

# The 2017 Muscle Study Group Scientific Annual Meeting

"Neuromuscular Therapeutics: Bench to Bedside and Beyond"

September 23-25, 2017

Snowbird Summer and Ski Resort in Snowbird, Utah

Day 1: Saturday, September 23, 2017

Time	Session	Title Topic	Faculty	Room
7:00-8:00 am	Breakfast			Golden Cliff
7:30-8:00 am	Check-In			Ballroom Lobby
8:00- 12:00 am	Investigator Meetings:	Closed to Public		
8:00-9:00 am		<i>FSHD Clinical Trials Research Network PCORI</i>	Jeffrey Statland Richard Barohn	Magpie A Magpie B
9:00-10:00 am		<i>NIH sIBM Neutral History Study Memantine</i>	Tahseen Mozaffar Richard Barohn	Magpie A Magpie B
10:00-11:00 am		<i>IPANEMA IBM International Genetics Consortium Study</i>	Tahseen Mozaffar Pedro Machado and Michael Benatar	Magpie A Magpie B
10:00-10:30 am		<i>MGSCig</i>	Mazen Diamchkie	Magpie B
10:30-11:00 am		<i>Arimoclomol in IBM</i>	Mazen Dimachkie	Magpie A
11:00-12:00am		<i>FOR-DMD</i>	Robert Griggs	Magpie B
12:00-1:30 pm	Lunch	<i>Muscle Study Group Executive Committee Meeting</i>		
				Magpie A
1:30-5:00 pm	Young Investigator, Coordinator, Clinical Evaluator and Underrepresented NM Minority Workshop			Ballroom
	Invited Speakers:			
1:30-2:00 pm		<i>Advice to Young Investigators</i>	<b>Richard Barohn, MD</b> <i>University of Kansas Medical Center</i>	
2:00-2:15 pm		<i>NIH funding</i>	<b>Glen Nuckolls, PhD</b> <i>National Institute of Health</i>	
2:15-2:30 pm		<i>MDA Support Programs</i>	<b>Grace Pavlath, PhD</b> <i>Muscular Dystrophy Association</i>	
2:30-3:30 pm	Panel Discussion:	<i>Early Career Development: How to Get Started</i>		
		Moderators		
		<b>Jeffrey Statland,</b> <i>University of Kansas Medical Center</i>	<b>Jaya Trivedi,</b> <i>UT Southwestern Medical Center</i>	
		Panel Members		
		<b>Kevin Flanigan,</b> <i>Nationwide Childrens Hospital</i>	<b>Gordon Smith, MD</b> <i>University of Utah Health Care</i>	
		<b>Lindsay Alfano,</b> <i>Nationwide Childrens Hospital</i>	<b>Richard Barohn, MD</b> <i>University of Kansas Medical Center</i>	
		<b>Carlayne Jackson,</b> <i>UT Health San Antonio</i>	<b>Laura Herbelin</b> <i>University of Kansas Medical Center</i>	
3:30-3:50pm	Break			Ballroom Lobby
3:50-5:00 pm	Break Out Discussion:	<i>Session leaders will be those on the panel and 2017 planning committee members</i>		
5:00-6:00pm	10 Minute Update of MSG Studies			Ballroom
6:00-7:30 pm	Dinner			Golden Cliff
7:30 PM	Robert C. Griggs Annual MSG Keynote Speaker	<i>Gene Therapy Development</i>	<b>Brian Kasper, PhD</b> <i>Nationwide Childrens Hospital</i>	Ballroom
9:00 PM	Campfire & S'mores Evening Reception			Golden Cliff & Creekside Patio

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Day 2: Sunday, September 24, 2017

