

2019 Muscle Study Group Scientific Annual Meeting

Challenges in clinical trials for rare neuromuscular diseases

September 20-22, 2019 in Snowbird, UT

Day 1: Friday, September 20, 2019

6:30am-7:45am	Buffet Breakfast and Check-In
8:00am-8:30am	Welcome and State of the Muscle Study Group Richard Barohn, <i>M.D.</i> and Michael Hanna, <i>M.D.</i>
8:30am-8:50am	NIH sIBM Natural History Study <i>Tahseen Mozaffar, M.D.</i>
8:55am-9:15am	IPANEMA <i>Tahseen Mozaffar, M.D.</i>
9:20am-9:40am	Memantine <i>Richard J. Barohn, M.D.</i>
9:45am-10:05am	IBM International Genetics Consortium Study <i>Pedro Machado, M.D., and Prof Michael G. Hanna</i>
10:10am-10:30am	FOR-DMD <i>Robert Griggs, M.D.</i>
10:30am-10:45am	Break
10:45am-11:05am	MGSClg - Sub Cut IG in CIDP & MG <i>Mazen Dimachkie, M.D.</i>
11:10am-11:30am	Arimoclomol in IBM <i>Mazen Dimachkie, M.D.</i>
11:35am -11:50am	A Randomized, Prospective Trial of Immunosuppressant Dose Reduction in Stable Myasthenia Gravis <i>Michael Hehir, M.D.</i>
12:00-1:00pm	Lunch <i>Muscle Study Group Executive Committee Meeting</i>

1:00pm-5:30pm	Young Investigator Workshop William Meurer, Richard Hughes, Richard Barohn, Laurie Gutmann
1:00pm-3:00pm	Clinical Evaluators and Study Coordinator breakout session <i>Clinical Trial Design and Management</i> Richard Barohn, Laura Herbelin, Lindsay Alfano, DPT, Heidi Runk CCRP
6:00pm-7:30pm	Dinner
7:30 pm - 9:00 pm	Poster walk through session
9:00 pm-Midnight	Campfire and S'mores Reception

Day 2: Saturday, September 21, 2019

7:30am-8:30am	Buffet Breakfast
7:30am-8:30am	Breakfast meeting for New Members and 1st time attendees hosted by Dr. Barohn, Dr. Hanna, and Dr. Griggs
8:30am-10:10am	Session I: Why trials fail- lessons learned Moderator: Aisling Carr
8:30am-8:50am	Speaker Michael Shy: <i>what have we learned CMT trials</i>
8:55am-9:15am	Speaker Richard Barohn: <i>Why trials Dr. Barohns' team have been involved in were negative or failed</i>
9:20am-9:40am	Speaker Anthony Amato: <i>Why IBM studies may have Failed</i>
9:45am-10:05am	Speaker Jon Katz: <i>Trials in ALS</i>
10:10am-10:30am	Refreshments/Exhibitor Break
10:30am-11:40pm	Session II - Novel trial designs/ biomarker development Moderator: Tom Lloyd
10:30am-10:50am	Speaker Tim Miller: <i>Development of an antisense oligonucleotide therapeutic for SOD1 ALS</i>
10:55am-11:15am	Speaker Byron Gajewski: <i>Bayesian Adaptive Design</i>
11:20am-11:40am	Speaker Robert Bowser: <i>Biomarker development in ALS and use in clinical trials</i>

11:45am-1:00pm	Lunch
1:15pm-3:05pm	Session III – Particular challenges in complex intervention trials (AHP) Moderator: Gita Ramdharry
1:15pm-1:35pm	Speaker Kim Kimminau: <i>under-represented populations</i>
1:40pm-2:00pm	Speaker: Vanina Dal Bello: <i>exercise trials in ALS/MND</i>
2:05pm-2:25pm	Speaker Rob Singleton: <i>exercise in diabetic neuropathy</i>
2:30pm –2:50pm	Speaker Jeff Burns: <i>Challenges of Exercise and Diet Interventions</i>
2:55pm-3:10pm	Break
3:15pm-4:50pm	Session IV - New therapies and how we test them Moderator: Carolina
3:15pm-3:35pm	Speaker David Hermann: <i>new treatments in – HSN1/ ATTR</i>
3:40pm-4:00pm	Speaker Jeff Statland: <i>FSHD - network</i>
4:05pm-4:25pm	Speaker James Chip Howard: <i>about complement inhibition in MG</i>
4:30pm-4:50pm	Sally Dunaway: <i>PEDS Exercise in spinal muscular atrophy</i>
6:00pm-6:15pm	MSG Group Picture
6:15pm-7:30pm	Dinner
7:30pm –8:30pm	Robert C. Griggs Annual MSG Keynote Speaker - Richard Hughes <i>Clinical Trials in Rare Neuropathies: Rights, Wrongs and Remedies</i>
9:00pm-12:00	After hours

Day 3: Sunday, September 22, 2019

7:00am-8:00am	Breakfast
8:10 am-10:35am	Platform Presentations
8:10am-8:20am	Fellow Reza Seyedsadjadi: <i>Proximal nerve imaging in CMT</i>
8:25am-8:35am	Fellow Davut Pelivan: <i>Elucidating the genetic etiology of neuromuscular disorders using high-throughput sequencing approaches</i>
8:40am-8:50am	Platform Presenter

8:55am-9:05 am	Platform Presenter
9:10am-9:20am	Platform Presenter
9:25am-9:35am	Platform Presenter
9:40am-9:50am	Platform Presenter
9:55am-10:05am	Platform Presenter
10:10am-10:20am	Platform Presenter
10:25am-10:35am	Platform Presenter
10:40am-10:55am	Break
10:55am-12:30pm	Session V - Sponsor Updates
10:55am-11:15am	
11:20am-11:40am	
11:45am-12:05pm	
12:10pm-12:30pm	
12:30	Conclusion of meeting
12:30pm-1:30pm	Lunch