2013 MSG Scientific Annual Meeting

Novel Molecular Mechanisms of Neuromuscular Disease: Implications for Therapy
Lady Margaret Hall, Oxford, UK
September 16 – 18, 2013

Please note: All General Sessions are in the Simpkins Lee Theatre. Ancillary Meetings will be held in the Old Library or Mary O’Brien Rooms. Poster session presentations will be in the Simpkins Lee Theatre. Posters will be displayed in the Monson Room.

FINAL AGENDA

Sunday, September 15, 2013

Arrival at Lady Margaret Hall for those attending ancillary workshops/meetings on Monday

7:00 – 8:00 p.m. Dinner for Guests Attending Ancillary Meetings on Monday
Deneke Dining Hall

Day 1: Monday, September 16, 2013

7:30 – 8:00 AM Buffet Breakfast
Deneke Dining Hall

8:00 AM – 5:00 PM Ancillary Meetings/Workshops (Multicenter investigator groups [open to all attendees; Trainees encouraged to participate])

8:00 – 9:00 Methotrexate Ancillary Meeting (Richard Barohn and Laura Herbelin)
Old Library

9:00 – 10:00 Rasagiline Ancillary Meeting (Richard Barohn and Laura Herbelin)
Old Library

10:00 – 11:00 Sarepta Meeting (Richard Barohn and Laura Herbelin)
Jerwood Room

11:00 – 12:00 The International IBM Consortium Genetic Study Ancillary Meeting (Pedro Machado and Michael Hanna)
Old Library

12:00 – 1:30 Lunch for Ancillary Study Participants
Deneke Dining Hall

MSG Executive Committee Meeting over Lunch
Photos will be taken of Executive Committee Members
Jerwood Room

1:30 – 2:30 CINCH Ancillary Meeting (Richard Barohn, Robert Griggs, Michael Hanna)
Old Library

2:30 – 3:00 Poster Session Set-Up
Monson Room
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Day 1: Monday, September 16, 2013 (Continued)

2:30 – 3:30  Orphazyme Ancillary Meeting (Richard Barohn and Laura Herbelin)
Old Library

3:30 – 5:00 PM
Update on Current/Planned MSG Trials (all MSG meeting attendees
couraged to attend)
Simpkins Lecture Hall

3:30 – 3:40  FOR – DMD Study (Griggs)

3:40 – 3:50  Arimoclomol IBM (Orphazyme) Study (Dimachkie)

3:50 – 4:00  Methotrexate Study (Barohn)

4:00 – 4:10  Rasagiline Study (Barohn)

4:10 – 4:20  International IBM Consortium (Hanna/Macado)

4:20 – 4:30  Catching CROMFiSH: Preparing the Way for Therapeutic Trials in
Facioscapulomuscular Dystrophy (Heatwole)

4:30 – 4:40  Epitome (Benetar)

4:40 – 4:50  Inherited Neuropathy Consortium (Shy)

4:50 – 5:00  HYP HOP (Griggs)

5:00 – 6:30 PM
Brief Presentations and Poster Session I
Moderators: Mazen Dimachkie (University of Kansas) and Carlayne Jackson
(University of Texas Health Science Center)
Simpkins Lecture Hall

5:00 – 5:10  Presentation I - Validation of the MRC MRI Diagnostic Score for Inclusion
Body Myositis
Jasper Morrow (University College London)

5:10 – 5:20  Presentation II - A Preliminary Score Tool for the Evaluation of Muscle
Biopsies in Patients with Inclusion Body Myositis – Application in a
Randomised Placebo-Controlled Clinical Trial
Stefen Brady (University College London)
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Day 1: Monday, September 16, 2013 (Continued)

5:20 – 5:30  Presentation III - Cytosolic 5’-Nucleotidase 1A Autoimmunity in Sporadic Inclusion Body Myositis
Steven Greenberg (Harvard Medical Center)

