

## AGENDA

# MDUK Oxford Neuromuscular Centre Annual Meeting

Wednesday 26<sup>th</sup> March 2025

L2, Andrew Wiles Building, 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*), Jake Massa (*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**

A/Prof 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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