

A scenic view of Lake Lugano, Italy, with the town of Lugano in the foreground and the Alps in the background. The lake is a deep blue, and the mountains are a mix of green and brown. The town of Lugano is built on a hillside, with red-roofed buildings and a large church. In the middle of the lake, there is a small island with a church and a castle. The sky is a clear, pale blue.

# **26<sup>th</sup> Annual NMSG Scientific Meeting**

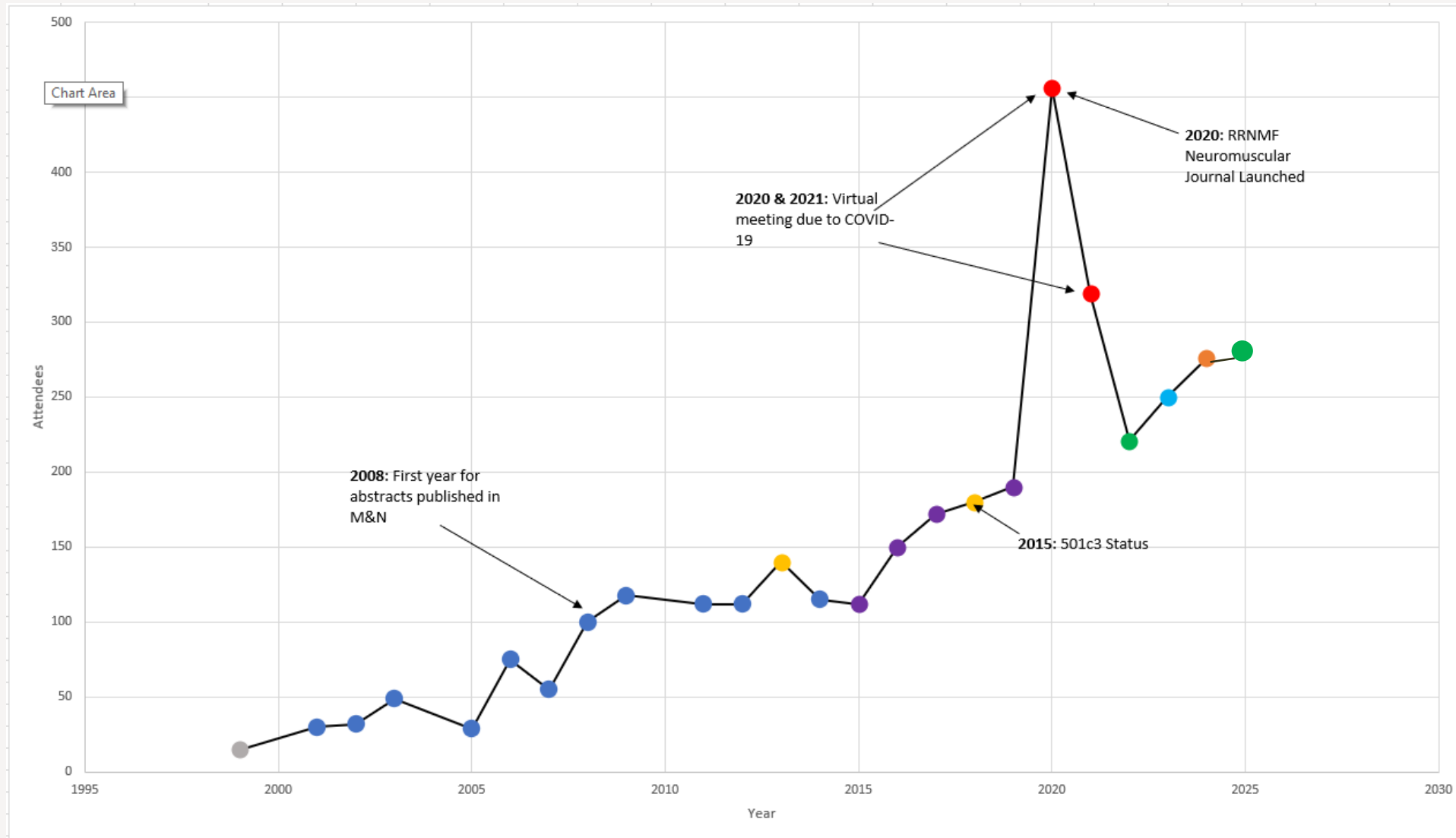
**Day One: Friday, September 26, 2025**

# Welcome/Benvenuti!

- 275 registered attendees
- 74 of those are junior investigators
- **Attendees from 14 countries:** Brazil, Canada, Denmark, France, India, Italy, The Netherlands, Poland, Saudia Arabia, Slovakia, Spain, Switzerland, United Kingdom, United States



# History of the NMSG Annual Meeting



- Chicago, IL
- Beaver Hollow
- England
- Snowbird, UT
- Remote
- Stresa, Italy
- Orlando, FL
- Tarrytown, NY

# NMSG Goals

- Maintain position as the premier organization and annual meeting for neuromuscular clinical trials.
- Support and develop young investigators in the field.
- Foster international collaboration.
- Connect to pre-clinical investigators who are exploring potential pathways for new treatments for neuromuscular disease.
- Engage with the industry.





