

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 **Arimocloamol 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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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**
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

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FINAL AGENDA

Day 2: Tuesday, September 20, 2011

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| 6:30 - 8:00 am | Breakfast Buffet
<i>Mallard/Drake</i> |
| 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)
<i>Drake</i> |
| 8:00 - 8:10 am | Welcome
Robert C. Griggs (University of Rochester), Michael Hanna (University College London) and John Kissel (Ohio State University)
<i>Fireside Room</i> |
| 8:10 - 10:30 am | <u>Session I: RNA Metabolism as Therapeutic Target in Muscle Disease</u>
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 |

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10:30 - 12:00 noon	<u>Session II: RNA Metabolism as Therapeutic Target in Motor Neuron Disease</u> Moderator: Chris Lorson (University of Missouri – Columbia)
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 - 2:50 pm	<u>Session III: Government/Foundation Perspectives on NM Themes</u> Moderator: John Kissel (Ohio State University) and Michael Hanna (University College London)
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)

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Day 2: Tuesday, September 20, 2011 - Continued

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|-------------------------|---|
| 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. | <u>Session IV: Animal Models and Translational Research</u>
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
<i>Forest View Room</i> |
| 6:00 – 7:30 pm | <u>Poster Session 2</u> with hors d'oeuvres & cash bar
<i>Forest View Room</i> |

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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

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Day 3: Wednesday, September 21, 2011

7:00 - 8:00 a.m.	Breakfast & Checkout <i>Mallard/Lounge</i>
8:00 – 11:20 a.m.	<u>Session V: Exercise as a Therapy for NM Disease</u> Moderator: Ronald Haller (Presbyterian Hospital – Dallas)
8:00 - 8:20	Overview of Exercise Physiology Richard Moxley (University of Rochester)
8:20 – 8:40	Applying Exercise Therapy in Peripheral Nerve Disease Gita Ramdharry (University College London)
8:40 – 9:00	Exercise in Mitochondrial Disease Ronald Haller (Presbyterian Hospital – Dallas)
9:00 – 9:20	Exercise in SBMA Kurt Fischbeck (NIH)
9:20 – 9:40	Exercise in ALS Julaine Florence (Washington University)
9:40 – 10:00	Discussion
10:00 – 10:20 a.m.	Break and Check-out
10:20 – 10:40	Exercise in Glycogen Storage Disease Timothy Miller (Genzyme Corporation)
10:40 – 11:00	Future Directions in Exercise Therapy – What Do We Need to Know? John Vissing (University of Copenhagen)
11:00 – 11:20	Late Breaking News Rabi Tawil (University of Rochester)
11:20	Lunch <i>Mallard/Lounge or outside location</i>