

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 pre-symptomatic 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*

Stride velocity 95th centile (SV95C) in children with DMD (title TBD)

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

DMD therapies (title TBD)

Prof Richard Finkel, *St Jude Children's Research Hospital*

Industry perspective on early interventional therapies in DMD

Panel members: Dr Steve Hughes (*Avidity Biosciences*), Dr Ashish Dugar (*Dyne Therapeutics*), Dr Michelle Mellion (*PepGen*), Dr Shabir Hasham (*Santhera Pharmaceuticals*), Dr Suki Malhi (*Wave Life Sciences*), and Dr Rachel Salazar (*Sarepta Therapeutics*)

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*

Experimental medicine approaches to ALS treatments

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

Expanding the CRISPR toolkit for neuromuscular disease
Prof Carlo Rinaldi, *Dept of Paediatrics, University of Oxford*

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*

Wrap-up and closing
Dr Sithara Ramdas, *Oxford University Hospitals*

17:00 - 18:00 **Posters & Drinks Reception**

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