

26th Annual Neuromuscular Study Group Scientific Meeting

Thursday, September 25, 2025

Aperitivo and check in 5-7PM

Day 1: Friday, September 26, 2025

6:30am-8:00am	Buffet Breakfast and Check-In
8:00am-8:15am	Welcome and State of the Neuromuscular Study Group Dr. Richard Barohn and Prof Michael Hanna
8:15am-8:30am	OPENING Scientific Meeting Summary <i>Session I: Gene Based Therapeutics</i>
8:30am-8:50am	Management of Select Adverse Events Following Delandistrogene Moxeparovec Gene Therapy for Patients with Duchenne Muscular Dystrophy - Craig Zaidman, MD – Wash U, St Louis
8:55am-9:15am	Liver involvement in AAV gene - Prof Anil Dhawan, MD, FRCPC, H, King's College London
9:20am-9:40am	Newborn screening – Bo Hoon Lee, MD, University of Rochester
9:45am-10:05am	How to arrange the set-up and infrastructure at any place for gene therapy, what is needed – Emma Cialfoni, MD, University of Rochester
10:10am-10:25am	Refreshment/Exhibitor Break <i>Session II: Platform Presentations</i>
10:25am –10:40am	TBD by highest scored abstracts
10:45am-11:00am	TBD by highest scored abstracts
11:05am-11:20am	TBD by highest scored abstracts
11:25am-11:40am	TBD by highest scored abstracts

11:45am-12:00pm TBD by highest scored abstracts
12:00pm-1:15pm Lunch
12:00pm-1:15pm *NMSG Executive Committee Meeting Breakout Lunch*

Session III – Flash Presentations

1:15pm-1:20pm TBD by scored abstracts
1:22pm-1:27pm TBD by scored abstracts
1:29pm-1:34pm TBD by scored abstracts
1:36pm-1:41pm TBD by scored abstracts
1:43pm-1:48pm TBD by scored abstracts
1:50pm-1:55pm TBD by scored abstracts

Session IV: Clinical Trial Units

2:00pm-2:20pm **Establishing a clinical trial site for both academic and private practices, from A to Z, as HR needs, studies recruitments, budgets, quality control** – Yessar Hussein, MD and Aziz Shaibani, MD

2:25pm-2:45pm ***Placebo response and placebo effect: is there a difference?***
Fabrizio Benedetti, MD, MAE

2:50pm-3:10pm **Setting up a Clinical Trial Unit: the experience at the NeMO site** -
Valeria Sansone, MD, PhD

3:15pm-3:35pm ***Allied Health professionals in Clinical trial delivery – a QS experience*** - Gita Ramdharry, PhD, UCL

3:35pm-3:45pm **Refreshment/Exhibitor Break**

3:45pm-5:00pm **Session V: Young Investigator Session/Evaluator Session
Breakout; Coordinator Breakout**

- 6:00pm-8:00pm Abstract poster session
- 8:00pm-9:00pm Dinner @ Regina Palace (group picture)
- 9:00pm-11:30pm Reception @ The Bar, Regina Palace

Day 2: Saturday, September 27, 2025

- 7:00am-8:00am Buffet Breakfast
- 8:30am-8:45am Dr Barohn and Prof Hanna Opening
- 8:45am-9:05am **Session II: AI/E-Health**
MRI as a biomarker in neuromuscular disorders - Prof. John Thornton, QS UCL Institute of Neurology
- 9:10am-9:30am **AI in the neuromuscular clinic: how can it help?** - Saverio d'Amico, Humanitas Research Hospital, Milan
- 9:35am-9:55am **Clinical Trials Powered by AI - TBD**
- 10:00am-10:45am **Session: VI Debate "To Treat or not to Treat"**
Angela Genge, MD, McGill University
John Vissing, MD, University of Copenhagen
- 10:45am-11:00am Break
- Session VII: Outcome Measures and how to approach regulatory requirements with the FDA: Lessons Learned**
- 11:00am-11:20am TBD
- 11:25am-11:45am TBD
- 11:45am-12:45pm Lunch
- Session VIII: Smorgasbord New clinical Phenotypes**
- 12:45pm-1:05pm **Health economics to clinical practice** – Susanna Gallani, PhD, Harvard Business School
- 1:10am-1:30pm **New phenotypes and outcome measures for myotonic dystrophy**, Johanna Hamel, MD, University of Rochester

1:35pm-1:55pm **Clinical features of the peripheral neuropathies that occur in large arteriole vasculitis as compared to nerve microvasculitis**, P. James Dyck, MD, Mayo Clinic

Break

Session IX – Industry updates

2:10pm-2:30pm *

2:35pm-2:55pm *

3:00pm-3:20pm *

3:25pm-3:45pm *

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5:30pm-6:50pm **2025 NMSG Fellow Sophie Rengarajan, MD, PhD**

Robert C. Griggs Keynote Lecture – Prof Valeria Sansone, NEMO

6:50pm *Buses to La Tenuta del Golfo*

7:00pm-11:30pm **aperitivo/dinner/evening reception**

Day 3: Sunday, September 28, 2025

7:00am-8:00am **Breakfast**

8:15am-8:30am **Dr Barohn and Prof Hanna opening**

Session X: NMSG Young investigators projects

8:30am-8:45am **MAPP: MRI as a biomarker in Periodic Paralysis. A prospective longitudinal pilot study in periodic paralysis**, Dr. Murva Asad, University College London

8:50am-9:05am **Is a mucosal trigger responsible for MuSK myasthenia gravis?** Gianvito Masi, MD, Yale University

9:10am-10:45am **Session XI: Shark Tank Session**

This is an opportunity for young investigators to present a research proposal with a neuromuscular focus for pump-prime funding of \$10,000. The scheme is

intended to drive new and developing research collaborations and result in further grant applications.

10:45am-11:00am ***Refreshments/Exhibitor Break***

Session XII: Caution in the interpretation and conclusions on some trials

11:00am-11:15am **Unresolved issues with efgartigimod in CIDP, and broader concerns for future trial designs, Jonathan S Katz, MD**

11:20am-11:35am ***SOD1*, Suma Babu, MBBS, MPH, Mass General Hospital, Harvard Medical School**

11:40am-11:55am ***TBD***

Meeting Closing

12:00pm-1:30pm **Lunch**

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