



## **13<sup>th</sup> UK Neuromuscular Translational Research Conference**

*UCL GOSH ICH, 30 Guilford Street, London WC1N 1EH  
Kennedy Lecture Theatre*

Wednesday 22 and Thursday 23 April 2020

### **PROGRAMME**

#### **Day 1 – Wednesday 22<sup>nd</sup> April**

09:00 – 09:30      **Registration and Coffee**

09:30 – 09:45      **Introduction**  
Prof Michael Hanna  
Director, MRC Centre and UCL Institute of Neurology

#### **Session 1: Chairs:                      Genetic Therapies in NM disease**

09:45 – 10:15      **Intrathecal AAV for Giant Axonal Neuropathy: lessons learned**  
Carsten Bönnemann, M.D.  
NIH, USA

10:15 – 10:45      **Therapeutic developments for Laminin2 deficient muscular dystrophy**  
Prof Dr Markus Ruegg  
Biozentrum, University of Basel

10:45 – 11:15      **Coffee**

11:15 – 11:45      **RNA therapeutics for neuromuscular and neurological conditions**  
Professor Adrian Krainer  
Cold Spring Harbor Laboratory, New York

11:45 – 11:55      Platform presentation 1

11:55 – 12:05      Platform presentation 2

- 12:05 – 12:15      **Late breaking scientific update**
- 12:15 – 14:30      **Lunch and guided poster sessions (poster session commences at 13:00)**
- Session 2:  
Chairs:**
- 14:30 – 15:00      **Somatic CTG repeat expansion in myotonic dystrophy: dynamics, consequences and therapeutic opportunities**  
Professor Darren Monckton  
University of Glasgow
- 15:00 – 15:30      **New therapeutic Developments in ALS**  
Professor Chris Shaw  
Kings College London
- 15:30 – 16:00      **Coffee and posters**
- 16:00 – 16:10      Platform presentation 3
- 16:10 – 16:20      Platform presentation 4
- 16:20 – 16:30      Platform presentation 5
- 16:30 – 16:45      **MRC Update**
- 16:45 – 17:00      **MDUK Update**
- 17:00 – 18:00      The fifth Morgan-Hughes Thomas lecture  
**The origin of mtDNA mutations: implications for neuromuscular diseases**  
Prof Patrick Chinnery, Cambridge University
- 18:00 – 18:30      **Poster viewing**
- 19:00                  **Drinks reception**  
Goodenough College, Mecklenburgh Square, London WC1N 2AB
- 20:00                  **Gala dinner**  
Goodenough College, Mecklenburgh Square, London WC1N 2AB

*To Note: Those displaying posters. To allow for judging and poster sessions, posters may only be put up and taken down during these times:*

*Day 1 Weds 22 April*

*Posters up: 8.45 am to 9.15 am*

*Posters down: 18:30 – 18.45 pm*

## Day 2 – Thursday 23<sup>rd</sup> April

### Session 3:

- 08:30 – 09:00 Prof Mary Reilly
- 09:00 – 09:30 **Developing gene therapies for inherited demyelinating neuropathies**  
Prof. Kleopas A. Kleopa  
Cyprus Institute of Neurology and Genetics
- 09:30 – 10:00 **Genetics of Painful neuropathies**  
Professor David Bennett  
University of Oxford
- 10:00 – 11:40 **Poster guided sessions and coffee (poster session commences at 10:10)**
- 11:40 – 11:50 Platform Presentation 6
- 11:50 – 12:00 Platform presentation 7
- 12:00 – 12:45 The fifth Victor Dubowitz Lecture  
**Spinal Muscular Atrophy : About a lethal disorder that is disappearing for 2 years (in Southern Belgium)**  
Prof Laurent Servais, Oxford University
- 12:45 – 13:30 **Lunch**
- 13:30 – 14:30 **Poster flash sessions**  
Chaired by Prof. Michael Hanna, UCL Institute of Neurology
- Session 4: MRI in NM disease**
- 14:30 – 15:00 **ImagingDMD long-term natural history study in DMD**  
Dr Rebecca Willcocks  
Research Assistant Professor  
University of Florida
- 15:00 – 15:30 **Coffee and posters**
- 15:30 – 16:00 **Using machine learning for supporting diagnostic MR imaging in NMD**  
Dr Jordi Díaz Manera  
Hospital de la Santa Creu i Sant Pau de Barcelona
- 16:00 – 16:30 MRI Talk title tbc

- 16:30 – 16:45 Platform presentation 8
- 16:45 – 17:00 Platform presentation 9
- 17:00 – 17:15 **Poster prizes and close**

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*Day 2 Thurs 23 Aprilh*

*Posters up: evening of 22<sup>nd</sup> 18.45 pm onwards OR by 8.30 am latest 23 April*

*Building access is from 8am.*

*Posters down: 16:30 onwards*