

AGENDA

THURSDAY, SEPTEMBER 19, 2019

We will have early check in 5:30-7 p.m. on Level C at the top of the escalators for those who arrive early on Thursday.

DAY 1: FRIDAY, SEPTEMBER 20, 2019

6:30-8 a.m. Buffet Breakfast & Check-In
Golden Cliff/Eagles Nest

We ask for posters to be set up for display in the Primrose Room after 11 a.m.

8-8:20 a.m. Welcome and State of the Muscle Study Group Address
Ballrooms 2 & 3
Richard Barohn, M.D. & Michael Hanna, M.D.

8:20-8:40 a.m. NIH sIBM Natural History Study
Tahseen Mozaffar, M.D.

8:45-9:05 a.m. IPANEMA
Tahseen Mozaffar, M.D.

9:10-9:30 a.m. Memantine
Richard Barohn, M.D.

9:35-9:55 a.m. IBM International Genetics Consortium Study
Michael Hanna, M.D.

10-10:20 a.m. FOR-DMD
Robert Griggs, M.D.

10:20-10:35 a.m. Break
Ballroom Lobby

10:40-10:55 a.m. MGSCig
Mazen Dimachkie, M.D.

11-11:20 a.m. Arimoclomol in IBM
Mazen Dimachkie, M.D.

11:25-11:45 a.m. A Randomized, Prospective Trial of Immunosuppressant Dose Reduction in Stable Myasthenia Gravis
Michael Hehir, M.D.

11:50 a.m.-12:10 p.m. CSL Behring Update
Mazen Dimachkie, M.D.

12:10-12:15 p.m. The Myositis Association
Mazen Dimachkie, M.D.

12:15-1:30 p.m. Lunch
Golden Cliff/Eagles Nest

12:15-1:30 p.m. Muscle Study Group Executive Committee Meeting lunch
Magpie A

1:30-5:30 p.m. Young Investigator Workshop
Ballrooms 2&3
Richard Barohn, M.D., William Meurer & Richard Hughes, M.D.

1:30-3 p.m. Clinical Evaluators and Study Coordinator Breakout: Clinical Trial Design and Management
Magpie B
Richard Barohn, M.D., Laura Herbelin, Lindsay Alfano, DPT & Heidi Runk, CCRP

6-7:30 p.m. Dinner
Golden Cliff/Eagles Nest
Sponsored by Sarepta Therapeutics

7:30-9 p.m. Poster Walk Through Session
Primrose Room

9 p.m.-Midnight Networking Reception
Primrose Room & Patio
Sponsored by MT Pharma

9 p.m.-Midnight

DAY 2: SATURDAY, SEPTEMBER 21, 2019

7:30-8:30 a.m. Buffet Breakfast
Golden Cliff/Eagles Nest

7:30-8:30 a.m. Breakfast meeting for New Members and First Time Attendees
Magpie A
Hosted by Richard Barohn, M.D., Michael Hanna, M.D. & Robert Griggs, M.D.

8:30-10:10 a.m. Session I: Why Trials Fail - Lessons Learned
Ballrooms 2 & 3
Moderator: Aisling Carr, Ph.D.

8:30-8:50 a.m. What Have We Learned: CMT Trials
Michael Shy, M.D., University of Iowa

8:55-9:15 a.m. Why trials Dr. Barohn's Team Has Been Involved in Were Negative or Failed
Richard Barohn, M.D., University of Kansas Medical Center

9:20-9:40 a.m. An MSG IIT with Industry Partnership: Arimoclomol for IBM. Opportunities & Challenges
Mazen Dimachkie, M.D., University of Kansas Medical Center

9:45-10:05 a.m. Trials in ALS
Jon Katz, M.D., California Pacific Medical Center

10:10-10:30 a.m. Refreshments/Exhibitor Break
Primrose Room

10:30-11:40 a.m. Session II: Novel Trial Designs/Biomarker Development
Ballroom 2 & 3
Moderator: Tom Lloyd, M.D., Ph.D.

