

Agenda

25TH ANNUAL NEUROMUSCULAR
STUDY GROUP SCIENTIFIC MEETING

THURSDAY, SEPTEMBER 19

6:00 – 9:00 pm

Dinner and Check In
WINTER PALACE

DAY 1: FRIDAY, SEPTEMBER 20

7:00 – 8:00 am

Buffet Breakfast
WINTER PALACE

Check In
DUKE MANSION

8:00 – 8:20 am

Welcome and State of the Neuromuscular Study Group
Dr. Richard Barohn and Prof Michael Hanna

SESSION I: GENETICS

*Moderators: Karlien Mul, M.D., Ph.D., and
Dr. Vino Vivekanandam*

8:20 – 8:40 am

Gene Therapy for DM.D. and SMA: milestones, lessons learned
and current challenges
Emma Ciafaloni, M.D., *University of Rochester*

8:45 – 9:05 am

Overview of potential genetic treatments for FSHD
Scott Harper, Ph.D., *Nationwide Children's Hospital*

9:10 – 9:30 am

Genetic Therapeutics in neuropathies/CMT
Mario Saporta, M.D., Ph.D., *University of Miami*

9:35 – 9:55 am

Genetic Therapeutics in Myotonic Dystrophy
Charles Thornton, M.D., *University of Rochester*

10:00 – 10:20 am

Neuromuscular genetic therapies COL6, HSN1
Dr. Haiyan Zhou, *University College London*

10:25 – 10:40 am

Refreshment/Exhibitor Break

SESSION II: FLASH PRESENTATIONS

Moderator: Brendan McNeish, M.D.

10:40 – 10:50 am

Refractory myasthenia gravis characterised by widespread
innate and adaptive immune system changes
Katy Dodd, MBChB, MRCP,
Manchester Centre for Clinical Neurosciences

10:52 – 11:02 am

Remote monitoring to improve adherence to physical exercise:
pilot experience at the NeMO site
Michela Nani, RN, *NeMo Clinical Center, Milan*

11:05 – 11:15 am

Trial of Oxaloacetate in ALS, TOALS
Katie Lillig, BS, *University of Kansas Medical Center*

11:17 – 11:27 am

Outcome Measures to Quantify Longitudinal Changes in
Motor Function in FHD
Lawrence Hayward, M.D., Ph.D., *UMASS Med*

11:30 – 11:40 am

Treatment effects on ambulation loss in Spinal Muscular
Atrophy Type III: insights from the Italian ISMAC registry
Giorgia Coratti, TNPEE, MsC, Ph.D.
*Catholic University of Sacred Heart Fondazione Policlinico
Universitario Agostino Gemelli IRCCS*

11:42 – 11:52 am

The Myasthenia Gravis Patient Registry: Characteristics,
Insights, and Learnings After a Decade (2013-23)
Kelly Graham Gwathmey, M.D.,
Virginia Commonwealth University

12:00 – 1:00 pm

Lunch Buffet
WINTER PALACE

MSG Executive Committee Meeting Breakout Lunch
MUSIC ROOM



SESSION III: PLATFORM PRESENTATIONS

Moderator: Amanda C. Guidon, M.D., MPH

1:00 – 1:15 pm

Co-designing a Strategy to Engage People with Neuromuscular Diseases from Racially Minoritized Backgrounds in Research
Gita Ramdharry, Ph.D., MSc, PGCert, BSc(Hons), MCSP
University College London

1:20 – 1:35 pm

A Study of the Common Factors that Influence Fatigue in Myasthenia Gravis
Kelly Graham Gwathmey, M.D.,
Virginia Commonwealth University

1:40 – 1:55 pm

Combined personalized home-based aerobic exercise and coaching to improve physical fitness in neuromuscular diseases – a multicenter, single-blind, randomized controlled trial
Eric Voorn, Ph.D., *University of Oxford*

2:00 – 2:15 pm

An analysis of Mortality Rates and Causes of Death in an Oxford Cohort of Adult Myasthenia Gravis Patients
Dr. Pietro Zara, *Amsterdam UMC*

2:15 – 2:30 pm

Refreshment/Exhibitor Break

SESSION IV: YOUNG INVESTIGATOR/ EVALUATOR/COORDINATOR

Moderators: Dr Michael Hehir, Dr. Vino Vivekanandam, Prof Valeria Sansone, and Dr. Karen Suetterlin

2:30 – 4:30 pm

Clinical research lessons from intramural NINDS: building our field of dreams
Lauren Reoma, M.D., FAAN
*Deputy Clinical Director, NINDS
Director, NINDS Clinical Trials Unit*

NMSG Resources, Fellowships

Breakouts

5:30 – 7:30 pm

Abstract Poster Session
CARRIAGE HOUSE, TARRYTOWN ROOM

7:30 – 9:00 pm

Buffet Dinner
WINTER PALACE

9:00 – 11:00 pm

Reception
WEST TERRACE

DAY 2: SATURDAY, SEPTEMBER 21

7:00 – 8:00 am

Buffet Breakfast
WINTER PALACE

Meet the Experts Breakfast

8:00 – 8:15 am

Opening
Dr. Barohn and Prof Hanna
MARY DUKE BALLROOM

SESSION V: NMS AND THE BODY

Moderator: Dr. Kathy Mathews

8:20 – 8:40 am

More than Muscles: Non Motor Manifestations of Neuromuscular Disorders
Julie Parsons, M.D., *Children's Hospital Colorado*

