ENCALS DRAFT PROGRAM
Oxford 20th-22nd June 2018

Each plenary presentation is 25 minutes plus 5 minutes questions; each platform presentation is 12 minutes plus 3 minutes for questions.

WEDNESDAY 20TH JUNE

12.00-13.00 Lunch and Registration

13.00-13.30 Welcome (Leonard van den Berg, Orla Hardiman)
Sally Light, Chief Executive MND Association will open the meeting
Evy Reviers: EuPals

SESSION 1 Chair: Orla Hardiman, Dublin

13.30-14.00 Roadmaps to therapy in ALS
Matthew Kiernan, Sydney

14.00-15.30 Biophysical basis of the acute effects of riluzole and retigabine on motor axonal excitability in patients with ALS
Boudewijn Sleutjes, Leuven

Connectome-based disease progression model for amyotrophic lateral Sclerosis
Jil Meier, Utrecht

CSF chitinase protein performance as ALS biomarkers
Alexander Thompson, Oxford

Imaging of brain metabolism in asymptomatic C9orf72 repeat expansion carriers and non-carriers using 31P-MRSI at 7T
Henk-Jan Westeneng, Utrecht

Non-coding RNA serum biomarkers in ALS
Greig Joilin, Sussex

Electric shock and extremely low-frequency magnetic field exposure and risk of ALS: Euro-MOTOR
Susan Peters, Utrecht

15.30-16.00 BREAK
SESSION 2  Chair: Ammar Al-Chalabi, London (King’s)

16.00-16.30  How many DNA samples is enough?
Michael van Es, Utrecht

16.30-18.00  Oligogenic and discordant inheritance: A population based genomic study of Irish kindreds carrying the C9orf72 repeat expansion
Marie Ryan, Dublin

Next Generation Sequence Analysis of Telomere Length in ALS
Ahmad Al Khleifat, London (King’s)

Genome-wide Analyses Identify KIF5A as a Novel ALS Gene
Kevin Kenna, Dublin

Modelling FUS-amyotrophic lateral sclerosis using gene-edited isogenic iPSC reporter lines
Lara Marrone, Dresden

Exogenous recombinant FUS is able to accumulate in cortical neurons in mouse brain
Mattia Perez, Strasbourg

Superoxide dismutase prions transmit ALS disease in mice expressing hemizygous D90A hSOD1
Elaheh Ekhtiari Bidhendi, Umea

1800.19.30 POSTERS

19.30-20.30 Project MINE meeting (invitation only)
THURSDAY 21ST JUNE

SESSION 3 Chair: Wim Robberecht, Leuven

0900.09.30 Reversing aberrant phase transitions connected to ALS
James Shorter, Philadelphia (U Penn)

09.30-10.30 Studying the interaction between TARDBP and p62/SQSTM1: a look into RNA processing defects in ALS
Raphael Munoz-Ruiz, Paris (ICM)

Endogenous TDP-43 mutant mice develop ALS characteristics in vivo and show novel gain of splicing function
Pietro Fratta, London (UCL)

FUS-induced neurotoxicity in Drosophila is prevented by downregulating nucleocytoplasmic transport proteins
Jolien Steyaert, Leuven

Structural and functional MRI reveals frontal cortical deficits in a TDP-43 knock-in mouse model of ALS-FTD
Ziqiang Lin, London (King’s)

10.30-11.00 BREAK

SESSION 4 Chair:

11.00-11.30 Neuropathological heterogeneity across the ALS spectrum
Olaf Ansorge, Oxford

11.30-12.30 Synapse loss in the prefrontal cortex is associated with cognitive decline in amyotrophic lateral sclerosis
Christopher Henstridge, Edinburgh

The anterior cingulate cortex in the ALS-FTD spectrum: post mortem MRI-histology correlation
Anna Leonte, Oxford

Selective vulnerability of the primary motor cortex in amyotrophic lateral sclerosis
Matthew Nolan, Oxford