10:30-10:50 a.m. Development of an Antisense Oligonucleotide Therapeutic for SOD1 ALS
Tim Miller, M.D., Ph.D., Washington University of St. Louis

10:55-11:15 a.m. Bayesian Adaptive Design
Byron Gajewski, Ph.D., University of Kansas Medical Center

11:20-11:40 a.m. Biomarker Development in ALS and Use in Clinical Trials
Robert Bowser, Ph.D., Barrow Neurological Institute

11:45 a.m.-1 p.m. Lunch
Golden Cliff/Eagles Nest

1:15-3:05 p.m. Session III: Particular Challenges in Complex Intervention Trials (AHP)
Ballroom 2 & 3
Moderator: Gita Ramdharry, Ph.D.

1:15-1:35 p.m. Under-Represented Populations
Kim Kimminau, Ph.D., University of Kansas Medical Center

1:40-2 p.m. It's Complicated: Making Sense of Exercise Trials in ALS/MND
Vanina Dal Bello, Ph.D., McMaster University

2:05-2:25 p.m. Exercise in Diabetic Neuropathy
Rob Singleton, M.D., University of Utah

2:30-2:50 p.m. Challenges of Exercise and Diet Interventions
Jeff Burns, M.D., University of Kansas Medical Center

2:55-3:10 p.m. Break
Primrose Room

3:15-4:50 p.m. Session IV: New Therapies and How We Test Them
Ballrooms 2&3
Moderator: Carolina Barnett-Tapia, M.D., Ph.D.

3:15-3:35 p.m. New Therapies and How We Test Them: ATTR Polyneuropathy and HSAN1
David Hermann, MBBCh, University of Rochester Medical Center

3:40-4 p.m. FSHD - Network
Jeff Statland, M.D., University of Kansas Medical Center

4:05-4:25 p.m. The Role of Complement in the Treatment of Myasthenia Gravis
James "Chip" Howard, M.D., University of North Carolina Hospitals

4:30-4:50 p.m. Exercise in Spinal Muscular Atrophy
Sally Dunaway Young, DPT, Stanford University

6-6:15 p.m. MSG Group Picture
Outside Cliff Lodge

6:15-7:30 p.m. Dinner
Golden Cliff/Eagles Nest
Sponsored by PTC Therapeutics

7:40-8:40 p.m. Robert C. Griggs Annual MSG Keynote Speaker - Clinical Trials in Rare Neuropathies: Rights, Wrongs and Remedies
Ballroom 2&3
Richard Hughes, M.D., Honorary Professor, Institute of Neurology, University College London

9 p.m.-Midnight Campfire & S'mores Reception
The Atrium & Atrium Patio
Sponsored by CSL Behring

DAY 3: SUNDAY, SEPTEMBER 22, 2019

7-8 a.m. Breakfast
Golden Cliff/Eagles Nest

8-10:10 a.m. Platform Presentations
Ballrooms 2&3

8-8:10 a.m. Proximal Nerve Imaging in CMT
Fellow Reza Seyedsadjadi, M.D.

8:15-8:25 a.m. Elucidating the genetic etiology of neuromuscular disorders using high-throughput sequencing approaches
Fellow Davut Pehlivan, M.D.

8:30-8:40 a.m. Characterization of clinical and molecular findings in a large Brazilian cohort of ANO5 myopathy

8:45-8:55 a.m. Engaging ALS Research Ambassadors to Help Design the REFINE-ALS Biomarker Study

9-9:10 a.m. Excessive Daytime Sleepiness and disruption of sleep architecture in DM1

9:15-9:25 a.m. Longitudinal radiological and functional biomarkers in facioscapulohumeral dystrophy: Update from the Seattle Wellstone Project

9:30-9:40 a.m. Intrathecal Administration of AVXS-101 Gene-Replacement Therapy for Sitting but Non-Ambulatory Patients with Spinal Muscular Atrophy (SMA): Phase 1/2a Study (STRONG)

9:45-9:55 a.m. Characterizing divergent phenotypes in limb girdle muscular dystrophies

10-10:10 a.m. Evaluation of Default Mode Network Functional Connectivity in Children with Congenital Myotonic Dystrophy

10:15-10:25 a.m. Break
Ballroom 3 Lobby

10:30 a.m.-12:30 p.m. Session V: Sponsor Updates

10:30-10:50 a.m. MT Pharma

10:55-11:15 a.m. Sarepta Therapeutics

11:20-11:40 a.m. AveXis

11:45 a.m.-12:05 p.m. PTC Therapeutics

12:10-12:30 p.m. Takeda/Shire

12:30-1:30 p.m. Lunch
Golden Cliff/Eagles Nest
Sponsored by AveXis