

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	<p>Welcome from the Director Prof Matthew Wood, <i>Dept of Paediatrics, University of Oxford</i></p> <p>Early diagnosis and pre-symptomatic treatment in DMD Prof Laurent Servais, <i>Dept of Paediatrics, University of Oxford</i></p>
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Session 2: DMD diagnosis and evaluation – Chair: Prof Dame Kay Davies

09:20 - 10:40	<p>Newborn screening for DMD: milestones and remaining challenges Prof Emma Ciafaloni, <i>University of Rochester Medical Center</i></p> <p>Stride velocity 95th centile (SV95C) in children with DMD (title TBD) Damien Eggensteiner, <i>SYSNAV Healthcare</i></p> <p>Access to treatments: what we should all be thinking about from diagnosis and beyond Emily Reuben, <i>Duchenne UK</i></p>
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10:40 - 11:00 **Tea & Coffee**

Session 3: DMD therapies – Chair: Prof Laurent Servais

11:00 - 12:30	<p>DMD therapies (title TBD) Prof Richard Finkel, <i>St Jude Children's Research Hospital</i></p> <p>Industry perspective on early interventional therapies in DMD Panel members: Dr Steve Hughes (<i>Avidity Biosciences</i>), Dr Ashish Dugar (<i>Dyne Therapeutics</i>), Dr Steve Han (<i>PepGen</i>), Dr Shabir Hasham (<i>Santhera Pharmaceuticals</i>), Dr Suki Malhi (<i>Wave Life Sciences</i>), and Dr Rachel Salazar (<i>Sarepta Therapeutics</i>)</p>
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12:30 - 13:30 **Lunch**

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

13:30 - 15:20	<p>Studying pre-symptomatic populations at risk of ALS Dr Alex Thompson, <i>Nuffield Dept of Clinical Neurosciences, University of Oxford</i></p> <p>From pre-clinical models to Experimental Medicine in ALS Prof Kevin Talbot, <i>Nuffield Dept of Clinical Neurosciences, University of Oxford</i></p> <p>Biomarkers of treatment response in antisense oligonucleotide (Tofersen) studies in ALS Dr Carrie Rubel, <i>Biogen</i></p> <p>Creation of de novo cryptic splicing for ALS and FTD precision medicine Prof Pietro Fratta, <i>University College London</i></p>
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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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