

2010 MSG Scientific Annual Meeting

Treatment Strategies for Neuromuscular Diseases: The Challenge of Recruitment Beaver Hollow Conference Center, Java Center, NY September 20 – 22, 2010

Please note: All General Sessions are in L-1-in the Main Lodge. Drake and Mallard Rooms are in the Main Building. Board Rooms A, B & C and The Aud are located in the Conference Center. Poster Session 2 will be in Team Center – ELC.

AGENDA

Day 1: Monday, September 20, 2010

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|-------------------------|---|
| 7:00 - 8:00 a.m. | Breakfast Buffet
<i>Mallard Room</i> |
| 8:00 - 12:00 noon | ANCILLARY MEETINGS |
| 8:00 - 9:00 a.m. | Methotrexate Myasthenia Gravis Study Group
<i>Board Room C</i> |
| 9:00 - 10:00 a.m. | Mexiletine in Nondystrophic Myotonia Study Group
<i>Board Room C</i> |
| 10:00 a.m. - 12:00 p.m. | EPITOME Study Group
<i>Board Room B</i> |
| 12:00 noon - 6:00 p.m. | ANCILLARY MEETINGS |
| 12:00 - 1:00 p.m. | Lunch
<i>Lakeside (weather permitting) or Mallard</i> |
| 12:00 - 1:00 pm. | Arimoclomol Study Meeting with Lunch
<i>Board Room A</i> |
| 1:00 - 2:00 p.m. | CINCH Study Group
<i>The Aud</i> |
| 2:00 - 3:00 p.m. | CMT Study Group
<i>Board Room B</i> |
| 3:00 - 4:00 p.m. | HYP HOP Study Group
<i>The Aud</i> |
| 4:00 - 6:00 p.m. | Free Time: Informal Discussion |
| 4:00 - 6:00 p.m. | Cytokinetics Meeting (Michael Benatar)
<i>Board Room B</i> |
| 4:00 - 6:00 p.m. | MSG Executive Committee Meeting
Photos will be taken of Executive Committee Members
<i>Board Room C</i> |

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- 5:30 - 6:00 p.m.** **Poster Session 1 Set up**
Main Lodge
- 6:00 - 7:30 p.m.** **Poster Session 1 with hors d'oeuvres & cash bar**
Main Lodge
- 6:15 p.m.** **Poster Session Moderator: Robert T. Leshner (Children's National Medical Center)**
- 7:30 - 8:30 p.m.** **Dinner**
L-1
- 8:30 -** **Evening 1 Symposium: Chair, Kate Bushby (International Center for Life)**
Evening 1 Keynote Speaker: Robert Brown (University of Massachusetts)
Anti-Sense RNAi Study Results
L-1

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AGENDA

Day 2: Tuesday, September 21, 2010

- 6:30 - 8:00 a.m. **Breakfast Buffet**
Mallard/Drake
- 7:00 - 8:00 a.m. **Informal Breakfast Seminar for new members/guests [open to all]**
Overview/Update on Study Groups: Kate Bushby (TREAT-NMD),
Michael Hanna (British Myology Society), Richard Barohn
(Muscle Study Group), Robert Leshner (CINRG)
Moderator: Robert C. Griggs (University of Rochester)
Drake
- 8:00 - 8:10 a.m. **Welcome**
Robert C. Griggs (University of Rochester) and Anthony Amato
(Brigham and Women's Hospital)
L-1
- 8:10 - 10:30 a.m. **Session I: The Challenges of Patient Recruitment**
Moderator - Glen Nuckolls (NIAMS)
- 8:10 - 8:30 **Valerie Cwik (Muscular Dystrophy Association)**
Role of Foundations in Recruitment for Rare Neuromuscular
Diseases
- 8:30 - 8:50 **Bob Goldberg (The Myositis Association)**
Myositis Patient Registry
- 8:50 - 9:10 **Mike Hanna (University College London)**
European Perspective for Recruitment
- 9:10 - 9:30 **Discussion**
- 9:30 - 10:10 **Silvere van der Maarel (Leiden University)**
Molecular Pathophysiology of FSHD
- 10:10 - 10:30 a.m. **BREAK**
- 10:30 - 12:00 noon **Session II: Recruiting the Next Generation of NMD Investigators**
Moderator: - Robert Griggs
- 10:30 - 10:50 **Ahmet Hoke (Johns Hopkins)**
Young Neurologists into the Basic Laboratory

