

Event programme

15th UK Annual Neuromuscular Translational Research Conference

Online event

26th and 27th April 2022

Please note this is a draft programme to be used for reference/illustrative purposes.

This programme has not yet been advertised.

Speakers names / talk titles may change.

The order in which speakers appear in the programme may change from this initial draft.

(international colleagues pls note time zone is UTC+01:00)

Day 1 – Tuesday 26th April 2022

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 – 13:15

Introduction and Housekeeping

Professor Michael Hanna
Director, CNMD, ICGNMD and UCL Institute of Neurology

Session 1:

Session Chairs: [2 named session chairs]

13:15 – 13:45

Transcript Imbalance in Charcot-Marie-Tooth Disease: Validating Causes and Consequences

Professor John Svaren
Director, IDD Models Core
University of Wisconsin-Madison

13:45 – 14:15

Applying and integrating genomic and transcriptomic technologies to the diagnosis of rare neuromuscular disease.

Dr Vijay Ganesh
Broad Institute

14:15 – 14:30

Comfort Break and sponsor slides

14:30 – 15:00

The relevance of Smart Technologies for patient reported outcomes/remote assessments

Dr Elin Haf Davies
CEO, Aparito

15:00 – 15:20

Sponsor symposium 20 mins

15:20 – 16:20

Flash poster sessions 1 hour

Poster session chairs: [2 named session chairs]

8 x flash posters of 5 min + 2 min questions each. 3 slides max.

16:20 – 16:35

Comfort break and sponsor slides

16:35 – 17:05

How understanding AAV capsid biology can improve viral-mediated gene therapies

Speaker tbc

17:05 – 17:35

Rare disease research in Europe and beyond – why the UK must continue to collaborate

Victoria Hedley
Rare Disease Policy Manager
Newcastle University

Day 2 – Weds 27th April 2022

- 13:00 – 13:10 *Welcome and housekeeping day 2*
- 13:10 – 14:10 **Platform presentations** 1 hour
Platform presentation chairs: [2 named session chairs]
- 4 x platform presentation of 10 min talk + 3 min questions
- 14:10 – 14:20 **MDUK update** 10 min
- 14:20 – 14:40 **Sponsor symposium** 20 mins
- 14:40 – 14:55 *Comfort break and sponsor posters*

Session 2

Session Chairs: [2 named session chairs]

- 14:55 – 15:25 **Therapeutic Approaches for FSHD**
Professor Scott Harper
Center for Gene Therapy, Nationwide Children's Hospital // Ohio State University College of Medicine
- 15:25 – 15:55 **Application of omics-technologies in congenital myopathies**
Dr Jim Dowling
Senior Scientist, Genetics & Genome Biology, Hospital for Sick Children (Sick Kids) Toronto/ University of Toronto
- 15:55 – 16:10 *Comfort break and sponsor posters*
- 16:10 – 16:40 **Implementing the UK Rare Diseases Framework**
Dr Louise Wood CBE
Director: Science, Research & Evidence, Deputy CEO - National Institute for Health Research
Department of Health and Social Care
- 16:40 – 17:10 **Systems biochemistry approaches to exploring mitochondrial protein function and disease pathogenesis**
Dr David J. Pagliarini
BJC Investigator and the Hugo F. and Ina C. Urbauer Professor of Cell Biology & Physiology, Biochemistry & Molecular Biophysics, and Genetics. Washington University in St. Louis School of Medicine
- 17:10 Prizes and close

CPD credits: *This event (ref xxxxxx) has been approved by the Federation of the Royal Colleges of Physicians of the United Kingdom for 6 category 1 (external) CPD credit(s).*

Event sponsorship: *This meeting is sponsored by the companies listed at the bottom of this page as 'event sponsors'. The companies have had no input into the content or agenda of this meeting. As sponsors, the companies have been invited to provide the following specific items:*

Sponsor research poster: xxxx

Sponsor slide in event breaks: xxxxx

Logo and brief company description of each sponsor is included in the delegate booklet.