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 Forest View Room.

FINAL AGENDA

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 in 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 Arimoclomol Study Meeting with Lunch
Chair: Richard Barohn
Drake Room

1:00 - 2:00 HYP HOP Study Group
Chair: Robert Griggs
The Aud

2:00 – 4:00 CMT Study Meeting
Chair: Michael Shy
Chestnut Room

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

Day 1: Monday, September 19, 2011 - Continued

<table>
<thead>
<tr>
<th>Time</th>
<th>Event Description</th>
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| 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  
Forest View Room |
| 6:00 - 7:30 p.m. | Poster Session 1 with hors d’oeuvres & cash bar  
Forest View 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 |
Day 2: Tuesday, September 20, 2011

6:30 - 8:00 am  Breakfast Buffet  
Mallard/Drake

7:00 - 8:00 am  Informal Breakfast Seminar for new members/guests [open to all]  
Overview/Update on Study Groups: Michael Hanna (British Myology Society), Richard Barohn (Muscle Study Group), Moderator: Robert C. Griggs (University of Rochester)  
Drake

8:00 - 8:10 am  Welcome  
Robert C. Griggs (University of Rochester), Michael Hanna (University College London) and John Kissel (Ohio State University)  
Fireside Room

8:10 - 10:30 am  Session I: RNA Metabolism as Therapeutic Target in Muscle Disease  
Moderator: Charles Thornton (University of Rochester)

  8:10 - 8:30  RNA-based Therapy for Neuromuscular Disease  
Mathew Wood (Oxford)

  8:30 – 8:50  Status of AVI Pre-Clinical and Clinical Research in DMD  
Ryszard Kole (AVI Bio)

  8:50 – 9:10  RNA Therapy of the Muscular Dystrophies  
Michela Guglieri (Newcastle University)

  9:10 – 9:30  RNA Approaches in Myotonic Dystrophy  
Charles Thornton (University of Rochester)

  9:30 – 9:50  Discussion

  9:50 – 10:10  New MSG Strategies  
Robert Griggs (University of Rochester)

10:10 – 10:30  BREAK
## Day 2: Tuesday, September 20, 2011 – Continued

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<thead>
<tr>
<th>Time</th>
<th>Session II: RNA Metabolism as Therapeutic Target in Motor Neuron Disease</th>
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<tbody>
<tr>
<td>10:30-12:00</td>
<td>Moderator: Chris Lorson (University of Missouri – Columbia)</td>
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<tr>
<td>10:30-10:50</td>
<td>Antisense Approach to SMA</td>
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<td>Chris Lorson (University of Missouri – Columbia)</td>
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<tr>
<td>10:50-11:10</td>
<td>RNA Mediated Approaches to ALS</td>
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<td>Stephen Kolb (Ohio State University)</td>
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<tr>
<td>11:10-11:30</td>
<td>Preclinical and Clinical Studies of SBMA</td>
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<td>Kurt Fischbeck (NIH/NINDS)</td>
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<td>11:30-11:50</td>
<td>Neuromuscular Pipeline</td>
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<td>Jay Barth and Robert Spiegel (PTC)</td>
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<td>11:50-12:10</td>
<td>Discussion</td>
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<tr>
<td>12:10</td>
<td>Group Photo</td>
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<td>Pool Area (weather permitting)/The Aud</td>
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<thead>
<tr>
<th>12:15-1:15 pm</th>
<th>Lunch</th>
<th>Trailside or Mallard/Lodge</th>
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<tr>
<th>1:15 – 1:30 pm</th>
<th>MSG Business Meeting</th>
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<td>Robert Griggs and Rabi Tawil</td>
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<tr>
<th>1:30 - 2:50 pm</th>
<th>Session III: Government/Foundation Perspectives on NM Themes</th>
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<td>Moderator: John Kissel (Ohio State University) and Michael Hanna (University College London)</td>
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| 1:30 - 1:50    | NIAMS Strategy Planning for Muscle Disease Research and Training |
|                | Glen Nuckolls (NIH/NIAMS)                                      |

| 1:50 – 2:10    | Neuromuscular Research Activities and Funding Opportunities through NICHD |
|                | Melissa Parisi (NIH/NICHD)                                       |
2:10 - 2:30  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)

2:30 – 2:50  NEXT Initiative
Elizabeth McNeil (NIH)

2:50 – 3:10  BREAK

3:10 - 5:00 p.m.  Session IV: Animal Models and Translational Research
Moderator: Louis Ptacek (University of California San Francisco)

3:10 - 3:30  Animal Models in ALS – The Good, Bad, and Ugly
Michael Benatar (Emory University)

3:30 – 3:50  Animal Models in SMA
Arthur Burghes (Ohio State University)

3:50 - 4:10  Animal Models for the MDs
Cathleen Lutz, PhD (The Jackson Laboratory)

4:10 - 4:30  Ascorbic Acid Trial in CMT1
David Herrmann (University of Rochester)

4:30 – 5:00  Open Discussion/Debate on Animal Models – Panel

5:00 - 6:00 pm  Free time for informal discussions/meetings/networking

5:30 – 6:00 pm  Poster Session 2 Set-up
Forest View Room

6:00 – 7:30 pm  Poster Session 2 with hors d'oeuvres & cash bar
Forest View Room
Day 2: Tuesday, September 20, 2011 - Continued

6:15  Poster Session Moderators: Susan Iannaccone and Gil Wolfe (University of Texas Southwestern)

7:30 – 8:30 p.m.  Dinner  
Mallard/Lodge

8:30 -  
Symposium Chair: Michael Hanna  
Keynote Speaker: Louis Ptacek (University of California San Francisco)  
Phenotyping Animal Models of Neurological and Neuromuscular Diseases – Successes, Challenges and Pitfalls  
Fireside Room
### Final Agenda

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session V: Exercise as a Therapy for NM Disease</th>
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<tr>
<td>7:00 - 8:00 am</td>
<td>Breakfast &amp; Checkout</td>
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<tr>
<td>8:00 – 11:20 a.m.</td>
<td>Overview of Exercise Physiology</td>
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<td>8:00 - 8:20</td>
<td>Richard Moxley (University of Rochester)</td>
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<td>8:20 – 8:40</td>
<td>Applying Exercise Therapy in Peripheral Nerve Disease</td>
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<td>8:40 – 9:00</td>
<td>Gita Ramdharry (University College London)</td>
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<tr>
<td>9:00 – 9:20</td>
<td>Exercise in Mitochondrial Disease</td>
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<td>9:40 – 10:00</td>
<td>Discussion</td>
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<td>10:00 – 10:20 a.m.</td>
<td>Break and Check-out</td>
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<tr>
<td>10:20 – 10:40</td>
<td>Exercise in Glycogen Storage Disease</td>
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<td>10:40 – 11:00</td>
<td>Future Directions in Exercise Therapy – What Do We Need to Know?</td>
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<td>11:00 – 11:20</td>
<td>Late Breaking News</td>
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<tr>
<td>11:20</td>
<td>Lunch</td>
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*Mallard/Lounge or outside location*