



**Update in Neuromuscular Disorders  
Monday 17<sup>th</sup> - Thursday 20<sup>th</sup> March 2014**

***Clinical Neuroscience Lecture Theatre at 33 Queen Square, London WC1N 3BG***

***PROGRAMME***

**Day 1 – Monday 17 March**

**10:00 – 10:30 Registration and coffee**

**Morning Muscular dystrophies**

**10:30 – 10:45 DMD: Evolving natural history**

Dr Adnan Manzur, ICH Dubowitz Neuromuscular Centre

**10:45- 11:10 Experimental Therapies in DMD: where are we?**

Professor Francesco Muntoni, ICH Dubowitz Neuromuscular Centre

**11:10 – 11:30 Psychological aspects of participating in clinical trials**

Professor Elena Garralda, Child and Adolescent Psychiatry, Imperial College

**11:30 – 11:50 COFFEE**

**11:50 – 12:30 Limb girdle muscular dystrophies: old and new**

Dr Anna Sarkozy, University of Newcastle

**12 30 – 12 45 A study of families' perspective on participation in research trials**

Naomi Antcliff and Dr Valeria Ricotti

Clinical Trials Unit, Great Ormond Street Hospital & ICH Dubowitz Neuromuscular Centre.

**12:45 – 13:00 Illustrative cases**

Dr Adeline Seow, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital

**13:00 – 14:00 LUNCH**

**Afternoon Cardiac rhythm disorders in NMD**

**14:00 – 14:30 DMD: Cardiomyopathy and cardiac arrhythmia – (Brief: focus on arrhythmia risk and implications especially in young men/ evolving natural history)**

Professor Perry Elliott, Heart Hospital, London

- 14:30 – 15:00 Cardiac Rhythm disorders in NMD - (briefing: EDMD; Myotonic dystrophy; myofibrillar myopathies)**  
Juan Kaski, Great Ormond Street Hospital
- 15:00 – 15:30 Case presentations**  
Dr Adeline Seow, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- 15:30 – 16:00 TEA**
- 16:00 – 17.00 Special Guest speaker**  
**Therapeutic approaches to childhood mitochondrial diseases**  
Professor Rita Horvath, Newcastle University International Centre for Life
- 17:00 – 18:30 Drinks reception and canapies in the lecture theatre foyer**

**Day 2 – Tuesday 18<sup>th</sup> March**

**AM: Childhood Neurogenic Disorders**

- 09:00 – 09:25 Spinal muscular atrophy**  
Prof Victor Dubowitz, Emeritus Professor of Paediatrics, ICH Dubowitz Neuromuscular Centre
- 09:25 – 09:50 Outcome measures in SMA**  
Professor Eugenio Mercuri, Catholic University, Rome
- 09:50 – 10:15 Illustrative cases**  
Dr Luigi D'Argenzio. Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- 10:15 – 10:45 COFFEE**
- 10:45 – 11:30 CIDP in children: age-specific aspects, and what can be learned from adults**  
Professor Rudolf Korinthenberg, Children's Hospital, University Hospital Freiburg
- 11:30 – 12:00 Illustrative cases**  
Dr Anna Schugal, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- 12:00 – 12:30 A treatable neuronopathy: Brown Vialetto Von Laere Syndrome**  
Dr Shamima Rahman, Institute of Child Health
- 12:30 - 13.00 Illustrative cases**  
Dr Anna Schugal, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- 13:00 – 14:00 LUNCH**
- Afternoon Congenital myopathies and their differential diagnosis**
- 14:00 – 14:30 Overview on congenital myopathies**  
Professor Francesco Muntoni, ICH Dubowitz Neuromuscular Centre
- 14:30 – 15:00 Vacuolar myopathies and other neuromuscular disorders associated with defective autophagy**  
Dr Heinz Jungbluth, Childrens Neuroscience Centre, St Thomas' Hospital
- 15:00 – 15:30 Illustrative cases**  
Dr Maria Sframeli, ICH, Great Ormond Street Hospital
- 15:30 – 15:50 TEA**
- 15:50 – 16.30 Myofibrillar Myopathies**  
Prof Hanns Lochmüller, University of Newcastle
- 16.30 – 17:00 Late onset Glycogen Storage diseases: Differential diagnosis and therapeutic perspectives**  
Tracey Willis, Robert Jones and Agnes Hunt Orthopaedic Hospital

