

# 33rd international Symp@Slum on ALS/MND



Organised by the Motor Neurone Disease Association in co-operation with the International Alliance of ALS/MND Associations



virtual 33rd international SYMD@SIUM on ALS/MND

### Organiser of the Symposium:



#### **Motor Neurone Disease Association**

Francis Crick House, 6 Summerhouse Road Moulton Park, Northampton NN3 6BJ

### Held in co-operation with:



INTERNATIONAL ALLIANCE OF ALS/MND ASSOCIATIONS

#### The International Alliance of ALS/MND Associations Email: alliance@als-mnd.org

Website: www.alsmndalliance.org

### Foreword

Welcome to the 33rd annual symposium on ALS/MND, which for the third year in a row is being held online.

In the Harry Potter stories, Harry begins with no knowledge of his magical abilities, but as time goes on, he discovers how to wield them and use them to achieve his ends. Our understanding of ALS has followed a similar trajectory. We started with very little insight as to why ALS happens or how to intervene to stop it, but bit by bit we are making progress through science, building on our knowledge to develop new tools to find significant therapies. Initially, Harry could only use one spell at a time to defend his pals, but as his knowledge improved, he was able to simultaneously use many. Similarly, for many years we have had only one or two clinical trials to offer our patients at any one time, but now we have multiple trials, using varied approaches. Like Harry, we are achieving this progress with the help of many others, all working to the same goal – and we can see our friends and colleagues in this international symposium, bringing together researchers from across the globe.

Although the buzz and benefits of an in-person meeting cannot be replicated by a remote conference, we have done our best to mitigate and to capture that feeling as far as possible. A virtual symposium also means that people who would otherwise have difficulty attending are able to participate. This year, we showcase exciting advances in research through the entire pathway from discovery science to translation into meaningful treatments.

A major theme is neuroinflammation, its role in sporadic and familial ALS and the outcomes of therapies to tackle it. The design of clinical trials, and the outcome of a number of different approaches to treatment are also two big subjects. They are likely to feature more heavily over the next few years.

Genetics and other risk and modifier factors are always a major focus, but bioinformatics and AI are featuring more and more, as conventional methods for identifying genetic variants and environmental factors that influence ALS and ALS risk are exhausted.

Three more prominent and increasingly important topics this year are the role of metabolism in ALS, the role of RNA and protein processing, and the modelling of non-genetic ALS in the laboratory, something that has been challenging and largely missing from our armoury of research tools.

The International Symposium on ALS/MND is a major annual event for the ALS community, bringing scientists and clinical practitioners together to evidence the progress being made to cure ALS/MND. I wish you a very informative and enjoyable meeting.

### Ammar Al-Chalabi

Programme Committee Chair

### Tuesday 6 December 2022

#### Please note that all timings are GMT

### SESSION 1

### **OPENING SESSION**

Chairs: B Dickie (UK) A Al-Chalabi (UK)

13.45 – 13.50	Welcome – B Dickie (UK) and A Al-Chalabi (UK)	
13.50 – 14.35	The Stephen Hawking Memorial Lecture Introduction	L Hawking (UK,
	<b>C01</b> The contribution of aging to neurodegenerative disease	F Gage (USA)
14.35 – 14.45	Discussion/Questions	
14.45 – 14.55	International Alliance Humanitarian Award	
	Forbes Norris and Humanitarian Awards	

### 15.10 – 15.40 ICEBREAKER SESSION

### **SESSION 2**

### **CLINICAL TRIALS**

Chairs: K Talbot (UK) B Dickie (UK)

1540 – 1600	<b>C02</b> Results from a randomized, double-blind, placebo-controlled trial of RNS60 in people with ALS	E Pupillo (Italy)
1600 – 1620	<b>C03</b> Modifying immune response and outcomes in ALS (MIROCALS): design and results of a phase 2b, double-blind randomized placebo- controlled trial of low dose interleukin-2 (Id IL2) in ALS	G Bensimon (France)
1620 – 1630	<b>C04</b> Evidence for a survival benefit in ALS with CNM-Au8 treatment: interim results from the RESCUE-ALS trial long-term open extension	S Vucic (Australia)
1630 – 1640	<b>C05</b> Evaluating efficacy and safety of Tofersen in adults with SOD1 ALS: Results from the Phase 3 VALOR trial and open label extension	T Miller (USA)
1640 – 1655	Discussion/Questions	
16.55 - 17.05	BREAK	

