

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)

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| 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 encouraged 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 Consortitium (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 | <u>Brief Presentations and Poster Session I</u>
Moderators: Mazen Dimachkie (University of Kansas) and Carlyne Jackson (University of Texas Health Science Center)
Simpkins Lecture Hall |
| 5:00 – 5:10 | <u>Presentation I</u> - Validation of the MRC MRI Diagnostic Score for Inclusion Body Myositis
Jasper Morrow (University College London) |
| 5:10 – 5:20 | <u>Presentation II</u> - 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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- 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)

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

8:10 AM – 8:30 PM	<u>Novel Molecular Mechanisms: Implications for Therapy Development</u> <u>Session I: Hereditary Myopathies</u> 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 <i>Deneke Dining Hall</i>
1:10 – 2:10	FOR – DMD Ancillary Meeting (Robert Griggs and Kate Bushby) Jerwood Room
2:10 – 5:30 PM	<u>Overcoming Hurdles of Developing and Performing Clinical Trials – Panel Format</u> Moderators: Michael McDermott (University of Rochester) and Gil Wolfe (University of Buffalo)

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- 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 Carlyne 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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- 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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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 <i>David Bennett (Oxford)</i>
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	<u>Disorders of the NMJ: The View from Oxford</u> Moderator: <i>Angela Vincent</i>
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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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**