# 2025 NMSG Planning Committee

**Valeria Sansone, M.D., Ph.D., Co-Chair**

*NeMO, Milan*

**Emma Ciafaloni, M.D., Co-Chair**

*University of Rochester*

**Richard J. Barohn, M.D., NMSG Chair**

*University of Missouri*

**Prof Michael Hanna, M.D., NMSG Co-Chair**

*University College London Institute of Neurology*

**Michael Hehir, M.D., Past Chair**

*University of Vermont Medical Center*

**Amanda Guidon, M.D.**

*Massachusetts General Hospital*

**Jose Manuel Sanz Mengibar, Ph.D.**

*University College London*

**Karen Suetterlin, MBBS, MRCP, Ph.D.**

*Newcastle University*

**Prof John Vissing, M.D.**

*Copenhagen University Hospital*

**Vino Vivekanandam, Ph.D., 2026 Planning Chair**

*University College London*



# 2025 NMSG Executive Committee

**Richard J. Barohn, M.D., Chair**

*University of Missouri*

**Michael Hanna, M.D., Chair**

*University College London*

**Robert Griggs, M.D., Immediate Past Chair**

*University of Rochester School of Medicine*

**Emma Ciafaloni, M.D., FAAN, Director, NMSG Coordination Center**

*University of Rochester Medical Center*

**Mazen Dimachkie, M.D., Treasurer**

*University of Kansas Medical Center*

**Michael McDermott, Ph.D., Biostatistician**

*University of Rochester Medical Center*

**Michaela Walker, MPH, CCRP,**

**Coordinator Member, University of Kansas Medical Center**

**Amanda Guidon, M.D., MPH,**

**Investigator Member,**

*Massachusetts General Hospital*

**James Lilleker, MBChB, MRCP, Ph.D.,**

**Investigator Member,**

*University of Manchester*

**John Vissing, M.D., Investigator Member,**

*University of Copenhagen*

**Bhaskar Roy, MBBS, MHS, Young**

**Investigator Member, Yale School of Medicine**

**Michael Hehir, M.D., Outgoing**

**Investigator Member,**

*University of Vermont Medical Center*

**Valeria Sansone, M.D., Ph.D.,**

**Incoming Investigator Member, NeMO Milan**



# Special thanks to:

Amardeep Gill, *Livestream Director, StreamGuru.net*

Roberto and Lucrezia Petruzzelli, *Word of Mouth AV*

Liz Russo Paulk, *NMSG Administrative Manager*

Donna Schwartze, *NMSG Administrative Assistant*

Grace Reap, *University of Kansas Medical Center*

Charlie Brennan, *University of Missouri*

J. Andrea Sierra Delgado, M.D., *University of Missouri*

Christine Oldfield, *University College London*

Susanna Pozzi, *NeMO Milan*

Vera Bril, M.D., *University of Toronto*

Eleonora Padulazzi, *Hotel Regina Palace*



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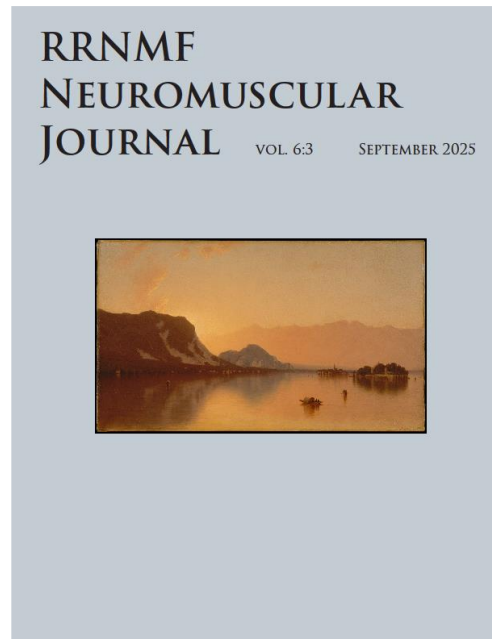
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# NMSG Abstracts

The accepted abstracts for this conference are published in *RRNMF Neuromuscular Journal*. Use the QR code in your agenda book to retrieve them.