### LIVE POSTER SESSION A

#### 17.05 - 18.35 GMT

**EPI-01 Frequency of mutations in C9orf72, SOD1, FUS, and TARDBP genes among amyotrophic lateral sclerosis cases: a review and meta-analysis** Ms Julie Barberio

**EPI-02 Lessons Learned from Survival Modeling in an Expanded Access Protocol** Mr Alex Berger

**EPI-03 The Canadian Neuromuscular Disease Registry: A national ALS registry for real world evidence** Dr Victoria Hodgkinson

EPI-04 Detection of spatial association

between ALS and airborne lead (Pb) in Ohio

Dr Meifang Li

**GEN-01 A case of an uncommon late onset of ALS associated with an undescribed SOD1 mutation** Dr Lucrezia Becattini

**GEN-02 A novel mutation in ZFYVE26 gene (c.195-1G>A) in a young manaffected by spastic paraplegia** Dr Alessandro Bombaci

**GEN-03 Genetic testing of people with early-onset MND in the West of Scotland** Dr Fraser Brown

GEN-04 The Role of Nicotinic Receptor Genes (CHRN) in Amyotrophic Lateral Sclerosis

Dr Filippo De Mattei

**GEN-05 WITHDRAWN** 

**GEN-06 Genetics in ALS: A change in attitude, no change in results.** Dr Ana Sanchez Fernandez

**GEN-07 A Genome-wide association study on the New England and Ohio Amyotrophic Lateral Sclerosis Cohort** Mr Jiang Gui

GEN-08 Unraveling Clinico-Pathological Heterogeneity in Motor Neuron Diseases using Long-Read Sequencing Ms Angita Jain

IVT-01 Superoxide Dismutase-1 Alters the Rate of Prion Protein Aggregation and Resulting Fibril Conformation

Miss Kamile Mikalauskaite

IVT-02 Investigating variants in NUP50 as risk factors for Amyotrophic Lateral Sclerosis Ms Olga Roman

IVT-03 Effect of complement C3 inhibition in an iPSC-derived ALS neuromuscular junction model of neuroinflammation

Dr Scott Baver

IVT-04 Characterization of PPIA/ EMMPRIN Pathway in Familial Models of Amyotrophic Lateral Sclerosis. Mrs Gloria Nwamaka Edozie

**IVT-05 Cellular function of hnRNP A1B, a longer HNRNPA1 isoform** Miss Mariana Llasera Ballester

IVT-06 The interactors of FUS Dr Helena Motaln

IVV-01 Mapping PHD1-mediated neuroprotection in mutant SOD1 ALS mice at single-cell resolution. Ms Christine Germeys

IVV-02 Sympathetic neurons are additional cell types affected in Amyotrophic Lateral Sclerosis Mr Antonio Mazzaro

IVV-03 Novel insights on the role and therapeutic potential of Glycoprotein nonmetastatic melanoma protein B(Gpnmb) in Amyotrophic Lateral

**Sclerosis.** Dr Mauro Giuseppe Spatafora

IVV-04 Investigating ASO therapeutic strategies in targeting the ALS-causing FUSR521H variant using zebrafish Mr Rampal Christian

IVV-05 Zebrafish TDP-43 knock-in CRISPR mutants develop a robust ALS-like phenotype independent of TDP-43 mislocalization

Mr Ziyaan Harji

IVV-06 Loss of C9orf72 induces neurodegeneration and autophagic deficits in a mouse model of TDP-43 proteinopathy

Ms Lilian Lin

IVV-07 Variants in NEMF, a ribosomal quality control protein, harbored in ALS patients cause motor phenotypes in mice Ms Jennifer Stauffer HCB-01 Intracellular transport and mitochondrial bioenergetics are reduced in iPS-derived motor neurons from ALS patients with TDP-43 mutations Dr Ruxandra Dafinca

