

# 2020 Muscle Study Group Scientific Annual Meeting

## *New Disease-Modifying Treatments for Neuromuscular Disorders: Challenges and Opportunities*

September 25-27, 2020, Georgetown, DC

### Day 1: Friday, September 25, 2020

6:30am-8:00am	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	OPEN
9:20am-9:40am	Memantine <i>Richard J. Barohn, M.D.</i>
9:45am-10:05am	IBM International Genetics Consortium Study <i>Prof Michael G. Hanna, M.D.</i>
10:10am-10:30am	Electrical Impedance Myography in assessing muscle health in IBM <i>Bhaskar Roy, M.D.</i>
10:30am-10:45am	Break
10:50am-11:10am	3,4-DAP in Treatment of ALS <i>Raghav Govindarajan, MD</i>
11:15am-11:35am	Arimoclomol in IBM <i>Mazen Dimachkie, M.D.</i>
11:40am -12:00	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-2:30pm	Young Investigator Breakout session - Will Meurer

<b>1:00pm – 2:30pm</b>	<b>Clinical Evaluators and Study Coordinator breakout session – Kim Hart</b>
<b>2:30pm-3:00pm</b>	Break
<b>3:00pm – 5:30pm</b>	<b>Young investigators Shark Tank</b> <b>Panel: Vera Brill ~ Tahseen Mozzafar ~ others TBD</b>
<b>6:30pm-7:30pm</b>	Dinner
<b>7:30 pm - 9:00 pm</b>	Poster walk through session
<b>9:00 pm-Midnight</b>	Reception

## **Day 2: Saturday, September 26, 2020**

<b>7:30am-8:30am</b>	<b>Buffet Breakfast</b>
<b>7:30am-8:30am</b>	<b>Breakfast meeting for New Members and 1<sup>st</sup> time attendees</b> hosted by Dr. Barohn, Dr. Hanna, and Dr. Griggs
	<b>Session I - Implications of novel treatments for hereditary NM Disorders</b> Moderator: Chafic Karam
<b>8:30am-8:50am</b>	<b>Rick Chapman.</b> ICER. Health Economic Assessment for Spinraza and Zolgensma
<b>8:55am-9:20am</b>	<b>Tom Brannagan</b> - Challenges and opportunities of new TTR medications
<b>9:25am-9:45am</b>	<b>John Day</b> - Treatment of adult SMA in the context of new therapies
<b>9:50am-10:20am</b>	<b>Charlotte Sumner</b> - SMA affect neuron in mainly in development and suggests that treatment in adulthood is ineffective
<b>10:25am-10:40am</b>	<b>BREAK</b>
	<b>Session II –Challenges in the Implementation of new therapies in Neuromuscular disorders</b> Moderator:
<b>10:40 am-11:00am</b>	<b>Geoff Curran</b> - Implementation Science - possibility to expand on this subject, or allow for longer talk
<b>11:05am-11:25am</b>	<b>Philip Young</b> - Ethical and social implications of genetic screening for SMA

<b>11:30am-11:50am</b>	<b>Trevor Richter CADTH</b> - Health technology and health economic assessment for rare and ultra-rare diseases. The Canadian experience.
<b>11:55am-12:15pm</b>	<b>Betty Vertin</b> — View of patients and caregiver regarding new interventions (mother of three Duchenne boys)
<b>12:20pm-12:40 pm</b>	<b>Abi Fisher</b> , Associate Professor at UCL School of Behaviour Change - app based technologies for increasing physical activity
<b>12:45pm- 1:45 pm</b>	<b>Lunch</b>
<b>1:45pm-3:30pm</b>	<b>Session III – Challenges and opportunities of new interventions for NM diseases</b> <b>Moderator:</b> <i>Gita Ramdharry</i>
<b>1:45pm-2:05pm</b>	<b>Eric Lancaster</b> - Role of antibody testing in peripheral neuropathies
<b>2:05pm-2:25pm</b>	<b>Duaa Jabari</b> — Pilot study of Phenylbutyrate in ibm
<b>2:30pm-2:50pm</b>	<b>Vera Bril</b> - New Treatments for Myasthenia Gravis
<b>2:55pm –3:15pm</b>	<b>James Lilleker</b> — Biotherapeutics in myositis
<b>3:20pm-3:40pm</b>	<b>Gordon Smith</b> — Challenges in Neuropathy trials
<b>3:45pm-4:00pm</b>	<b>Break</b>
<b>4:00pm-5:30pm</b>	<b>Session IV – Platform Presentations</b> <b>Moderator:</b>
<b>4:00pm-4:10pm</b>	Fellow Reza Seyedsadjadi: <i>Proximal nerve imaging in CMT</i>
<b>4:15pm-4:25pm</b>	Fellow Paloma Gonzalez-Perez: <i>Molecular Biomarkers In Myotonic Muscular Dystrophy Type 2</i>
<b>4:25pm-4:35pm</b>	
<b>4:35pm-4:45pm</b>	
<b>4:45pm-4:55pm</b>	
<b>4:55pm-5:10pm</b>	
<b>5:10pm-5:20pm</b>	
<b>5:20pm-5:30pm</b>	
<b>6:30pm – 7:30pm</b>	<b>Dinner</b>

7:45pm - 8:45pm *Dr Robert C Griggs Keynote speaker -*  
**Dr Rabi Tawil, University of Rochester Medical Center**

9:00pm – 11:30pm **After hours reception**

## **Day 3: Sunday, September 27, 2020**

7:30am-8:30am **Breakfast**

8:30 am-9:50am **Session V:  
Moderator: Challenges and opportunities in Neuromuscular Diseases**

8:30am-8:50am **Jeffrey Statland**— Beta agonist tx for NM disorders

8:55am-9:15am **Tahseen Mozaffar** – Challenges and opportunities of enzyme replacement and gene therapy in Pompe’s disease.

9:20am-9:40am **Sabrina Paganoni**—ALS, new trial design and challenges

9:45am-10:05am **Megan Ianmarino** A novel approach to optimizing movement in treated children with spinal muscular atrophy

10:05am-10:25am **Brian Callaghan**—Value of extensive testing in peripheral neuropathies, in the context of new treatments

10:30am-10:45am **Break**

10:45am-12:30am **Session VI- Sponsor Updates**

10:45am-11:05am

11:05am-11:25am

11:25am-11:45am

11:45am-12:05pm

12:05pm-12:25pm

12:30pm-1:30pm **Lunch**