# Continuing Education

In support of improving patient care, this activity has been planned and implemented by Amedco LLC and Neuromuscular Study Group (NMSG). Amedco LLC is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.

## Amedco Joint Accreditation #4008163

Physicians (ACCME) Credit Designation Amedco LLC designates this live activity for a **14.25 credits** *AMA PRA Category 1 Credits™*. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

**This information is also included in the meeting's agenda booklet.**



# General Meeting Information

- Restrooms are directly upstairs.
- Water and coffee all day in the Gallè room.
- Speakers - Please provide your presentation on a USB drive to Amardeep Gill, our onsite AV expert, as early as possible so your slides can be loaded into our laptop computer.
- If you have a question after a presentation, raise your hand and we'll bring a microphone to you.
- If you have questions not related to the presentation, please ask a member of our staff at the table just outside the Gallè room.
- The NMSG has a special WiFi access for meeting attendees. This network can be used throughout the hotel.

**Network name:** Neuromuscular Study

**Password:** NMSG@LakeMaggiore



# Friday Highlights

**Sessions** – Gene-Based Therapeutics, Platform Presentations, Flash Presentations, Clinical Trial Units, and the Young Investigators Session

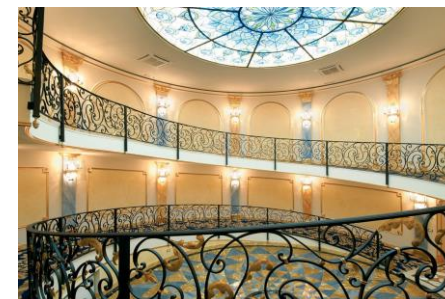
## **6-8pm – Abstract Poster Session – Tiffany Room**

- If you're presenting, have your poster up and ready to go by 5:00pm.
- Use any board that's available. There's space on both sides of each board for presentations.
- Remove your poster right after the session ends or they will be disposed of,

**8:05pm – Group picture in the Rotunda**

**8:15pm – Dinner in New Liberty**

**9:30pm – Reception in the hotel bar**



Rotunda



# One Last Thought

Whether you are presenting a poster or discussing trials between sessions, we encourage you to engage fully with your colleagues and our industry partners as your active participation will greatly strengthen the value of our time together.

**Andiamo!**





# **26<sup>th</sup> Annual NMSG Scientific Meeting**

**Day Two: Saturday, September 27, 2025**

# Reminders

- If you have a question after a presentation, raise your hand and we'll bring a microphone to you.
- If you have questions not related to the presentation, please ask a member of our staff at the table just outside the sponsor room.
- The NMSG has a special WiFi access for meeting attendees. This network can be used throughout the hotel.

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# Become an NMSG Member!

1. On the NMSG website the application is under the membership tab.
2. The NMSG Executive Committee will review your application and contact you with any questions.
3. If your site is approved, we will invoice you for \$500 which is good for the calendar year and includes all researchers at your site.

## Questions?

Ask our group's administrators –  
Liz Paulk or Donna Schwartze



# NMSG Member Benefits

- Discounted registration to this meeting
- Brainstorming potential study ideas
  - Steering committee input
- Critiquing grant submissions
- Mentorship
- Database management
- Statistical support
- Funding assistance
- Shark Tank competition
- Hang out in cool places with other neuromuscular academics and industry partners



# 2025 NMSG Member Sites

- Austin Neuromuscular Center
- Brigham and Women's Hospital
- Cedars-Sinai Los Angeles
- Centro Clinico NEMO –  
Universitario Agostino Gemelli
- Copenhagen Neuromuscular  
Center
- Indiana University
- Louisiana State University
- Massachusetts General Hospital
- McGill University
- Nationwide Children's Hospital
- Nerve and Muscle Center of Texas
- NorthShore University Health  
System
- Northwestern University
- Ohio State University
- Penn State University
- Stanford Medicine
- SUNY Downstate
- University College London
- University of California, Irvine
- University of Colorado Denver
- University of Florida Health  
Science Center, Jacksonville
- University of Florida, Gainesville
- University of Iowa
- University of Kansas Medical  
Center
- University of Manchester
- University of Mass Medical School
- University of Miami
- University of Milan, NEMO
- University of Missouri
- University of Nebraska
- University of North Carolina
- University of Pittsburgh
- University of Rochester  
Medical Center
- University of São Paulo -  
Brazil
- University of Texas Health  
Science Center San Antonio
- University of Utah
- University of Vermont
- Virginia Commonwealth  
University
- Yale University School of  
Medicine

