

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
**Beaver Hollow Conference Center, Java Center, NY**  
**September 21-23, 2014**

### Final Agenda

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Saturday, September 20, 2014</u></b>			
<b>Arrival at Beaver Hollow for those attending ancillary workshops/meetings on Sunday</b>			
<b>7:00 – 8:00 pm</b>	<b>Dinner for those attending ancillary meetings on Sunday</b>		Mallard Room
<b><u>Day 1: Sunday, September 21, 2014</u></b>			
<b>7:00 – 8:00 am</b>	<b>Buffet Breakfast</b>		
<b>8:00 am – 5:00 pm</b>	<b>ANCILLARY MEETINGS/WORKSHOPS (MULTICENTER INVESTIGATOR GROUPS (OPEN TO ALL ATTENDEES; TRAINEES ENCOURAGED TO PARTICIPATE))</b>		
8:00 – 9:00 am	Methotrexate Ancillary Meeting	Richard Barohn, MD and Ms. Laura Herbelin	Maple Room
9:00 – 10:00 am	Rasagiline Ancillary Meeting	Richard Barohn, MD and Ms. Laura Herbelin	Maple Room
10:00 – 11:00 am	FOR-DMD Ancillary Meeting	Robert Griggs, MD and Katie Bushby, MD	The Aud
11:00 am – 12:00 pm	The International IBM Consortium Genetic Study Ancillary Meeting	Pedro Machado, MD and Michael Hanna, MD	The Aud
<b>12:00 – 1:30 pm</b>	<b>LUNCH FOR ANCILLARY STUDY PARTICIPANTS</b>		
12:00 – 1:30 pm	<b>MSG Executive Committee Meeting</b> Photos will be taken of Executive Committee Members		Drake Room
1:30 – 2:30 pm	NeuroNEXT: Rituxamab in MG Study	Richard Nowak, MD	Maple Room
<b>2:30 – 3:00 pm</b>	<b>Poster Session Set-Up</b>		Lodge
2:30 – 3:30 pm	Arimoclomol FDA Study	Mazen Dimachkie, MD	Maple Room
2:30 – 3:30 pm	<i>Investigating <u>P</u>ompe Prevalence in <u>NE</u>uromuscular <u>M</u>edicine <u>A</u>cademic Practices (IPANEMA Study)</i>	Tahseen Mozaffar, MD	Hemlock Room

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
**Beaver Hollow Conference Center, Java Center, NY**  
**September 21-23, 2014**

### Final Agenda

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 1: Sunday, September 21, 2014 (continued)</u></b>		
<b>3:30 – 4:40 pm</b>	<b>UPDATE ON CURRENT/PLANNED MSG TRIALS (ALL MSG MEETING ATTENDEES ENCOURAGED TO ATTEND)</b>	
3:30 – 3:40 pm	FOR – DMD Study	Robert Griggs, MD (University of Rochester)
3:40 – 3:50 pm	Arimoclomol IBM (Orphazyme)	Mazen Dimachkie, MD (Kansas University Medical Center)
3:50 – 4:00 pm	Methotrexate Study	Laura Herbelin/Richard Barohn, MD (Kansas University Medical Center)
4:00 – 4:10 pm	Rasagiline Study	Richard Barohn, MD (Kansas University Medical Center)
4:10 – 4:20 pm	International IBM Consortium	Michael Hanna, MD/Pedro Machado, MD University College London
4:20 – 4:30 pm	International Neuropathy Consortium	Michael Shy, MD University of Iowa
4:30 – 4:40 pm	<u>I</u> nvestigating <u>P</u> ompe Prevalence in <u>NE</u> uromuscular <u>M</u> edicine <u>A</u> cademic Practices (IPANEMA Study)	Tahseen Mozaffar, MD (University of California, Irvine)
4:40 – 5:20 PM	<b>Break</b>	
<b>5:20 – 6:00 PM</b>	<b>BRIEF PRESENTATIONS AND POSTER SESSION I</b> <b>Moderators: Ted Burns, MD (University of Virginia) and Gil Wolfe, MD (University at Buffalo)</b>	
5:20 – 5:30 PM	Complications of Pregnancy in Myotonic Dystrophy	Nicholas Johnson, MD (University of Utah)
5:30 – 5:40 PM	MRI Quantification of Fixed Myopathy in Hypokalaemic Periodic Paralysis Identifies Potential Therapeutic Window	Jasper Morrow, FRACP (University College London)
5:40 – 5:50 PM	Combined N-of-1 Trials Mexiletine vs. Placebo in Non-Dystrophic Myotonia using a Bayesian Approach	Bas Stunnenberg, MD (University Nijmegen Medical Center)

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
*Beaver Hollow Conference Center, Java Center, NY*  
**September 21-23, 2014**