5:30 – 5:40  Presentation IV - Treatment of Sporadic Inclusion Body Myositis (sIBM) with an Anti-Activin Receptor II Antibody
Steven Greenberg (Harvard Medical Center)

5:35 – 6:30 PM  Poster Session with hors d’oeuvres & cash bar
Monson Room

6:30 – 7:00 PM  Travel to Magdalen College via Motor Coach

7:30 - 8:30 PM  Dinner
Magdalen College - Hall

8:30 PM  Keynote Speaker:
David Hilton-Jones (Oxford)
NMD: A Historical Perspective

9:00 PM  Travel to Lady Margaret Hall via Motor Coach

Day 2: Tuesday, September 17, 2013

7:30 – 8:00 AM  Buffet Breakfast for New Members/Guests [open to all]
Jerwood Room
Moderators: Richard Barohn (University of Kansas), Robert Griggs (University of Rochester)

7:30 – 8:00 AM  Breakfast
Deneke Dining Room

8:00 – 8:10 AM  Welcome/General Session
Simpkins Lee Theatre
Robert C. Griggs (University of Rochester), Mary Reilly (University College London) and Gil Wolfe (University at Buffalo)
Day 2: Tuesday, September 17, 2013 (Continued)

8:10 AM – 8:30 PM  Novel Molecular Mechanisms: Implications for Therapy Development
                 Session I: Hereditary Myopathies
                 Moderators: Michael Hanna (University College London) and Kate Bushby (Newcastle University)

8:10 – 8:50  Calpainopathy: Human Gene Therapies
             William Lostal (Genethon)

8:50 – 9:30  Molecular Therapies for Neuromuscular Diseases
             Jerry Mendell (Nationwide Children’s)

9:30 – 10:00  OPMD
              Bernard Brais (University of Montreal)

10:00 – 10:20  Discussion

10:20 – 10:50 AM  Break

10:50 – 11:30  FSHD
               Rabi Tawil (University of Rochester)

11:30 – 12:10  Dystrophinopathies
               Francesco Muntoni (University College London)

12:10 – 12:50  Myotonic Dystrophy
               Charles Thornton (University of Rochester)

12:50 – 1:10  Discussion

1:10 – 2:10 PM  Lunch
                 Deneke Dining Hall

1:10 – 2:10  FOR – DMD Ancillary Meeting (Robert Griggs and Kate Bushby)
              Jerwood Room

2:10 – 5:30 PM  Overcoming Hurdles of Developing and Performing Clinical Trials – Panel Format
                 Moderators: Michael McDermott (University of Rochester) and Gil Wolfe (University of Buffalo)
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FINAL AGENDA

Day 2: Tuesday, September 17, 2013 (Continued)

2:10 – 2:30
Neuro NEXT and NMD: Update
Petra Kaufmann (NINDS)

2:30 – 2:50
What is ‘Adequate Rationale’ to Initiate a Trial? [Spoiler Alert: No Holy Grail Here, Only Equipoise—Good, Bias—Bad]
John Porter (NINDS)

2:50 – 3:00 PM
Break

3:00 – 4:00
Clinical Trial #1: Translating Bumetanide to the PP Clinical Trial Theatre
Presenters: Michael Hanna (University of College London) and Jeff Statland (University of Rochester)
Discussants: Jaya Trivedi (University of Texas Southwestern), Richard Barohn (University of Kansas), Michael McDermott (University of Rochester) (University College London), Gil I. Wolfe (University of Buffalo)

Clinical Trial #2: Clinical Trials in CMT: The Past and Present and Directions for the Future
Presenter: Michael Shy (University of Iowa)
Discussants: Steven Scherer (University of Pennsylvania), Mary Reilly (University College London) Michael McDermott (University of Rochester), Gil Wolfe (University of Buffalo) and Scott Evans (Harvard)

4:00 – 5:00
Challenges of Conducting International Multicenter Clinical Trials: The FOR-DMD Study: A Case Study
Michela Guglieri, Kate Bushby and Robert Griggs