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# Saturday Highlights

**General Session** – AI/E Health, Regulatory Requirements, New Clinical Phenotypes, Industry Updates

**6:00pm**

**2025/26 NMSG Fellow Presentation**

*Sophie Rengarajan, M.D., Ph.D. UCLA Medical Center*

**Robert C. Griggs Keynote Lecture**

*Prof Valeria Sansone, M.D., Ph.D. NeMO Clinic*

**REMINDER: If you're registered for tonight's Gala dinner at La Tenuta del Golfo:**

**6:45pm** - Meet buses at the back entrance of the Regina Palace

**7:00pm** - Aperitivo

**8:00pm** - Dinner

**9:00pm** - Reception





# **26<sup>th</sup> Annual NMSG Scientific Meeting**

**Day Two: Saturday, September 27, 2025**



# 2025/26 NMSG Fellow Presentation

Interrogating a Role for Endothelial Cells  
in Modulating Pathophysiology and  
Gene Therapy Response in Human DMD  
Using Single Nuclei Transcriptomics

*Sophie Rengarajan, M.D., Ph.D.*  
*UCLA*



# Robert C. Griggs Annual NMSG Keynote Presentation

**2017:** Gene Therapy Development, *Brian Kasper, Ph.D.*

**2018:** A Small Circle of Life; Genetics, Disease, and Prevention, *Professor Sir Doug Turnbull*

**2019:** Clinical Trials in Rare Neuropathies: Right, Wrongs, and Remedies, *Richard Hughes, M.D.*

**2020:** How the FSHD Puzzle Was Solved, *Rabi Tawil, M.D.*

**2021:** Idiopathic Does Not Mean Cryptogenic: Therapeutic Development for Metabolic Neuropathy, *Gordon Smith, M.D.*

**2022:** Neuromuscular Diseases; Lessons Learned 1980-2020, *Shree Pandya, PT, DPT*

**2023:** The Circle of Translation, *Professor Mary Reilly*

**2024:** ALS Updates: New Treatments and Trial Approaches, *Merit Cudkowicz, M.D., MSc*



# 2025 NMSG Keynote Presentation

It Takes a Team: Lessons in  
Leadership and Collaboration in  
Myotonic Dystrophy and Beyond

*Prof Valeria Sansone, M.D., Ph.D.*  
*NeMO, Milan*

The image shows the Regina Palace, a large, ornate, multi-story hotel building with a light-colored facade and numerous windows. The building features decorative architectural elements, including domes and spires. A large sign on the roof reads "REGINA PALACE". The hotel is surrounded by a well-manicured garden with colorful flower beds and a gravel path. In the foreground, there is a large, circular garden bed with yellow and red flowers. The sky is clear and blue.

# **26<sup>th</sup> Annual NMSG Scientific Meeting**

**Day Three: Sunday, September 28, 2025**

# Sunday Highlights

**General Session** – Young Investigators Projects, Shark Tank, Caution in the Interpretation and Conclusions of Some Trials

**9:25AM** – Shark Tank Presentations by:

*Silvia Falso, M.D.*

*Grace McMacken, MBBCh, Ph.D.*

*Laura Tufano, M.D.*

*Carola Rita Ferrari Aggradi, M.D.*

**New this year, the audience will be able to score the presentations so be sure to attend!**

**12:15pm** – Shark Tank winner announced.



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Susanna Pozzi, *NeMO Milan*

Vera Bril, M.D., *University of Toronto*

Eleonora Padulazzi, *Hotel Regina Palace*



# A Fond (and very reluctant) Farewell to Liz Paulk

- **Began with the NMSG in 2018, since then:**
  - 1385 in-person attendees
  - 775 online attendees
  - 120 sponsors
  - 20 Executive Committee members
  - 57 Planning Committee members
  - 6 cities with onsite meetings
  - 2 successful online meetings
  - Lots of \$\$ raised!
- 2026 - one wedding!



# And welcoming, Donna Schwartze

- Bachelor of Journalism from The University of Missouri
- Master of Arts in Public Relations from Webster University
- Extensive domestic and international meeting planning experience
- Worked in sports public relations for the majority of her career including owning her own company where she managed the careers of four NBA basketball players.
- Now - a novelist. Working on her 11<sup>th</sup> book which she plans to publish by the end of the year.



# A SPECIAL THANK YOU!!



To Dr. Valeria Sansone, Dr. Emma Ciafaloni, and the entire 2025 planning committee for making this meeting a huge success!



**And thanks again to  
our 2025 sponsors!**



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Join us next year in  
San Antonio, Texas,  
from September 25-27.



See you next year!