8:45 – 9:05 am

Cognitive SMA
Valeria Sansone, M.D., Ph.D., *NeMO Milan*

9:10 – 9:30 am

Cognitive involvement/deficits in myotonic dystrophy in children and adults
Nick Johnson, M.D., *Virginia Commonwealth University*

9:35 – 9:55 am

Cardiomyopathies in the Muscular Dystrophies
Carol Wittlieb-Weber, M.D., *Children's Hospital of Philadelphia*

10:00 – 10:15 am

Refreshments/Exhibitor Break

SESSION VI: NEUROPATHY

Moderator: W. David Arnold, M.D.

10:20 – 10:40am

Cryptic splicing: from foe to friend in tackling ALS and IBM
Pietro Fratta, M.D., Ph.D.
University College London and Francis Crick Institute

10:45 – 11:05 am

Overview and Advances in the work up and Management of Immune Mediated Peripheral Neuropathies
Karissa Gable, M.D., *Duke University School of Medicine*

11:10 – 11:30 am

Peripheral nerve imaging in CMT
Reza Seyedsadjadi, M.D., *Massachusetts General Hospital*

11:30 am – 1:00 pm

Lunch
WINTER PALACE

NMSG 2025 planning committee meeting breakout lunch
MUSIC ROOM



SESSION VII: CLINICAL TRIALS DESIGN

Moderators: *Dr. Michael Hehir and
Dr. Vino Vivekanandam*

1:00 - 2:20 p.m.

Clinical Design Presentations

Life of Clinical Trials

Gordon Smith, M.D., FAAN

Virginia Commonwealth University

N-of-1 Trials for Personalized Medicine

Mike McDermott, Ph.D., *University of Rochester*

Greener Trials

Dr. Vino Vivekanandam, MBBS(Hons), FRACP

University College London

Queen Square Institute of Neurology

Trial Delivery and Logistics

Matt Parton, MB, BChir, FRCP, Ph.D.

Recruiting/Engaging Pediatric Participants

Kathy Mathews, M.D., *University of Iowa*

Starting a Platform Trial - helpful tips

Merit Cudkowicz, M.D., MSc

Massachusetts General Hospital, Harvard Medical School

2:05 - 2:20 pm

Panel Q&A

2:20 - 2:50 pm

Refreshments/Exhibitor Break

SESSION VIII: INDUSTRY PRESENTATIONS

Moderator: *Michael Hehir, M.D.*

2:50 - 3:10 pm

Rare Disease Connect in Neurology (RDCN):

An international MG community and forum providing needs-driven medical education

James F. Howard, Jr., M.D., FAAN

Director, Myasthenia Gravis Clinical Trials and Translational

Research Program, The University of North Carolina at Chapel Hill

3:15 - 3:35 pm

Precision Genetic Medicines for Patients with Rare Neuromuscular Diseases

Damon Asher, Ph.D.

Senior Director, GMAL GT Team Lead, Sarepta Therapeutics

3:40 - 4:00 pm

A spotlight on the argenx pipeline: Innovation in the development of treatments for neuromuscular disease

Jeffrey Guptill M.D., MA, MHS, FAAN

Neuromuscular Franchise Lead, Clinical Development, argenx

4:05 - 4:25 pm

Exploring Corticosteroid Structure and Function in DMD

Omer Abdul Hamid, M.D.,

Nemours Children's Health | Orlando, Florida

4:30 - 4:50 pm

CHAMPION MG and Open-Label Extension Trial in Adult Patients with Generalized Myasthenia Gravis who are Anti-Acetylcholine Receptor Antibody-Positive

Gordon Smith, M.D., FAAN, *Virginia Commonwealth University*

4:55 - 5:15 pm

Efficacy and Safety of Targeted Immunotherapy with ANX005 in Treating Guillain-Barré Syndrome: A Phase 3 Multicenter Study

Henk-André Kroon, M.D., SVP

Head of Translational Medicine, Annexon Biosciences

FELLOW AND KEYNOTE PRESENTATIONS

Moderator: *William David, M.D., Ph.D.*

7:00 - 8:15 pm

NMSG Research Presentation: Development of Novel

Imaging Biomarkers for use in Pediatric Facioscapulohumeral Muscular Dystrophy

Natalie Katz, M.D.

