

## AGENDA

# MDUK Oxford Neuromuscular Centre Annual Meeting

Wednesday 26<sup>th</sup> 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><b>Welcome from the Director</b> Prof Matthew Wood, <i>Dept of Paediatrics, University of Oxford</i></p> <p><b>Early diagnosis and treatment in DMD</b> 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><b>Newborn screening for DMD: milestones and remaining challenges</b> Prof Emma Ciafaloni, <i>University of Rochester Medical Center</i></p> <p><b>Stride velocity 95<sup>th</sup> centile (SV95C) in children with DMD (title TBD)</b> Damien Eggensteiner, <i>SYSNAV Healthcare</i></p> <p><b>Access to treatments: what we should all be thinking about from diagnosis and beyond</b> 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><b>DMD therapies (title TBD)</b> Prof Richard Finkel, <i>St Jude Children's Research Hospital</i></p> <p><b>Industry perspective on early interventional therapies in DMD</b> 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><b>Studying pre-symptomatic populations at risk of ALS</b> Dr Alex Thompson, <i>Nuffield Dept of Clinical Neurosciences, University of Oxford</i></p> <p><b>From pre-clinical models to Experimental Medicine in ALS</b> Prof Kevin Talbot, <i>Nuffield Dept of Clinical Neurosciences, University of Oxford</i></p> <p><b>Biomarkers of treatment response in antisense oligonucleotide (Tofersen) studies in ALS</b> Dr Carrie Rubel, <i>Biogen</i></p> <p><b>Creation of de novo cryptic splicing for ALS and FTD precision medicine</b> 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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