### *Final Agenda*

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 1: Sunday, September 21, 2014 (continued)</u></b>		
<b>5:50 – 7:00 PM</b>	<b>POSTER SESSION WITH HORS D’OEUVRES &amp; CASH BAR</b>	Fireside/Lodge
<b>7:00 – 8:00 PM</b>	<b>DINNER</b>	Mallard/Lodge
<b>8:00 PM</b>	<b>Keynote Speaker W. King Engel, MD University of Southern California</b> Historical Perspectives on Evolution of Diagnosis and Management of Muscle Disorders During his Career	Fireside

<b><u>Day 2: Monday, September 22, 2014</u></b>		
<b>7:00 – 8:00 AM</b>	<b>BREAKFAST</b>	Mallard/Lodge
<b>7:00 – 8:00 AM</b>	<b>Informal Breakfast Seminar for New Members/Guests (open to all)</b>  Moderators: Richard Barohn and Robert C. Griggs	Drake Room
<b>8:00 – 8:10 AM</b>	<b>WELCOME/GENERAL SESSION</b>  Robert C. Griggs (University of Rochester), Mazen Dimachkie (Kansas University Medical Center), Jaya Trivedi (UT Southwestern) and Tahseen Mozaffar (UC Irvine)	Fireside Room
<b>8:10 – 11:30 AM</b>	<b>ECONOMIC CONSIDERATIONS IN THE DEVELOPMENT OF NEW TREATMENTS FOR NEUROMUSCULAR DISEASES</b> <b>SESSION I: Challenges in Developing and Running Clinical Trials in Rare Diseases</b>  Moderator: Karl Kiebertz, MD, MPH	
<b>8:10 – 8:40 AM</b>	Evidentiary Criteria for Accelerated Approval of New Medicines: Cataloging FDA’s Flexibility in Subpart H Approvals	Frank Sasinowski, MS, MPH, JD (Hyman, Phelps and McNamara)

## 2014 MSG Scientific Annual Meeting

### Economic Considerations in the Development of New Treatments for Neuromuscular Diseases Beaver Hollow Conference Center, Java Center, NY September 21-23, 2014

#### Final Agenda

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 2: Monday, September 22, 2014 (Continued)</u></b>		
8:40 – 9:10 AM	Patient-Reported Outcomes in Randomized Clinical Trials	Ira Shoulson, MD (Georgetown)
9:10 – 9:40 AM		
<b>9:40 – 10:00 AM</b>	<b>Break</b>	
10:00 – 10:30 AM	Funding New Investigators: the Role of the CTSA's	Karl Kieburtz, MD (University of Rochester)
10:30 – 11:00 AM	Panel Discussion	Mr. Sasinowski, Drs. Shoulson and Kieburtz
<b>11:00 – 11:30 AM</b>	<b>Break/Group Photo</b>	<b>Pool Area or The Aud/Lodge</b>
<b>11:30 – 12:30 PM</b>	<b>MINI-SYMPOSIUM ON POMPE DISEASE</b>	
11:30 AM – 12:00 PM	Advances in Therapeutics for Pompe Disease	Loren Pena, MD, PhD (Duke University)
12:00 – 12:30 PM	Perspectives on Gene Therapy for Pompe Disease	Barry Byrne, MD, PhD (University of Florida)
<b>12:30 – 1:30 PM</b>	<b>Lunch</b>	Mallard/Lodge
<b>1:30 – 2:30 PM</b>	<b>CLINICAL TRIAL DEVELOPMENT: DIFFICULTIES OF ENROLLING IN RARE DISEASES</b>	
1:30 – 2:00 PM	EPITOME Study in Ocular MG	Michael Benatar, MD, DPHIL (University of Miami)
2:00 – 2:30 PM	Burden of Illness in DMD	Kate Bushby, MD (Newcastle University)
<b>2:30 – 3:30 PM</b>	<b>UPDATES FROM THE NIH</b>	
2:30 – 2:50 PM	NeuroNext	Richard Barohn, MD (Kansas University Medical Center)
2:50 – 3:10 PM	NIAMS	Glen Nuckolls, PhD (NIH/NIAMS)
3:10 – 3:30 PM	NINDS	John Porter, PhD (NIH/NINDS)
<b>3:30 – 4:00 PM</b>	<b>Break</b>	

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
*Beaver Hollow Conference Center, Java Center, NY*  
**September 21-23, 2014**

