

AGENDA

MDUK Oxford Neuromuscular Centre Annual Meeting

Wednesday 26th March 2025
L2, Mathematical Institute, University of Oxford

08:00 - 08:30 **Registration, Tea, & Pastries**

Session 1: Future plans for the MDUK Oxford Neuromuscular Centre

08:30 - 09:20

Welcome from the Director
Prof Matthew Wood, *Dept of Paediatrics, University of Oxford*

Early diagnosis and treatment in DMD
Prof Laurent Servais, *Dept of Paediatrics, University of Oxford*

Session 2: DMD diagnosis and evaluation – Chair: Prof Dame Kay Davies

09:20 - 10:40

Newborn screening for DMD: milestones and remaining challenges
Prof Emma Ciafaloni, *University of Rochester Medical Center*

SV95C: Can digital endpoints be the solution for early evaluation of boys with DMD?
Damien Eggensteiner, *SYSNAV Healthcare*

Access to treatments: what we should all be thinking about from diagnosis and beyond
Emily Reuben, *Duchenne UK*

10:40 - 11:00 **Tea & Coffee**

Session 3: DMD therapies – Chair: Prof Laurent Servais

11:00 - 12:30

Treating the patient with DMD: choices and challenges
Prof Richard Finkel, *St Jude Children's Research Hospital*

Industry perspective on early interventional therapies in DMD
Panel members: Dr Angela Guerra (*Avidity Biosciences*), Dr Ashish Dugar (*Dyne Therapeutics*), Dr Hayley Parker (*PepGen*), Dr Shabir Hasham (*Santhera Pharmaceuticals*), Dr Rachel Salazar (*Sarepta Therapeutics*), and Dr Alexander Lin (*Wave Life Sciences*)

12:30 - 13:30 **Lunch**

Session 4: Motor Neurone Disease – Chair: Prof Martin Turner

13:30 - 15:20

Studying pre-symptomatic populations at risk of ALS
Dr Alex Thompson, *Nuffield Dept of Clinical Neurosciences, University of Oxford*

From pre-clinical models to Experimental Medicine in ALS
Prof Kevin Talbot, *Nuffield Dept of Clinical Neurosciences, University of Oxford*

Biomarkers of treatment response in antisense oligonucleotide (Tofersen) studies in ALS
Dr Carrie Rubel, *Biogen*

Creation of de novo cryptic splicing for ALS and FTD precision medicine
Prof Pietro Fratta, *University College London*

15:20 - 15:40 *Tea & Coffee*

Session 5: Featured Centre projects – Chair: Dr Sithara Ramdas

15:40 - 17:00

Using immunoassays to unravel the inflammatory neuropathies

A/Prof Simon Rinaldi, *Nuffield Dept of Clinical Neurosciences, University of Oxford*

Myasthenia gravis - changes in demographics, features and management over the last decades

A/Prof Isabel Leite, *Nuffield Dept of Clinical Neurosciences, University of Oxford*

Expanding the CRISPR toolkit for neuromuscular disease

Prof Carlo Rinaldi, *Dept of Paediatrics, University of Oxford*

Wrap-up and closing

Dr Sithara Ramdas, *Oxford University Hospitals*

17:00 - 18:00

Posters & Drinks Reception

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