

Event programme

14th UK Annual Neuromuscular Translational Research Conference

New therapies in Neuromuscular Diseases

Online event

25th and 26th March 2021

(international colleagues pls note all times given are in GMT – Greenwich Mean Time)

Day 1 – Thursday 25 March 2021

Poster viewing: Prior to the event official start time, of 13:00 hr, research posters will be available in the morning for viewing online . Links will be provided to all registered delegates to view the posters.

13:00 –13: 10 **Introduction and Housekeeping**
Professor Michael Hanna
Director, CNMD, ICGNMD and UCL Institute of Neurology

13: 10- 13:2 5 **Welcome from UCL President and Provost**
Dr Michael Spence AC

New therapy developments in neuromuscular diseases - I

Session Chairs: Professor Francesco Muntoni and Professor Volker Straub

13: 25–13: 55 **RNA antisense therapeutics for neuromuscular and neurological conditions**
Professor Adrian R Krainer
Cold Spring Harbor Laboratory , NY, USA

13: 55–14: 25 **New therapies in ALS**
Professor Dame Pamela Shaw
Sheffield Institute for Translational Neuroscience (SITraN)

14: 25–14: 40 Comfort Break and sponsor slides

14: 40–15: 10 **Window for optimal therapeutic intervention in SMA**
Dr Charlotte Sumner
Johns Hopkins University, Baltimore , USA

15: 10–16: 10 **Flash poster sessions**
Chairs: Professor Mary Reilly (CNMD and ICGNMD, UCL UCL) and Professor Rita Horvath (Dept of clinical neurosciences, University of Cambridge)

Comparing age-dependent changes in heteroplasmy of an mtDNA point-mutation between mitotic and post-mitotic tissues using a mouse model of mitochondrial disease

Dr Tiago Bernadino Gomes
Wellcome Trust Centre for Mitochondrial Research, Newcastle University

Investigation of Compounds Inducing Alpha Tubulin Acetylation by Drug Repurposing Approach

Ms Özge Çetin
Keele University

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Pre-operative exercise and pyrexia as modifying factors in Malignant Hyperthermia

Dr Heinz Jungbluth
IoPPN, King's College London

Combining the power of patients and mouse models to identify biomarkers in CMT

Mr Matthew Jennings
Department of Clinical Neurosciences,
Cambridge University

Therapeutic potential of reducing Krüppel-like factor 15 (Klf15) activity in Spinal Muscular Atrophy (SMA)

Ms Emma Sutton
Keele University

Impact of Teleneurology Neuromuscular Clinic on the accessibility of care for patients with Inherited Neuromuscular disorders during COVID-19 pandemic

Dr Vishnu Venugopalan Y
All India Institute of Medical Sciences, New Delhi, India

Clinical and genetic spectrum of a large cohort of delta-sarcoglycan muscular dystrophy

Dr Jorge Alonso -Perez
Hospital de la Santa Creu i Sant Pau, Universitat de Barcelona, Spain

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Day 2 – Friday 26th March

New therapy developments in neuromuscular diseases – II

Session Chairs: Professor Mary Reilly and Professor Bobby McFarland

13:00 –13:10 Welcome and housekeeping day 2
Professor Mary Reilly
CNMD and ICGNMD, UCL

13:10 –13:40 **Modifying Muscular Dystrophy**
Elizabeth McNally, M.D., Ph.D.
Northwestern University Feinberg School of Medicine
Chicago, USA

13:40 –14:10 **Giant Axonal Neuropathy:
From Outcome Measures to First-in-Human AAV Mediated Intrathecal Gene Transfer**

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