12th UK Neuromuscular Translational Research Conference

Centre for Life, Newcastle

Thursday 4 and Friday 5 April 2019

PROGRAMME

Day 1 – Thursday 4 April

09:00 – 09:30  Registration and Coffee

09:30 – 09:40  Introduction
Prof Michael Hanna
Director, MRC Centre for Neuromuscular Diseases and Institute of Neurology, UCL.

Session 1: International Collaborations for Genomic Research in NMD
Chairs: Prof Michael Hanna, Prof Volker Straub¹, and Prof Patrick Chinnery²
¹Director, John Walton Muscular Dystrophy Research Centre, Newcastle University and Newcastle Hospitals NHS Foundation Trust.
²Head of Department of Clinical Neurosciences, University of Cambridge

09:40 – 09:55  Overview of vision for Centre – Prof Mike Hanna and Prof Volker Straub

09:55 – 10:15  Brazil – Prof Wilson Marques- University of São Paulo
10:15 – 10:35  South Africa – Prof Francois van der Westhuizen – North West University
10:35 – 10:55  Zambia – Dr Michelle Kvalsund
10:55 – 11:15  Turkey – Yavuz Oktay - Dokuz Eylül University and Prof Haluk Topaloglu – Hacettepe University
11:15 – 11:35  India – Dr K Thangaraj - CCMB
11:35 – 12:00  Coffee
12:00 – 12:30  Platform 1 and Platform 2
12:30 – 12:45  Late breaking – 15 mins
12:45 – 13:30  Lunch
13:30 – 14:45  Poster guided sessions and coffee

Session 2:  Kate Bushby – an advocate for translational research in NMD
Chairs:  Prof Volker Straub, Newcastle University and Prof Mary Reilly, Co-Director MRC CNMD, UCL

14:45 – 15:05  Clinical services for Neuromuscular Disease patients
Prof Sir Doug Turnbull, Newcastle University
15:05 – 15:25  From Gene Discovery to Therapy
Prof Francesco Muntoni, UCL GOSH ICH
15:25 – 15:45  The FOR DMD trial: Individualized vs standardized treatment
Prof Robert C Griggs, University of Rochester, NY
15:45 – 16:05  Vision-DMD, developing an alternative to steroids
Dr Michela Guglieri, Newcastle University

16:05 – 16:35  Coffee and posters
16:35 – 16:50  EURO-NMD, a reference network for neuromuscular diseases
Dr Teresinha Evangelista, Newcastle University
16:50 – 17:05  The DMD-Hub, increasing trial capacity in the UK
Emma Heslop, Newcastle University
17:05 – 17:20  Promoting rare disease policies across Europe
Victoria Hedley, Newcastle University
17:20 – 17:30  Comfort break
17:30 – 18:30  1 hour keynote speaker – 4th John Walton Lecture
What makes Genetics Human?
Prof Sir John Burn, Newcastle University

19:30  Drinks reception and Gala dinner
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After dinner speaker: Prof Katie Bushby
Day 2 – Friday 4 April

Session 3: Developing Genetic Therapies
Chairs: Prof Dame Kay Davies\(^3\) and Francesco Muntoni\(^4\)
\(^3\)Dr Lee’s Professor of Anatomy Oxford Neuromuscular Centre
\(^4\)Director, Dubowitz Neuromuscular Centre, ICL GOSH ICH

08:30 – 09:00 Genome Editing for Duchenne Muscular Dystrophy
Dr Charles Gersbach, Duke University

09:00 – 09:30 Alternate gene therapy approaches to DMD
Dr Kevin Flanigan, Nationwide Children’s Hospital, Columbus, Ohio

09:30 – 10:00 Ignite DMD
Dr Barry Byrne, University of Florida

10:00 – 11:15 Poster guided sessions and coffee

11:15 – 11:45 Platform 3 and 4

11:45 – 12:45 Poster flash sessions
Chaired by: (tbc)

12:45 – 13:45 Lunch

Session 4: Mitochondrial Disease
Chairs: Professor Bobby Mcfarland\(^5\) and Professor Rita Horvath\(^6\)
\(^5\)Wellcome Centre for Mitochondrial Research, Newcastle University
\(^6\)Department of Clinical Neurosciences, University of Cambridge

13:45 – 14:15 Development and Optimisation of techniques to prevent transmission of mitochondrial DNA disease
Professor Mary Herbert, Newcastle University

14:15 – 14:45 Clinical aspects of prevention of mitochondrial disease
Grainne Gorman, Newcastle University

14:45 – 15:15 Coffee and posters

15:15 – 15:45 Platform 5 and 6

15:45 – 16:15 Platform 7 and 8

16:15 – 16:30 MDUK update (speaker tbc)
16:30 – 16:45  MRC international / post-Brexit strategy (speaker tbc)
16:45 – 17:00  Poster prizes and close