

AGENDA

MDUK Oxford Neuromuscular Centre Annual Meeting

Tuesday 26th September 2023

Investcorp Auditorium, St. Antony's College, 62 Woodstock Rd, OX2 6JF, Oxford

08:30 - 09:00 **Registration, Tea, & Pastries in Investcorp Foyer**

Session 1: Welcome & Keynote speaker

09:00 - 09:10 **MDUK Oxford Neuromuscular Centre – Welcome from the Director**
Prof Matthew Wood, *University of Oxford*

09:10 - 10:10 **Advanced in vitro modelling of neuromuscular diseases and therapies**
Prof Francesco Saverio Tedesco, *University College London and The Francis Crick Institute*

10:10 - 10:35 **Tea & Coffee in The Buttery**

Session 2: Pre-clinical NMD research – Chair: Prof David Bennett

A novel mouse model of muscle nAChR clustering deficiency CMS
Dr Yin Dong, *University of Oxford*

Mitochondrial dysfunction in iPSC-derived motor neurons from ALS patients
Dr Ruxandra Dafinca, *University of Oxford*

10:35 - 12:00 **Novel insights into dystrophic pathology and dystrophin restoration therapies from gene expression analysis**
Dr Thomas Roberts, *University of Oxford*

Insights into ALS from the cerebrospinal fluid proteome
Dr Alex Thompson, *University of Oxford*

Chemogenetic silencing of sensory neuron-driven pain
Dr Jimena Perez-Sanchez, *University of Oxford*

12:00 - 13:00 **Lunch in The Buttery and poster setup**

Session 3: Bridging the divide: where bench and bedside meet – Chair: Prof Laurent Servais

13:00 - 14:10	The Genomics England Generation Study Dr Katrina Stone, <i>Genomics England</i>
	What academics should know if they want to see their drug used in clinic Cliff Bechtold, <i>Biohaven Pharma</i>
	Planning for translation in Duchenne Muscular Dystrophy Dr Amy Donner, <i>Wave Life Sciences</i>
	Developing an experimental medicine approach to testing drugs in ALS Prof Martin Turner, <i>University of Oxford</i>

14:10 - **14:35** ***Tea & Coffee in The Buttery***

Session 4: NMD in the clinic – Chair: Assoc Prof Carlo Rinaldi

14:35 - 15:30	The importance of natural history studies – with nemaline myopathy as an example Dr Gemma Fisher, <i>University of Oxford</i>
	Microdystrophin in DMD: hopes, fears, and limitations Dr Serge Braun, <i>AFM-Telethon, Genosafe, and Genethon</i>
	Congenital Myasthenia Syndromes- Defining outcome measures for clinical trials Dr Sithara Ramdas, <i>University of Oxford</i>

Session 5: Closing speaker & Wrap-up – Chair: Prof Kevin Talbot

15:30 - 16:20	Expanding genetic therapies to treat all rare disorders Prof Stephan Sanders, <i>University of Oxford</i>
16:20 - 16:30	Wrap-up and Closing Prof Kevin Talbot, <i>University of Oxford</i>

16:30 - **18:00** ***Posters & Drinks Reception in The Buttery***