

25th 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 |
| | <i>Session I: Genetics</i> |
| | <i>Moderator</i> |
| 8:20am-8:40am | <i>Gene Therapy for DMD and SMA: milestones, lessons learned and current challenges</i> – Emma Ciafaloni, MD, University of Rochester |
| 8:45am-9:05am | <i>Overview of potential genetic treatments for FSHD</i> – Scott Harper, PhD, Nationwide Children’s Hospital |
| 9:10am-9:30am | <i>Genetic Therapeutics in neuropathies/CMT</i> - Mario Saporta, MD, PhD – University of Miami |
| 9:35am-9:55am | <i>side effects of Gene Therapy</i> - TBD |
| 10:00am-10:20am | <i>Neuromuscular genetic therapies COL6, HSN1</i> - Haiyan Zhou, University College London |
| 10:25am-10:40am | Refreshment/Exhibitor Break |
| | <i>Session II: Flash Presentations</i> |
| | <i>Moderator -</i> |
| 10:40am-10:50am | scored submitted abstracts |
| 10:52am-11:02am | scored submitted abstracts |

11:05am-11:15am scored submitted abstracts

11:17am-11:27am scored submitted abstracts

11:30am-11:40am scored submitted abstracts

11:42am-11:52am scored submitted abstracts

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

1:00pm –1:15pm highest scored abstracts submitted

1:20pm-1:35pm highest scored abstracts submitted

1:40pm-1:55pm highest scored abstracts submitted

2:00pm-2:15pm highest scored abstracts submitted

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

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

Dr Michael Hehir and Dr Vino Vivekanandam and Prof Valeria Sansone to moderate

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 Dinner (Winter Palace)

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

Day 2: Saturday, September 21, 2024

7:00am-8:00am Buffet Breakfast (Winter Palace)

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

Session V: NMS and the body

Moderator

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

10:20am -10:40am Value of Extensive Testing - TBD

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

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

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, VCU*

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

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

1:50-1:55 – *lessons from acute trials* – TBD

1:55-2:00 – *recruiting/engaging paediatric participants* – Kathy Mathews, MD, University of Iowa

2:00-2:05 – *practical tips for platform trials* – Merit Cudkowicz, MD, Massachusetts General Hospital, Harvard Medical School

2:05 – 2:40 - *panel Q&A*

2:40pm-3:10pm

Refreshments/Exhibitor Break

Session VIII: Industry Presentations

Moderator

3:10pm-3:30pm

3:35pm-3:55pm

4:00pm-4:20pm

4:25pm-4:45pm

4:50pm-5:10pm

5:15pm-5:35pm

7:00pm – 8:15pm

NMSG Research Presentation

**Development of Novel Imaging Biomarkers for use in Pediatric
Facioscapulohumeral 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

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** *Fatigue in neuromuscular diseases* – 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** Instrumented insoles (AI Sole) to accurately collect kinematic and kinetic gait parameters in SMA and DMD Damiano Zanotto, PhD – Stevens Institute of Technology
- 11:25am-11:45am** *NM MRI* - Jasper Morrow – UCL
- 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**
- 12:30pm-1:30pm** Lunch at the Winter Palace