**Day 3 – Wednesday 19 March**

- 09:00 – 09:45**      **Distal inherited motor neuropathies**  
Professor Mary Reilly, MRC Centre for Neuromuscular Diseases
- 09:45 – 10:15**      **Illustrative cases**  
Dr Alex Rossor, Kings College London
- 10:15 – 11:00**      **CIDP update on diagnosis and pathogenesis**  
Dr Mike Lunn, MRC Centre for Neuromuscular Diseases
- 11:00 – 11:30**      **COFFEE**
- 11:30 – 12:15**      **Practical approach to diagnosing and treating metabolic myopathies**  
Dr Mark Roberts, Neurosciences, Salford
- 12:15 – 13:00**      **Metabolic muscle disease in adults**  
Dr Ros Quinlivan, MRC Centre for Neuromuscular Diseases
- 13:00 – 14:00**      **LUNCH**
- 14:00 – 14:45**      **Fatty acid oxidation disorders – making the diagnosis and options for treatment**  
Dr Elaine Murphy, National Hospital for Neurology and Neurosurgery
- 14:45 - 15:15**      **Familial amyloid polyneuropathies**  
Professor Mary Reilly, MRC Centre for Neuromuscular Diseases
- 15:15 – 15:30**      **TEA**
- 15:30 – 16:15**      **MRI in adult neuromuscular disease**  
Dr Jasper Morrow, MRC Centre for Neuromuscular Diseases
- 16:15 – 17.00**      **Current approach to genetic diagnosis of inherited neuromuscular diseases**  
Professor Henry Houlden, UCL Institute of Neurology

## **Day 4 – Thursday 20 March**

(AM summary of CPC at 15.20 given to delegates. Votes due in by lunchtime)

<b>09:00 – 09:30</b>	<b>Muscle channelopathies 2014</b> Mike Hanna, MRC Centre for Neuromuscular Diseases
<b>09:30 – 10:15</b>	<b>Diagnosis and management of myotonic dystrophies</b> Chris Turner, MRC Centre for Neuromuscular Diseases
<b>10:15 – 11:00</b>	<b>Mitochondrial myopathies- diagnosis and management</b> Dr Rob Pitceathly, King's
<b>11:00 – 11:15</b>	<b>COFFEE</b>
<b>11:15 – 12:00</b>	<b>Acquired Myasthenia Gravis</b> Professor Dimitri Kullman, UCL Institute of Neurology
<b>12:00 - 12:45</b>	<b>Congenital Myasthenia Gravis Syndrome</b> Professor David Beeson, Weatherall Institute of Molecular Medicine, Oxford
<b>12:45 - 13:30</b>	<b>LUNCH</b>
<b>13:30 – 14:00</b>	<b>POEMs Syndrome</b> Dr Mike Lunn, MRC Centre for Neuromuscular Diseases
<b>14:00 – 14:30</b>	<b>Mononeuropathies</b> Dr Hadi Manji, MRC Centre for Neuromuscular Diseases
<b>14:30 – 15:00</b>	<b>Diagnosis and management of inflammatory myopathies</b> Dr David Hilton-Jones, John Radcliffe Hospital, Oxford
<b>15:00 – 15:20</b>	<b>TEA</b>
<b>15:20 – 15:50</b>	<b>CPC discussion</b> Dr Carolyn Gabriel, Imperial College Healthcare NHS Trust
<b>15:50 – 16:20</b>	<b>CPC conclusion</b> Professor Mary Reilly, MRC Centre for Neuromuscular Diseases
<b>16:20</b>	<b>Close of course</b>