### Final Agenda

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 2: Monday, September 22, 2014 (Continued)</u></b>		
<b>4:00 – 5:30 PM</b>	<b>CURRENT STATUS OF IMAGING AS BIOMARKER FOR NEUROMUSCULAR DISORDERS</b>	
4:00 – 4:20 PM	MRI in aiding clinical diagnosis of Muscle Diseases	Volker Straub, MD, PhD (Newcastle University)
4:20 – 5:00 PM	MRI in experimental muscle therapeutics	Tracey Willis, PhD (Newcastle University)
5:00 – 5:20 PM	Muscle Ultrasound in the Evaluation of Neuromuscular Diseases	Craig Zaidman, MD (Washington University)
5:20 – 5:35 PM	Panel Discussion	Drs. Straub, Willis and Zaidman
<b>5:35 – 6:00 PM</b>	<b>Break/Free Time/Informal Discussion</b>	
<b>6:00 – 7:30 PM</b>	<b>BRIEF PRESENTATIONS AND POSTER SESSION II</b> <b>Moderators: Ted Burns, MD (University of Virginia) and Gil Wolfe, MD (University at Buffalo)</b>	
6:00 – 6:10 PM	Distinct Autoantibodies Associate with Specific Histological Abnormalities in Dermatomyositis	Iago Pinal-Fernandez, MD (Johns Hopkins)
6:10 – 6:20 PM	Investigating Pathogenic B Cell Populations in MuSK Myasthenia Gravis	Richard Nowak, MD, MS (Yale University)
6:20 – 6:30 PM	Transcriptome Profiling Identifies Key Differences Between patients with Recessive or Dominant Negative Mutations in Collagen VI Related Myopathies	Russell Butterfield, MD (University of Utah)
<b>6:30 – 7:30 PM</b>	<b>POSTER SESSION WITH HORS D'OEUVRES &amp; CASH BAR</b>	Fireside/Lodge
<b>7:30 – 8:30 PM</b>	<b>DINNER</b>	
<b>8:30 PM</b>	<b>Keynote Speaker</b> <b>Valerie Askanas, MD, PhD</b> <b>University of Southern California</b> Overview of IBM advances and future directions	Fireside Room

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
**Beaver Hollow Conference Center, Java Center, NY**  
**September 21-23, 2014**

### Final Agenda

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 3: Tuesday, September 23, 2014</u></b>		
<b>7:00 – 9:00 AM</b>	<b>Checkout (Those staying at the Hampton Inn should bring their luggage to Beaver Hollow. Space will be available near the front desk for luggage storage.)</b>	
<b>7:00 – 8:00 AM</b>	<b>Buffet Breakfast</b>	Mallard/Lodge
<b>8:00 – 8:10 AM</b>	<b>MSG Status Report</b>	Robert Griggs, MD and Rabi Tawil, MD (University of Rochester)
	<b>Nominations</b>	Robert Griggs, MD (University of Rochester)
<b>8:10 – 11:30 AM</b>	<b>ECONOMIC CONSIDERATIONS IN THE DEVELOPMENT OF NEW TREATMENTS FOR NEUROMUSCULAR DISEASES</b> <b>SESSION II: Molecular Discoveries with Implications for the Development of Novel Therapies</b> Moderator: Charles Thornton, MD	
8:10 – 8:40 AM	Development of Novel Therapies in Musculoskeletal Diseases	Edward Kaye, MD (Sarepta Therapeutics)
8:40 – 9:10 AM	Challenges of Setting Up a Pivotal Clinical Trial in Muscle Diseases	Brian Tseng, MD, PhD (Novartis)
9:10 – 9:20 AM	Discussion	
<b>9:20 – 9:30 AM</b>	<b>Break</b>	
9:30 – 9:50 AM	Targeting Mitochondrial Oxidative Stress to Improve Aging Skeletal Muscle Function	David Marcinek, PhD (University of Washington)
<b>9:50 – 10:20 AM</b>	<b>LESSON LEARNED FROM THE C9ORF72 RELATED ALS</b>	
9:50 – 10:20 AM	Modeling the Disease in iPS Cells and the Lessons Learned	Robert H. Baloh, MD, PhD (Cedars Sinai)
<b>10:20 – 11:20 AM</b>	<b>Updates</b>	
10:20 – 10:40 AM	FSHD	Silvere van der Maarel, MD (Leiden University)
10:40 – 11:00 AM	Congenital Muscular Dystrophies: A Translational Update	Carsten Bönnemann, MD PhD (NIH/NINDS)
11:00 – 11:20 AM	Myofibrillar Myopathies	Ami Mankodi, MD (NIH/Neurogenetics Branch)
11:20 – 11:40 AM	HYP-HOP Study	Robert Griggs, MD (University of Rochester)

## 2014 MSG Scientific Annual Meeting

**Economic Considerations in the Development of New Treatments for Neuromuscular Diseases**  
*Beaver Hollow Conference Center, Java Center, NY*  
**September 21-23, 2014**

### ***Final Agenda***

*Please note: All General Sessions are in the Fireside Room in the Main Lodge. The Drake and Mallard Rooms are in the Main Lodge. The Chestnut, Hemlock, Maple Rooms and The Aud are located in the Conference Center. Poster Sessions 1 & 2 will be in the Main Lodge.*

<b><u>Day 3: Tuesday, September 23, 2014 (continued)</u></b>		
<b>11:40 AM – 12:00 Noon</b>	<b>Discussion</b>	<b>Drs. van der Maarel, Bönnemann and Mankodi</b>
<b>12:00 – 1:00 PM</b>	<b>Lunch and Departures</b>	<b>Mallard/Lodge</b>