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- 10:50 - 11:10 **Mary Reilly (University College London)**
UK Recruiting Young Investigators into Neuromuscular Diseases
- 11:10 - 11:30 **Mike Shy (Wayne State University)**
Mentoring Clinical Fellows
- 11:30 - 12:00 **Discussion**
- 12:00 - 1:30 p.m. **Group Photo Lunch** *Pool Area (weather permitting)/The Aud
Trailside or Mallard/Lodge*
- 1:30 - 3:05 pm **Session III: Inflammatory Myopathies: Pathogenesis, Treatment, and Clinical Trials**
Moderator: Jerry Mendell
- 1:30 - 1:50 **Steve Greenberg (Brigham and Women's Hospital)**
Pathogenesis of Inflammatory Myopathies
- 1:50- -2:10 **Kian Salajegheh (Brigham and Women's Hospital)**
Inclusion Body Myositis
- 2:10 - 2:30 **Mazen Dimachkie (University of Kansas)**
Arimoclomol in IBM
- 2:30 - 2:50 **Discussion**
- 2:50 - 3:05 **BREAK**
- 3:05 - 4:45 p.m. **Session IV: Inflammatory Myopathies: Pathogenesis, Treatment, and Clinical Trials (Continued)**
Moderator: Larry Phillips (University of Virginia)
- 3:05 - 3:25 **Chester Oddis (University of Pittsburgh)**
IMACS History and Development
- 3:25 - 3:45 **Zafer Ozturk (MedImmune)**
MEDI545 Interferon and Upcoming Trials
- 3:45 - 4:05 **Anthony Amato (Brigham and Women's Hospital)**
Etanercept Study: Recruitment – Lessons Learned

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- 4:05 - 4:20** **Richard Cloud (Prothelia)**
Prothelia Therapeutics for Muscular Dystrophy
New Studies and Future Directions
- 4:20 - 4:35** **Timothy Miller (Genzyme)**
Lumizyme
- 4:35 - 6:00 p.m.** **Free time for informal discussions/meetings/networking**
- 5:00 – 6:00 p.m.** **SMA Natural History Study Meeting**
Board Room C
- 5:30 – 6:00 p.m.** **Poster Session 2 Set up**
Team Center - ELC
- 6:00 – 7:30 p.m.** **Poster Session 2 with hors d'oeuvres & cash bar**
Team Center - ELC
- 6:15** **Poster Session Moderator: Donald B. Sanders (Duke University)**
- 7:30 – 8:30 pm** **Dinner**
L-1
- 8:30 -** **Evening 2 Symposium: Chair, Anthony Amato (Brigham and Womens)**
Evening 2 Keynote Speaker: Jerry Mendell (Nationwide Childrens Hospital)
Gene Therapy of Muscular Dystrophies
L-1

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AGENDA

Day 3: Wednesday, September 22, 2010

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|-------------------|--|
| 7:00 - 8:00 am | Breakfast & checkout
Mallard/Lounge |
| 7:00 - 8:00 a.m. | Informal Breakfast Workshop: Challenges for Recruitment in Neuromuscular Diseases – Richard Barohn (University of Kansas) and Robert Griggs (University of Rochester)
Drake |
| 8:00 - 8:15 a.m. | Glen Nuckolls (NIAMS)
NIH Update
L-1 |
| 8:15 - 8:30 a.m. | Valerie Cwik (MDA)
MDA update |
| 8:30 - 12:30 p.m. | <u>Session V: Past, Current, and Future Protocols: Lessons Learned</u>
Moderator: Anthony Amato (Brigham and Women's) |
| 8:30 - 8:50 | Stuart Peltz (PTC Therapeutics, Inc.)
Ataluren (PTC 124) in DMD |
| 8:50 - 9:10 | Michael Benatar (Emory University)
EPITOME trial |
| 9:10 - 9:30 | Michael Shy (Wayne State University)
Vitamin C in CMT |
| 9:30 - 9:50 | Richard Barohn (University of Kansas)
Methotrexate in Myasthenia Gravis |
| 9:50 - 10:10 | Robert C. Griggs (University of Rochester)
HYP HOP |
| 10:10 - 10:30 | Discussion |
| 10:30 - 11:00 | Break and Check-out |
| 11:00 - 11:20 | Kate Bushby (International Center for Life) and
Robert C. Griggs (University of Rochester)
FOR-DMD Steroid Trial |

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11:20 - 11:40	Michael Hanna (University College of London) Update from British Myology Society
11:40 - 12:00	Late Breaking News
12:00 - 12:30	MSG Update: Robert Griggs and Rabi Tawil New Protocols/New Ideas: Workshop Robert Griggs, Anthony Amato and Richard Barohn
12:30 - 12:45	Cytokinetics Study Michael Benatar, Jacqueline Lee and Andrew Wolfe
12:45 - 1:00	Discussion
1:00 - 1:45 p.m.	Lunch Mallard/Lounge or outside location