

## 2011 MSG Scientific Annual Meeting

Translational research in Neuromuscular Diseases: Diverse Diseases, Convergent Themes  
*Beaver Hollow Conference Center, Java Center, NY*  
*September 19 – 21, 2011*

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 Forestview Room.

### TENTATIVE AGENDA – Updated 9/8/2011

#### Sunday, September 18, 2011

Arrival for those attending Ancillary Meetings on Monday

7:00 – 8:00 p.m.                      **Dinner**  
*Mallard Room*

#### Day 1: Monday, September 19, 2011

7:00 - 8:00 a.m.                      **Breakfast Buffet**  
*Mallard Room*

8:00 - 12:00 noon                    **ANCILLARY MEETINGS**

8:00 - 9:00 a.m.                      **Methotrexate Myasthenia Gravis Study Group**  
Chair: Richard Barohn  
*Maple Room*

10:00 a.m. - 12:00 p.m.            **EPITOME Study Group**  
Chair: Michael Benatar  
*Hemlock Room*

12:00 - 1:00 p.m.                    **Lunch**  
*Lakeside (weather permitting) or Mallard*

12:00 noon - 6:00 p.m.            **ANCILLARY MEETINGS**

12:00 - 1:00 p.m.                    **Arimocloamol Study Meeting with Lunch**  
Chair: Richard Barohn  
*Drake Room*

1:00 - 2:00 p.m.                      **HYP HOP Study Group**  
Chair: Robert Griggs  
*The Aud*

2:00 – 4:00 p.m.                      **CMT Study Meeting**  
Chair: Michael Shy  
*Chestnut Room*

3:15 - 6:00 p.m.                      **Free Time: Informal Discussion**

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#### **Day 1: Monday, September 19, 2011 - Continued**

- 4:00 - 6:00 p.m.**                    **MSG Executive Committee Meeting**  
**Photos will be taken of Executive Committee Members**  
*Hemlock Room*
- 5:30 - 6:00 p.m.**                    **Poster Session 1 Set-up**  
*Forestview Room*
- 6:00 - 7:30 p.m.**                    **Poster Session 1 with hors d'oeuvres & cash bar**  
*Forestview Room*
- 6:15 p.m.**                            **Poster Session Moderators: James Howard (University of North Carolina) and Carlayne Jackson (University of Texas – San Antonio)**
- 7:30 - 8:30 p.m.**                    **Dinner**  
*Mallard/Lodge*
- 8:30 -**                                 **Symposium Chair, John Kissel**  
**Keynote Speaker: Daniel Drachman (Johns Hopkins University)**  
**“Oscillation: From the Clinic to the Laboratory, and Back Again: My Dual Life”**  
*Fireside Room*

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### TENTATIVE AGENDA – Updated 9/8/2011

#### Day 2: Tuesday, September 20, 2011

- |                 |   |
|-----------------|---|
| 6:30 - 8:00 am  | <b>Breakfast Buffet</b><br><i>Mallard/Drake</i>   |
| 7:00 - 8:00 am  | <b>Informal Breakfast Seminar for new members/guests [open to all]</b><br><b>Overview/Update on Study Groups: Michael Hanna (British Myology Society), Richard Barohn (Muscle Study Group),</b><br><b>Moderator: Robert C. Griggs (University of Rochester)</b><br><i>Drake</i> |
| 8:00 - 8:10 am  | <b>Welcome</b><br><b>Robert C. Griggs (University of Rochester), Michael Hanna (University College London) and John Kissel (Ohio State University)</b><br><i>Fireside Room</i>  |
| 8:10 - 10:30 am | <b><u>Session I: RNA Metabolism as Therapeutic Target in Muscle Disease</u></b><br><b>Moderator: Charles Thornton (University of Rochester)</b>   |
| 8:10 - 8:30     | <b>RNA-based Therapy for Neuromuscular Disease</b><br><b>Mathew Wood (Oxford)</b>   |
| 8:30 – 8:50     | <b>Status of AVI Pre-Clinical and Clinical Research in DMD</b><br><b>Ryszard Kole (AVI Bio)</b>   |
| 8:50 – 9:10     | <b>RNA Therapy of the Muscular Dystrophies</b><br><b>Michela Guglieri (Newcastle University)</b>  |
| 9:10 – 9:30     | <b>RNA Approaches in Myotonic Dystrophy</b><br><b>Charles Thornton (University of Rochester)</b>  |
| 9:30 – 9:50     | <b>Discussion</b>   |
| 9:50 – 10:10    | <b>New MSG Strategies</b><br><b>Robert Griggs (University of Rochester)</b>   |
| 10:10 – 10:30   | <b>BREAK</b>  |

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#### Day 2: Tuesday, September 20, 2011 – Continued

