Update in Neuromuscular Disorders  
Tuesday 5- Friday 8 May 2015  
Clinical Neuroscience Lecture Theatre at 33 Queen Square, London WC1N 3BG

PROGRAMME

Day 1 – Tuesday 5 May

10:00 – 10:30  Registration* and coffee

*For the CPC discussion on Wednesday 6 May at 15.45, please collect the CPC information sheet at the Registration desk. The deadline for responses is at 13.00 on Wednesday 8 May. There is a prize for the winner.

Theme: Advances in Duchenne muscular dystrophy

10:30 – 11:00  Updates from UK NorthStar clinical network: Outcomes of corticosteroid initiation in very young boys with Duchenne muscular dystrophy & effect of dystrophin genotype on disease course.  
Dr Valeria Ricotti, ICH Dubowitz Neuromuscular Centre

11:00 – 11:15  Clinical outcomes in a large cohort of boys and adolescents with Duchenne muscular dystrophy  
Dr Adnan Manzur, ICH Dubowitz Neuromuscular Centre

11:15 – 12:00  Experimental therapies in Duchenne muscular dystrophy  
Prof Francesco Muntoni, ICH Dubowitz Neuromuscular Centre

12:00 – 12:15  COFFEE

Theme  Early onset LGMDs

12.15 – 12.45  Limb girdle muscular dystrophies of early onset  
Dr Anna Sarkozy, ICH Dubowitz Neuromuscular Centre
12:45 – 13.15 Illustrative case presentations

13.15 – 14.00 LUNCH

Theme

Spinal muscular atrophy type I (SMA1) workshop

14:00 – 14:10 SMA1 outcomes with contemporary care
Dr Adnan Manzur, ICH Dubowitz Neuromuscular Centre

14:10 – 14:20 NIV in SMA1.
Dr François Abel, Great Ormond Street Hospital

14:20 - 14:30 Gastrostomy in SMA 1- The surgeon’s perspective
Mr Joe Curry, Great Ormond Street Hospital

14 30 – 14 40 SMA1. Palliative care approach
Dr Finella Craig, Great Ormond Street Hospital

14 40 – 15 15 Panel discussion. Led by Dr Adnan Manzur, ICH Dubowitz Neuromuscular Centre

15:15 – 15:45 TEA

15:45– 16:30 Translational research in spinal muscular atrophy
Prof Francesco Muntoni, ICH Dubowitz Neuromuscular Centre

16:30 – 17.30 Special Guest Speaker,
Investigating the relationship between skeletal muscle mass and metabolism, Professor Ketan Patel, University of Reading

17.30 – 18.30 Drinks reception, lecture theatre foyer
### Day 2 – Wednesday 6 May

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker/Institution</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>09:00 – 09:20</strong></td>
<td><strong>Congenital muscular dystrophy (CMD) update</strong></td>
<td>Dr Anna Sarkozy, ICH Dubowitz Neuromuscular Centre</td>
</tr>
<tr>
<td><strong>09:20 – 09:45</strong></td>
<td><strong>Brain phenotypes in CMD</strong></td>
<td>Prof Francesco Muntoni, ICH Dubowitz Neuromuscular Centre</td>
</tr>
<tr>
<td><strong>09:45 – 10:30</strong></td>
<td><strong>Illustrative cases. Session chaired by Dr Kling Chong, Great Ormond Street Hospital</strong></td>
<td></td>
</tr>
<tr>
<td><strong>10:30 – 11:00</strong></td>
<td><strong>COFFEE</strong></td>
<td></td>
</tr>
<tr>
<td><strong>11:00 – 11:30</strong></td>
<td><strong>Congenital myasthenic syndromes- genetic update</strong></td>
<td>Prof David Beeson, Weatherall Institute of Molecular Medicine, Oxford</td>
</tr>
<tr>
<td><strong>11:30 – 12:00</strong></td>
<td><strong>Congenital myasthenic syndromes – presentations and management</strong></td>
<td>Dr Stephanie Robb, Institute of Child Health</td>
</tr>
<tr>
<td><strong>12:00 – 12:30</strong></td>
<td><strong>Neurophysiology in myasthenia. A guide for clinicians.</strong></td>
<td>Dr Matthew Pitt, Great Ormond Street Hospital</td>
</tr>
<tr>
<td><strong>12:30 – 13:00</strong></td>
<td><strong>Illustrative cases</strong></td>
<td></td>
</tr>
<tr>
<td><strong>13:00 – 14:00</strong></td>
<td><strong>LUNCH</strong></td>
<td></td>
</tr>
<tr>
<td><strong>14:00 – 14:10</strong></td>
<td><strong>Case presentation (recurrent respiratory infections in NMD)</strong></td>
<td></td>
</tr>
<tr>
<td><strong>14:10 – 14:30</strong></td>
<td><strong>Speech and language therapy evaluation.</strong></td>
<td>Juliet Taplin, Great Ormond Street Hospital</td>
</tr>
<tr>
<td><strong>14:30 – 15:00</strong></td>
<td><strong>Respiratory management</strong></td>
<td>Dr Collin Wallis, Great Ormond Street Hospital</td>
</tr>
</tbody>
</table>
15:00- 15:20  **Gastro-oesophageal reflux: fundoplication benefits and limitations**  
Mr Joe Curry, Great Ormond Street Hospital