Time	Session	Faculty	Room
7:00-8:00 am	Breakfast		Golden Cliff
7:00-8:00 am	Poster Set-Up		Primrose
8:00-8:30 am	Welcome and State of the Muscle Study Group Address	Richard Barohn and Michael Hanna	Ballroom
8:30-10:10 am	Session I: Platforms for Developments of Novel Therapeutics		Ballroom
	Moderator	TBD	
8:30-8:55 am	<i>Cas9</i>	Rhonda Bassel-Duby, PhD UT Southwestern	
9:00-9:25 am	<i>Developed Processed Around High Throughput Screening</i>	Chris Gibson, MD, PhD Recursion Pharma	
9:30-9:55 am	<i>ALS Therapeutics</i>	Robert Baloh, MD, PhD Cedars- Sinai	
10:00-10:25 am	<i>Dup2 Mouse to Therapy</i>	Kevin Flannigan, MD Nationwide Childrens Hospital	
10:30-11:00 am	Break		Ballroom Lobby
11:00-1:00 pm	Session II: Data Mining in Neuromuscular Therapeutics		Ballroom
	Moderator	TBD	
11:00-11:45 am	<i>Natural History and MRI Outcome Measures in Global Multi-Center Dysferlinopathy Studies</i>	Jordi Diaz, MD Hospital de la Santa Creu i Sant Pau	
11:50-12:15 am	<i>Dysferlin Gene Therapy</i>	Louise Rodino-Klapac, PhD Nationwide Children's Hospital	
12:20-12:45 pm	<i>ALS Natural History Study</i>	Matt Harms, MD Columbia University Paul Romitti, PhD	
12:50-1:15 pm	<i>Overview of the MDStar New Project</i>	University of Iowa College of Public Health	
1:20-3:00 pm	Lunch & Networking		Golden Cliff
3:00-3:50 pm	Platform Presentation: Session I		Ballroom
	Moderator	TBD	
2:00-2:10 pm	<i>Outcome Measures in Limb Girdle Muscular Dystrophy</i>	Jennifer Mansour Kennedy Krieger Institute	
2:10-2:20 pm	<i>ITGA7 Mutations Cause Arrhythmia and Respiratory Muscle Weakness</i>	Enrico Bugiardi University College London	
2:20-2:30 pm	<i>Subcutaneous immunoglobulin for Maintenance Treatment in CIDP - A multi-Center, randomized, Double Blind Trail: The PATH Study</i>	Mazen Dimachkie, MD University of Kansas Medical Center	
2:30-2:40 pm	<i>Human motor Neurospheres Reveal Altered Neurofilament Distribution in CMT2E Neuro Axons</i>	Mario Saporta, MD University of Miami Health System	
2:40-2:50 pm	<i>Thromboembolic Risk in Inflammatory Neuromuscular Disease Patients on Long-Term IVIg</i>	Aisling Carr, PhD National Hospital for Neurology and Neurosurgery	
4:00-5:55 pm	Session III: Outcome Measures in Neuromuscular Disease		Ballroom
	Moderator	TBD	
3:00-3:25 pm	<i>Image DMD</i>	TBD	
3:30-3:55 pm	<i>Novel functional Outcome Measures</i>	Lindsay Alfano, PT Nationwide Childrens Hospital	
4:00-4:25 pm	<i>PROs</i>	Chad Heatwole, MD University of Rochester Medical Center	
4:30-4:55 pm	<i>MRI Biomarker Outcome Measures in Neuromuscular Diseases</i>	John Thornton, PhD University of College London, UK	
6:00-7:00 pm	Dinner		Golden Cliff
7:00-10:00	Walk Through Poster Session & Evening Reception		Primrose & Atrium Patio



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Day 3: Monday, September 25, 2017

Time	Session	Faculty	Room
7:00-8:00 am	Breakfast		Golden Cliff
		2017-2018 MSG Planning Committee Meeting	Magpie B
8:00-8:50 am	Platform Presentation: Session II		Ballroom
		Moderator	TBD
8:00-8:10 am	<i>Nusinersen for SMA1: The Italian Expanded Access Program (EAP) Experience</i>	Valeria Sansone, MD University of Milan	
8:10-8:20 am	<i>Clinical, Pathological, and Radiological Correlates in FSHD Patients: Update from the Seattle Wellstone Project</i>	Leo Wang, MD, PhD University of Washington Medicine	
8:20-8:30 am	<i>A Phenotypic Profile of Cognitive function in CMD</i>	Melissa Dixon, PhD University of Utah Health	
8:30-8:40 am	<i>Tongue Abnormalities on Brain MRI in Patients with Neuromuscular Disorders</i>	Chafic Karam, MD Oregon Health Science University	
8:40-8:50 am	<i>RCT of Bumetanide in Hypokalaemic Periodic Paralysis</i>	Renata Scalco, PhD University College London	
9:00-11:00 am	Session IV: Post Marketing Surveillance in Neuromuscular Disease		Ballroom
		Moderator	TBD
9:00-9:25 am	<i>From the MDX Mouse to an Approved Therapy</i>	Edward Kaye, MD Sarepta Therapeutics	
9:30-9:55 am	<i>Biogen (SMA)</i>	Sandy Reyna, MD Biogen	
10:00-10:25 am	<i>NeoGAA Trial</i>	Susan Sparks, MD, PhD Genzyme	
10:30-10:55 am	<i>Health Economics in Rare NM Diseases- Points to Consider</i>	Gorana Capkun-Niggli, PhD Novartis Pharmaceuticals	
11:00-12:30 pm	Lunch		Golden Cliff
12:30-2:00pm	Session V: Identification of Neuromuscular Disease		Ballroom
		Moderator	TBD
12:30-12:55 pm	<i>Newborn Screening in DMD</i>	Jennifer Kwon, MD University of Rochester Medical Center	
1:00-1:25 pm	<i>Presymptomatic Diagnosis and Therapy in SMA</i>	Eduardo Tizzano MD, PhD, Hospital Valle Hebronm Barcelona, Spain	
1:30-1:55 pm	<i>Genetic Testing in LGMD</i>	Matt Wicklund, MD Penn State Health	