10:30 - 12:00 noon	<b><u>Session II: RNA Metabolism as Therapeutic Target in Motor Neuron Disease</u></b> Moderator: To be determined	
10:30- 10:50	Antisense Approach to SMA Chris Lorson (University of Missouri – Columbia)	
10:50 - 11:10	RNA Mediated Approaches to ALS Stephen Kolb (Ohio State University)	
11:10 - 11:30	Preclinical and Clinical Studies of SBMA Kurt Fischbeck (NIH/NINDS)	
11:30 – 11:50	Neuromuscular Pipeline Jay Barth and Robert Spiegel (PTC)	
11:50 – 12:10	Discussion	
12:10	Group Photo	<i>Pool Area (weather permitting)/The Aud</i>
12:15 - 1:15 pm	Lunch	<i>Trailside or Mallard/Lodge</i>
1:15 – 1:30 pm	MSG Business Meeting Robert Griggs and Rabi Tawil	
1:30 - 3:05 pm	<b><u>Session III: Government/Foundation Perspectives on NM Themes</u></b> Moderator: John Kissel (Ohio State University) and Michael Hanna (University College London)	
1:30 - 1:50	Filling the Pipeline for New Clinical Investigators Valerie Cwik (Muscular Dystrophy Association)	
1:50 - 2:10	NIAMS Strategy Planning for Muscle Disease Research and Training Glen Nuckolls (NIH/NIAMS)	
2:10 – 2:30	Neuromuscular Research Activities and Funding Opportunities through NICHD Melissa Parisi (NIH/NICHD)	

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#### Day 2: Tuesday, September 20, 2011 - Continued

2:30 - 2:50	Round Table Discussion Electronic Telephone Diaries to Capture Patient Recorded Outcomes: Issues and Suggestions from CINCH and HYP HOP Nina Gorham (U of Texas Southwestern) Kim Hart (University of Rochester) Laura Herbelin (University of Kansas)
2:50 – 3:10	NEXT Initiative (Tentative) Elizabeth McNeil (NIH)
3:10 – 3:25	BREAK
3:25 - 4:35 pm	<u>Session IV: Animal Models and Translational Research</u> Moderator: Louis Ptacek (University of California San Francisco)
3:25 - 3:45	Animal Models in ALS – The Good, Bad, and Ugly Michael Benatar (Emory University)
3:45 – 4:05	Animal Models in SMA Arthur Burghes (Ohio State University)
4:05 - 4:25	Animal Models for the MDs Cathleen Lutz, PhD (The Jackson Laboratory)
4:25 - 4:45	Ascorbic Acid Trial in CMT1 David Herrmann (University of Rochester)
4:45 – 5:05	Open Discussion/Debate on Animal Models – Panel
5:05 - 6:00 pm	Free time for informal discussions/meetings/networking
5:30 – 6:00 pm	Poster Session 2 Set-up <i>Forestview Room</i>
6:00 – 7:30 pm	<u>Poster Session 2</u> with hors d'oeuvres & cash bar <i>Forestview Room</i>

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#### **Day 2: Tuesday, September 20, 2011 - Continued**

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|-----------------------|--|
| <b>6:15</b>           | <b>Poster Session Moderators: Susan Iannaccone and Gil Wolfe<br/>(University of Texas Southwestern)</b>  |
| <b>7:30 – 8:30 pm</b> | <b>Dinner</b><br><i>Mallard/Lodge</i>  |
| <b>8:30 -</b>         | <b>Symposium Chair: Michael Hanna</b><br><b>Keynote Speaker: Louis Ptacek (University of California San Francisco)</b><br><b>Phenotyping Animal Models of Neurological and Neuromuscular Diseases – Successes, Challenges and Pitfalls</b><br><i>Fireside Room</i> |

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#### Day 3: Wednesday, September 21, 2011

7:00 - 8:00 am	<b>Breakfast &amp; Checkout</b> <i>Mallard/Lounge</i>
7:00 - 8:00 am	<b>Informal Breakfast Workshop: To be determined</b> <i>Drake</i>
8:00 – 8:20 am	<b>MDA Update</b> <b>Valerie Cwik</b> Fireside Room
8:20 - 12:10 pm	<b><u>Session V: Exercise as a Therapy for NM Disease</u></b> <b>Moderator: Ronald Haller (Presbyterian Hospital – Dallas)</b>
8:20 - 8:40	<b>Overview of Exercise Physiology</b> <b>Richard Moxley (University of Rochester)</b>
8:40 – 9:00	<b>Applying Exercise Therapy in Peripheral Nerve Disease</b> <b>Gita Ramdharry (University College London)</b>
9:00 – 9:20	<b>Exercise in Mitochondrial Disease</b> <b>Ronald Haller (Presbyterian Hospital – Dallas)</b>
9:20 – 9:40	<b>Exercise in SBMA</b> <b>Kurt Fischbeck (NIH)</b>
9:40 – 10:00	<b>Exercise in ALS</b> <b>Julaine Florence (Washington University)</b>
10:00 – 10:20	<b>Discussion</b>
10:20 – 10:40	<b>Break and Check-out</b>
10:40 – 11:00 am	<b>Exercise in Glycogen Storage Disease</b> <b>Timothy Miller (Genzyme Corporation)</b>
11:00 – 11:20	<b>Future Directions in Exercise Therapy – What Do We Need to Know?</b> <b>John Vissing (University of Copenhagen)</b>
11:20 – 11:40	<b>Late Breaking News</b> <b>Rabi Tawil (University of Rochester)</b>

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#### **Day 3: Wednesday, September 21, 2011**

**12:00 noon**

**Lunch**

*Mallard/Lounge or outside location*