

# 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	<b>Buffet Breakfast and Check-In</b>
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	<b>Lunch</b> <i>Muscle Study Group Executive Committee Meeting</i>

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

## Day 2: Saturday, September 21, 2019

<b>7:30am-8:30am</b>	<b>Buffet Breakfast</b>
<b>8:30am-10:10am</b>	<b>Session I: Why trials fail- lessons learned</b> Moderator: Aisling Carr
<b>8:30am-8:50am</b>	Speaker Michael Shy: <i>what have we learned CMT trials (accepted)</i>
<b>8:55am-9:15am</b>	Speaker Richard Barohn: <i>Why trials Dr. Barohns' team have been involved in were negative or failed (accepted)</i>
<b>9:20am-9:40am</b>	Speaker Anthony Amato: <i>IBM Novartis (accepted)</i>
<b>9:45am-10:05am</b>	Speaker Jon Katz: <i>Trials in ALS (accepted)</i>
<b>10:10am-10:30am</b>	<b>Refreshments/Exhibitor Break</b>
<b>10:30am-12:20pm</b>	<b>Session II - Novel trial designs/ biomarker development</b> Moderator: Tom Lloyd
<b>10:30am-10:50am</b>	Speaker Harry Selker: <i>E2E</i>
<b>10:55am-11:55am</b>	Speaker Byron Gajewski: <i>bayesian adaptive design (accepted)</i>
<b>12:00pm-12:20pm</b>	Speaker Robert Bowser: <i>Phoenix – biomarkers development in ALS and Mitochondria (accepted)</i>
<b>12:30pm-1:30pm</b>	<b>Lunch</b>

<b>1:30pm-3:05pm</b>	<b>Session III – Particular challenges in complex intervention trials (AHP)</b> <b>Moderator:</b> Gita Ramdharry
<b>1:30pm-1:50pm</b>	Speaker Kim Kimminau: <i>under-represented populations</i> (accepted)
<b>1:55pm-2:15pm</b>	Speaker: Vanina Dal Bello: <i>exercise trials in ALS/MND</i> (accepted)
<b>2:20pm-2:40pm</b>	Speaker Rob Singleton: <i>exercise in diabetic neuropathy</i> (accepted)
<b>2:45pm – 3:05pm</b>	Speaker Jeff Burns: <i>exercise and dosing/diet</i> (accepted)
<b>3:10pm-3:25pm</b>	<b>Break</b>
<b>3:25pm-5:30pm</b>	<b>Session IV - New therapies and how we test them</b> Moderator: Carolina
<b>3:25pm-3:45pm</b>	Speaker David Hermann: <i>new treatments in – HSN1/ ATTR</i> (accepted)
<b>3:50pm-4:10pm</b>	Speaker Jeff Statland: <i>FSHD - network</i> (accepted)
<b>4:15pm-4:35pm</b>	Speaker Tim Miller: <i>ALS – results of ASO sod1 gene therapy trial</i>
<b>4:40pm-5:00pm</b>	Speaker James Chip Howard: <i>about complement inhibition in MG</i> (accepted)
<b>5:05pm-5:25pm</b>	Sally Dunaway: <i>PEDS</i> (accepted)
<b>6:15pm-7:30pm</b>	<b>Dinner</b>
<b>7:40pm – 8:40 pm</b>	<b>Robert C. Griggs Annual MSG Keynote Speaker - Richard Hughes</b> (accepted)
<b>9:00pm-12:00</b>	<b>After hours</b>

## **Day 3: Sunday, September 22, 2019**

<b>7:00am-8:00am</b>	<b>Breakfast</b>
<b>8:10 am-10:35am</b>	<b>Platform Presentations</b>
<b>8:10am-8:20am</b>	Platform Presenter - Fellow Reza Seyedsadjadi
<b>8:25am-8:35am</b>	Platform Presenter - Fellow Davut Pelivan
<b>8:40am-8:50am</b>	Platform Presenter
<b>8:55am-9:05 am</b>	Platform Presenter
<b>9:10am-9:20am</b>	Platform Presenter

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

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