5:00 – 5:15
Discussion

5:15 – 6:00
Break/Free Time/Informal Discussion

6:00 – 7:30 PM
Brief Presentations and Poster Session II
Moderators: Mazen Dimachkie (University of Kansas) and Carlayne Jackson (University of Texas Health Science Center)

6:00 – 6:10
Presentation I – Potential Mechanisms in Musk-Myasthenia Gravis
Inga Koneczny (Oxford)
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Day 2: Tuesday, September 17, 2013 (Continued)

6:10 – 6:20  Presentation II – Characterization of CD4 and CD8 T-Cell Responses in MuSK Myasthenia Gravis
Donald Sanders (Duke University)

6:20 – 6:30  Presentation III – Results of REVERSE-DBMD: Revatio for Heart Disease in Duchenne and Becker Muscular Dystrophy
Kathryn Wagner (Kennedy Krieger)

6:30 – 7:30 PM  Poster Session with hors d’oeuvres & cash bar
Monson Room

7:30 – 8:30 PM  Dinner
Deneke Dining Hall

8:30 PM  Keynote Speaker:
Doug Turnbull (Newcastle University)
Mitochondrial Cytopathies
Simpkins Lee Theatre

Day 3: Wednesday, September 18, 2013

7:00 – 9:00 AM  Checkout in Porter’s Lodge (space will be available for luggage storage)

7:30 – 8:00 AM  Breakfast
Deneke Dining Hall

8:00 - 8:10 AM  MSG Status Report – Robert Griggs (University of Rochester)
Nominations – Carlayne Jackson (University of Texas Health Science Center)
Simpkins Lee Theatre

8:10 AM – 6:00 PM  Novel Molecular Mechanisms: Implications for Therapy Development
Session II: Hereditary Neuropathies
Moderators: Mary Reilly (University College London) and Steve Scherer (University of Pennsylvania)

8:10 – 8:40  Peripheral Demyelination
M. Laura Feltri (University of Buffalo)

8:40 – 9:20  Hereditary Axonopathies
Mary Reilly (University College London)
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### FINAL AGENDA

**Day 3: Wednesday, September 18, 2013 (Continued)**

<table>
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<tr>
<th>Time</th>
<th>Event</th>
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| 9:20 – 10:00 | **Connexin 32 Neuropathies**  
Steve Scherer (University of Pennsylvania) |
| 10:00 – 10:20| Discussion                                                           |
| 10:20 – 10:50| Break                                                               |
| 10:50 – 11:20| **Motor Neuron Disease**  
Kevin Talbot (Oxford) |
| 11:20 – 11:50| **Neuropathies:Channelopathies**  
*David Bennett (Oxford)* |
| 11:50 – 12:20| Discussion                                                           |
| 12:20 – 12:40 PM | **Group Photo**  
Simpkins Lee Theatre or Outside Courtyard |
| 12:40 – 1:40 PM | **Lunch**  
Deneke Dining Hall |
| 1:40 – 4:00 PM | **Disorders of the NMJ: The View from Oxford**  
Moderator: *Angela Vincent* |
| 1:40 – 2:10  | **Molecular Background to CMS**  
David Beeson (Oxford) |
| 2:10 – 2:40  | **CMS: Clinical Perspectives**  
Jacqueline Palace (Oxford) |
| 2:40 – 3:00  | Break                                                               |
| 3:00 – 3:40  | **Immune-Mediated Mechanisms**  
Angela Vincent (Oxford) |
| 3:40 – 4:00  | Discussion                                                           |
| 4:00 – 4:40  | Free Time                                                            |
| 4:40 – 6:40 PM | **Optional Walking Tour of Oxford – Meet at the Porter’s Lodge** |
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FINAL AGENDA

Day 3: Wednesday, September 18, 2013 (Continued)

6:40 PM Dinner on own

Day 4: Thursday, September 19, 2013

7:00 – 8:00 AM Breakfast

7:00 – 9:00 AM Checkout and Departures