15:20 – 15:45  **TEA**

15:45 – 16:15  **CPC discussion**  
Dr Imelda Hughes, Manchester Children’s Hospital

16:15 – 16:45  **CPC conclusion**  
Professor Francesco Muntoni and Adnan Manzur
Day 3 - Thursday 7 May

AM: 9.00 – 10.30 Common management problems in adult neuromuscular disorders

09:00 – 09:45 Prevention and treatment of osteoporosis in adults with neuromuscular disorders
Dr Richard Keen, UCL Hospitals NHS Trust and the Royal National Orthopaedic Hospital

09:45 – 10:30 Primary muscle disease and anaesthesia
Dr Nick Hirsch, National Hospital for Neurology and Neurosurgery

10:30 – 11:10 Management of pain (neuropathic, muscular and musculoskeletal) in adults with neuromuscular disorders
Dr Paul Nandi, UCL Hospitals NHS Trust

11:10 – 11:30 COFFEE

11:30 – 12:15 Genetics and pathogenesis of Motor Neurone Disease

12:15 – 13:00 Treatment and management of Motor Neurone Disease
Dr Katie Sidle, National Hospital for Neurology and Neurosurgery

13:00 – 14:00 LUNCH

14:00 – 14:45 Charcot Marie Tooth disease – emerging therapies
Prof Mary Reilly, MRC Centre for Neuromuscular Diseases

14:45 - 15:30 Current management strategies for Charcot Marie Tooth disease
Dr Matilde Laurá

15:30 – 15:45 TEA

15:45 – 16:30 To be confirmed
Day 4 – Friday 8 May 2014

09:00 – 09:30  Inclusion body myositis update
Dr Pedro Machado, MRC Centre for Neuromuscular Diseases

09:30 – 10:15  Mitochondrial Neuromuscular Diseases
Dr Rob Pitceathly

10:15 – 11:00  Muscle channelopathies
Professor Mike Hanna, Director, Institute of Neurology

COFFEE

11:30 – 12:15  Treatment of inflammatory neuropathies (CIDP, paraproteinaemic neuropathies)
Dr James Miller, Newcastle University

12:15 – 13:00  Illustrative cases inflammatory neuropathy – AIDP, CIDP, paraproteinaemic, vasculitis, infective)
(Hadi Manji and Mary Reilly)

13:00 – 13:45  LUNCH

13:45 – 14:30  The challenge of mononeuropathies
Dr Aisling Carr, MRC Centre for Neuromuscular Diseases

14:30 – 15:15  Surgical options for improving hand function in neuropathy
Dr Anthony McMullan, Royal National Orthopaedic Hospital

15:15 – 15:30  TEA

15:30 – 16.00  Rhabdomyolysis and Metabolic Disease in Adults
Dr Mark Roberts, Salford

16.00 – 16:30  Myotonic Dystrophy
Dr Chris Turner, MRC Centre for Neuromuscular Diseases

16.30 – 17.00  Drugs and Muscle Diseases
Dr Matt Parton, MRC Centre for Neuromuscular Diseases