NMSG Fellow, Duke University

Robert C. Griggs Annual NMSG Keynote Presentation:

ALS Updates: new treatments and trial approaches

Merit Cudkowicz, M.D., MSc

Massachusetts General Hospital, Harvard Medical School

8:15 - 8:30 pm

Group Photo

OUTSIDE BIDDLE MANSION

8:30 - 9:30 pm

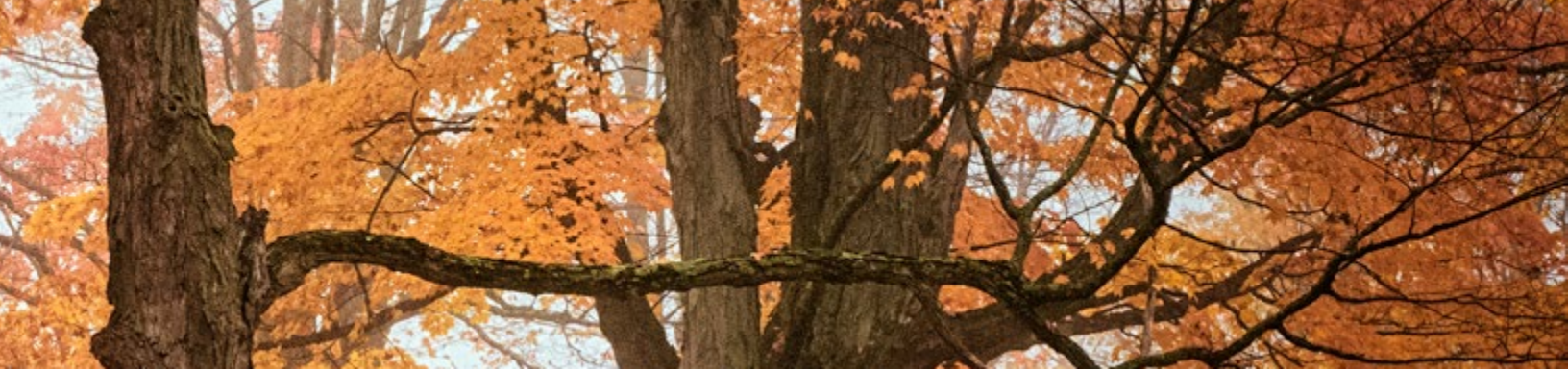
Outdoor Buffet Dinner

DUKE TERRACE

9:30 - 11:00 pm

Evening Reception

DUKE TERRACE



DAY 3: SUNDAY, SEPTEMBER 22

7:00 – 8:00 am

Breakfast
WINTER PALACE

7:15 – 9:15am

Specialty Coffees from the Espresso Guys
OUTSIDE THE MARY DUKE BALLROOM

8:00 – 8:10 am

Opening
Dr. Barohn and Prof Hanna
MARY DUKE BALLROOM

SESSION IX: FATIGUE FOR THE FATIGUED

Moderator: Jacqueline Montes, PT, EdD

8:10 – 8:30 am

Beyond weakness; the unyielding characteristic of fatigability in SMA
Jacqueline Montes, PT, EdD
Columbia University Irving Medical Center

8:35 – 8:55 am

Pain, fatigue and exercise in neuromuscular diseases: start low and go slow
Nicole Voet, M.D., Ph.D., *Radboud University*

9:00 – 9:15 am

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

SHARK TANK SESSION

Moderator: Aziz Shaibani, M.D., FACP, FAAN, FANA

Shark Panel: Dr. James Lilleker, Senda Ajroud-Driss, M.D., Dr. Amanda Guidon, Gordon Smith, M.D.

9:20 – 11:00 am

Shark Tank Presentations

MAPP: MRI as a biomarker in Periodic Paralysis.
A prospective longitudinal pilot study in periodic paralysis
Dr. Murva Asad, *University College London*

Is a mucosal trigger responsible for MuSK myasthenia gravis?
Gianvito Masi, M.D., *Yale University*

Fluctuations in Liver and Renal Function Tests in Myotonic Dystrophy Type 1 (Dml): When Should We Worry
Carola Rita Ferrari Aggradi, M.D.
NeMO Clinical Center, Neurorehabilitation Unit

Efficacy and Safety of Low Dose of anti-CD20 Therapy for New Onset Acetylcholine Receptor Antibody Positive Myasthenia Gravis in Older Adults
Pietro Zara, M.D., *Nuffield Department of Clinical Neurosciences, University of Oxford*

SESSION X: AI

Moderator: Karlien Mul, M.D., Ph.D.

11:00 – 11:20 am

AI-Enhanced Insoles for Accurate Kinematic and Kinetic Gait Monitoring in SMA and DM.D.
Damiano Zanotto, Ph.D., *Stevens Institute of Technology*

11:25 – 11:45 am

AI Tools in Muscle MRI Segmentation and Diagnosis
Jasper Morrow, Ph.D., *UCL*

11:50 am – 12:10pm

Towards Better Understanding of ALS using a Multi-Marker Discovery Approach from a Multi-Modal Database
Xing Song, Ph.D., *University of Missouri*

12:15 – 12:35am

AI methods for integrating multi-omics data and inferring gene regulatory networks
Jianlin Cheng, Ph.D., *University of Missouri*

Shark Tank Winner Announcement

Final Remarks

12:35 – 1:30pm

Lunch
WINTER PALACE