Investigation of dysfunction in cognitive brain networks in ALS by localisation of the sources of mismatch negativity
Roisin McMackin, Dublin
12.30-13.30  **LUNCH**

**SESSION 5**  Chair: **Karin Danzer, Ulm**

13.30-14.00  Axonal mRNA biology: implications for axonal maintenance  
**Christine Holt, Cambridge**

14.00-15.30  HDAC6 inhibition reverses axonal transport defects in iPSC-derived motor neurons from FUS-ALS patients  
**Wenting Guo, Leuven**

Serum microRNA profiles identify the Fragile-X-protein family as novel neuropathological markers in ALS  
**Axel Freischmidt, Ulm**

C9ORF72 repeat expansions cause axonal transport defects in iPSC-derived motor neurons  
**Laura Fumagalli, Leuven**

Axon-seq decodes the motor axon transcriptome and its modulation in response to amyotrophic lateral sclerosis  
**Jik Nijssen, Stockholm**

MicroRNAs secreted by C9orf72 patient-derived astrocytes contribute to impairment in axonal growth and cell death in vitro  
**Andrea Varcianna, Sheffield**

Directly converted Astrocytes from ALS patient fibroblasts stratify Disease phenotypes and identify miR-146a as a potential therapeutic target  
**Caítiia Gomes, Lisbon**

15.30-16.00  **BREAK**

**SESSION 6**  Chair: **Pam Shaw**

16.00-17.00  Transcriptomic analysis of iPSC-derived motor neurons from C9orf72 ALS/FTD patients  
**Ana Candalija, Oxford**

Synergistic mechanisms of C9orf72 gain and loss of function  
**Hortense de Calbiac, Paris (ICM)**

Pur-alpha provides a potential link between RNA toxicity and loss-of-
function in C9orf72 ALS
Bart Swinnen, Leuven

Inosine supplementation bypasses adenosine deaminase deficiency in C9orf72 astrocytes increasing bioenergetic capacity and motor neurone survival
Scott Allen, Sheffield

17.00-17.45 The ENCLS debate: This house believes ALS is a prion-like disease

Chair: Martin Turner, Oxford

FOR: Magdalini Polymenidou, Zurich (15 minutes)

AGAINST: Simon Mead, London (UCL) (15 minutes)

DISCUSSION (15 minutes)

17.45.19.15 POSTERS

20.00 CONFERENCE DINNER, KEBLE COLLEGE
FRIDAY 22ND JUNE

SESSION 7  Chair: Kevin Talbot, Oxford

09.00-9.30  ENCALS Awards

09.30-10.00  Prospects for genetic therapies in neurodegenerative disorders
Matthew Wood, Oxford

10.00-10.30  Clinical trials in ALS: stratification and personalised therapeutics
Angela Genge, Montreal

10.30-11.00  BREAK

11.00-12.45  Implementing evidence-based methods in amyotrophic lateral sclerosis
clinical trials
Ruben van Eijk, Utrecht

  Optimization of Preclinical Nucleic Acid based Therapeutic for the Most
Common Genetic Form of Amyotrophic Lateral Sclerosis
Helene Tran, Massachusetts (U Massachusetts)

  WVE-3972-01, an Investigational Stereopure Antisense Oligonucleotide,
Preferentially Knocks Down G4C2 Repeat-Containing C9ORF72 Transcripts
Jean-Cosme Dodart, Massachusetts (WAVE Lifesciences)

  G-quadruplex-binding small molecules ameliorate C9orf72 amyotrophic
lateral sclerosis/frontotemporal dementia pathology in iPSC neurons and in
vivo
Rubika Balendra, London (UCL)

  AAV vectors for ALS treatment and modelling
Maria Grazia Biferi, Paris (IM)

  An open-label trial of Triumeq in patients with ALS
Julian Gold, Sydney

  Randomized Phase 2B trial of NP001, a Novel Immune Regulator, in ALS
Jonathan Katz, San Francisco

12.45-13.00  INTRODUCTION TO ENCALS 2019 AND CLOSE OF MEETING