parsons, MD, Children’s Hospital Colorado

**8:45am-9:05am** Cognitive SMA – Valeria Sansone, MD, PhD, NEMO Milan

**9:10am-9:30am** Cognitive involvement/deficits in myotonic dystrophy in children and adults– Nick Johnson, MD, VCU

**9:35am-9:55am** *Cardiomyopathies in the muscular dystrophies* – Carol Wittlieb-Weber, MD, Children’s Hospital of Philadelphia

**10:00am-10:15am** Refreshments/Exhibitor Break

### ***Session VI: Neuropathy***

***Moderator - TBD***

**10:20am -10:40am** Kennedy's Disease - new clinical developments, biomarkers, current/upcoming trials – Prof Pietro Fratta, UCL Institute of Neurology

**10:45am - 11:05am** *advances in the work-up and management of immune mediated peripheral neuropathies*, Karissa Gable, MD, Duke University School of Medicine

**11:10am-11:30am**      *Peripheral nerve imaging in CMT - Reza Seyedsadjadi, MD, Mass General*

**11:30pm-1:00pm**      **Lunch (Winter Palace)**

***NMSG 2025 planning committee meeting breakout lunch - Music Room***

**Session VII: Clinical Design**

***Moderator (s) – Dr Michael Hehir and Dr Vino Vivekanandam***

*Highlight trials using innovative trial design strategies. Focus on the trials/research with discussion of the novelty of the way they are being conducted.*

*1:00pm – 1:20pm    Life of Clinical Trials – Gordon Smith, MD*

*1:25 – 1:40pm    n-of 1 trials – Mike McDermott, PhD*

*1:45 -1:50 – Greener Trials– Dr. Vino Vivekanandam*

*1:50-1:55 – trial delivery and logistics – Matt Parton, MD*

*1:55-2:00 – recruiting/engaging pediatric participants – Kathy Mathews, MD*

*2:00-2:05 practical tips for platform trials – Merit Cudkowicz, MD*

*2:05 – 2:30 - panel Q&A*

**2:30pm-3:00pm**      **Refreshments/Exhibitor Break**

**Session VIII: Industry Presentations**

***Moderator – Michael Hehir, MD***

**2:50pm-3:10pm**      \*\*

**3:15pm-3:35pm**      \*\*

**3:40pm-4:00pm**      \*\*

**4:05pm-4:25pm**      \*\*

**4:30pm-4:50pm**      \*\*

**4:55pm-5:20pm**      \*\*

**5:25pm-5:45pm**      \*\*

**7:00pm – 8:15pm**      *NMSG Research Presentation*

**Development of Novel Imaging Biomarkers for use in Pediatric  
Faciocapulo humeral Muscular Dystrophy**  
NMSG Fellow, Natalie Katz, MD, Duke University

**Robert C. Griggs Annual NMSG Keynote Speaker**  
Merit Cudkowicz, MD, MSC, Massachusetts General Hospital, Harvard Medical  
School

**8:15pm - 8:30pm**      **Group photo outside Biddle Mansion**

**8:30pm-9:30pm**      **Outdoor Buffet Dinner (Duke Terrace)**

**9:30pm-11:00pm**      **Evening Reception (Duke Terrace)**

## **Day 3: Sunday, September 22, 2024**

**7:00am-8:00am**      **Breakfast at the Winter Palace**

**8:00am-8:10am**      **Dr Barohn and Prof Hanna opening at Mary Duke Ballroom**

*Session IX: Fatigue for the fatigued (specialty coffees)*

**Moderator**

**8:10am-8:30am**      **Beyond weakness; the unyielding characteristic of fatigability in SMA - Jackie  
Montes, PT, EdD, Columbia University Irving Medical Center**

**8:35am-8:55am**      *Pain, fatigue and exercise in neuromuscular diseases: start low and go slow –  
Nicole Voet, MD, PhD, Radboud University*

**9:00am-9:15am**      *2023 Shark Tank Award update - Perceived Fatigability Tracker: Improving  
Assessment to Enhance Spinal Muscular Atrophy (SMA) Patient Outcomes Ralph  
Rodriguez-Torres, DPT, Columbia University*

**9:20am-11:00am**      **Shark Tank Session**

**Moderator: Aziz Shaibani, MD, FACP, FAAN, FANA**

**Shark Panel:** Dr. James Lilleker, Senda Ajroud-Driss, MD, Gordon Smith, MD

**Presentations: TBD by submitted proposals**

**Session X: AI**

**11:00am-11:20AM** *AI-Enhanced Insoles for Accurate Kinematic and Kinetic Gait Monitoring in SMA and DMD*, Damiano Zanotto, PhD – Stevens Institute of Technology

**11:25am-11:45am** *NM MRI* - Jasper Morrow – University College London

**11:50am-12:10pm** Innovative data analysis, Xing Song, Ph D – University of Missouri

**12:15pm-12:35am** AI/Omics - Cheng, Jianlin, PhD – University of Missouri

**Closing**

**12:35pm-1:30pm** **Lunch at the Winter Palace**

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