



2021 UK Annual Neuromuscular Translational Research Conference (Online event)

New therapies in Neuromuscular Diseases

25th and 26th March 2021

PROGRAMME

Day 1 – Thursday 25 March 2021

13:00 – 13:15

Introduction

Professor Michael Hanna
Director, MRC Centre and UCL Institute of Neurology

New therapy developments in neuromuscular diseases - I Session Chairs: Professor Francesco Muntoni and Professor Volker Straub

13:15 – 13:45

RNA therapeutics for neuromuscular conditions

Professor Adrian R Krainer
Cold Spring Harbor Laboratory, NY, USA

13:45 – 14:15

New therapies in ALS

Professor Dame Pamela Shaw
Sheffield Institute for Translational Neuroscience (SITraN)

14:15 – 14:30

Comfort Break and sponsor posters

14:30 – 15:00

Developing Therapies in Neuropathies

Dr Charlotte Sumner
Johns Hopkins University, Baltimore, USA

15:00 – 16:00

Flash poster sessions

Chairs: Professor Mary Reilly and Professor Rita Horvath

16:00 – 16:15

Comfort break and sponsor posters

16:15 – 16:30

MDUK update

16:15 – 17:00

The fifth Morgan-Hughes Thomas lecture introduced by Professor Michael Hanna

The origins of mtDNA mutations: implications for neuromuscular disease
Professor Patrick Chinnery
Cambridge University, UK

Day 2 – Friday 26th March

New therapy developments in neuromuscular diseases – II

Session Chairs: Professor Mary Reilly and Professor Bobby Macfarland

- 13:00 – 13:10 Welcome and housekeeping
- 13:10 – 13:40 **Gene Modifiers**
Elizabeth McNally, M.D
Northwestern University
Chicago, USA
- 13:40 – 14:10 **Intrathecal AAV for Giant Axonal Neuropathy: lessons learned**
Carsten Bönnemann, M.D.
NIH, USA
- 14:10 – 14:25 *Comfort break and sponsor posters*
- 14:25 – 15:25 **4 x Platform presentations**
Chairs: Professor Rob Taylor and Dr Rob Pitceathly
- 15:25 – 16:25 **Sponsored industry symposia**
Session Chairs: Professor Thomas Voit and Professor Patrick Chinnery
3 x 20 minute sponsored symposia
- 16:25 – 17:10 **The fifth Victor Dubowitz Lecture introduced by Professor Francesco Muntoni, UCL GOSH ICH**
Spinal Muscular Atrophy : About a lethal disorder that is disappearing for 3 years (in Southern Belgium)
Professor Laurent Servais, Oxford University
- 17:10 – 17:20 **Prizes and close**