



**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 (3)
Dr Vivek Mundada, Great Ormond Street Hospital
- 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**
Simon Blackburn, 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,** Prof Ketan Patel, University of Reading
- 17.30 – 18.30 *Drinks reception, lecture theatre foyer***

Day 2 – Wednesday 6 May

- Theme** **Congenital muscular dystrophy.
Is brain phenotype a diagnostic handle?**
- 09:00 – 09:20** **Congenital muscular dystrophy (CMD) update**
Dr Anna Sarkozy, ICH Dubowitz Neuromuscular Centre
- 09:20 – 09:45** **Brain phenotypes in CMD**
Prof Francesco Muntoni, ICH Dubowitz Neuromuscular Centre
- 09:45– 10:30** **Illustrative cases** (4) Case presentations lead:
Dr Daniel Carranza Rojo, Great Ormond Street Hospital
Session chaired by Dr Kling Chong, Great Ormond
Street Hospital
- 10:30 - 11:00** **COFFEE**
- Theme: congenital myasthenic syndromes**
- 11:00 – 11:30** **Congenital myasthenic syndromes- genetic update**
Prof David Beeson, Weatherall Institute of Molecular Medicine, Oxford
- 11:30 – 12:00** **Congenital myasthenic syndromes – presentations and
management**
Dr Stephanie Robb, Institute of Child Health
- 12:00 – 12:30** **Neurophysiology in myasthenia: a guide for clinicians.**
Dr Matthew Pitt, Great Ormond Street Hospital
- 12: 30 - 13.00** **Illustrative cases** (3)
Dr Pinki Munot, Great Ormond Street Hospital
- 13:00 – 14:00** **LUNCH**
- Theme: Management of frequent respiratory infections**
- 14:00 – 14:10** **Case presentation (recurrent respiratory infections in NMD)**
Adnan Manzur, ICH Dubowitz Neuromuscular Centre
- 14:10 – 14:30** **Speech and language therapy evaluation.**
Juliet Taplin, Great Ormond Street Hospital
- 14:30 – 15:00** **Respiratory management**
Dr Collin Wallis, Great Ormond Street Hospital

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, ICH

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** **Treatment and management of Motor Neurone Disease**
Dr Katie Sidle, National Hospital for Neurology and Neurosurgery
- 12:15 – 13:00** **Genetics and pathogenesis of Motor Neurone Disease**
Prof Chris Shaw, King's College London
- 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, MRC Centre for Neuromuscular Diseases
- 15:30 – 15:45** **TEA**
- 15:45 – 16:30** **New models of care for adults with neuromuscular diseases**
Emma Matthews, MRC Centre for Neuromuscular Diseases
- 16:30 – 17:00** **Diagnosis and management of polymyositis and dermatomyositis**
Dr Simon Hammans, Wessex Neurological Centre, University Hospital Southampton

Day 4 – Friday 8 May

- 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, National Hospital for Neurology and Neurosurgery
- 10:15 – 11:00** **Muscle channelopathies**
Prof Mike Hanna, Director, Institute of Neurology
- 11:00 - 11:30** **COFFEE**
- 11:30 – 12:15** **Treatment of inflammatory neuropathies (CIDP, paraproteinaemic neuropathies)**
Dr James Miller, Newcastle Upon Tyne NHS Foundation Trust
- 12:15 -13.00** **Illustrative cases inflammatory neuropathy – AIDP, CIDP, paraproteinaemic, vasculitis, infective)**
Professor Mary Reilly and Dr Hadi Manji, MRC Centre for Neuromuscular Diseases
- 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 Marco Sinisi, 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