HCB-02 Formyl Peptide Receptor-Like 1 Depleted Monocytes in Amyotrophic Lateral Sclerosis Mr Guy Hunt

HCB-03 Phosphorylation of Y526 FUS in FTD Professor Boris Rogelj

HCB-04 ALS fibroblasts derivedexosomes increase wound healing Mr Vincent Clément

HCB-05 Human motor neurons with mutations in TARDBP (TDP-43) derived from gene-edited iPSCs recapitulate features of ALS in vitro Ms Sarah Lépine

HCB-06 A C-terminally truncated TDP-43 splice isoform exhibits neuronal specific cytoplasmic aggregation and contributes to TDP-43 pathology in ALS Mr Marc Shenouda

#### **BIO-01 WITHDRAWN**

BIO-02 Creatinine Kinase-MB as a Complementary Biomarker in the Evaluation of Patients with Amyotrophic Lateral Sclerosis

Dr Sergio Castro-Gomez

**BIO-03 Chitinase dysregulation predicts ALS disease aggressiveness and stems from multiple CNS populations** Ms Nayana Gaur

**BIO-04 Blood Neutrophil-to-Lymphocyte ratio predict Survival in ALS patients** Miss Diana Monteiro Lopes

TST-01 Nicotinamide riboside, pterostilbene and ibudilast delay ALS progression in murine models Professor Elena Obrador

TST-02 Combination therapy approaches for the treatment of Amyotrophic Lateral Sclerosis

Mrs Megan Baird

TST-03 How Much Do Patients Know About Basic Research? A Survey of Knowledge and Preferences Among People Living with ALS and Other Neurological Conditions

Professor Danielle Boyce

TST-04 Development of an UNC13A cryptic exon skipping antisense oligonucleotide as a treatment for ALS and FTD

Dr Wen-Hsuan Chang

TST-05 Small Molecule Hepatocyte Growth Factor (HGF)/MET Positive Modulator ATH-1105 is Neuroprotective in the TDP-43 Mouse Model of Amyotrophic Lateral Sclerosis Dr Kevin Church

TST-06 Discovery and Development of Inhibitors of 15-Lipoxygenase to Reduce Ferroptosis-Induced Injury for ALS and Neurodegenerative Diseases Mr Jeff Trimmer

IMG-01 Motor Imagery in Amyotrophic Lateral Sclerosis: an fMRI study of postural control Ms Malek Abidi

IMG-02 Loss of brainstem white matter predicts onset and motor neuron symptoms in C9orf72 expansion carriers: a GENFI study

Mr Sergi Borrego - Écija

IMG-03 Brain-age predicts survival in ALS Professor Andreas Hermann

CLT-01 Independent Expert Review of Cardiac Adverse Events and Electrocardiographic Abnormalities and Parameters in a Phase 2 Trial of Sodium Phenylbutyrate and Taurursodiol in Amyotrophic Lateral Sclerosis (CENTAUR) Dr Jamie Timmons

**CLT-02 Tegoprubart (AT-1501) is safe and** well tolerated and reduces inflammation in patients with ALS Dr Jeffrey Bornstein

CLT-03 Clinical Trial Operations for the HEALEY ALS Platform Trial

Ms Marianne Chase

CLT-04 A variant-selective approach in C9orf72-associated amyotrophic lateral sclerosis (ALS) and frontotemporal

sclerosis (ALS) and frontotemporal dementia (FTD): Results from the FOCUS-C9 clinical trial Dr Kenechi Ejebe

CLT-05 Longitudinal comparison of the self-entry amyotrophic lateral sclerosis functional rating scale-revised (ALSFRS-RSE) and rasch-built overall amyotrophic lateral sclerosis disability scale (ROADS) as outcome measures in people with amyotrophic lateral sclerosis

Dr Stephen Johnson

CLT-06 Wearable and smartphone devices can track ALS disease progression and may serve as novel clinical trial outcome measures

Dr Marta Karas

**CLT-07 Expanded Access to Sodium Phenylbutyrate and Taurursodiol Coformulation in Amyotrophic Lateral Sclerosis: Updates and Initial Learnings** Mr Philip Green

CLT-08 QRL-201-01 - A multi-center, randomized, double-blind, placebocontrolled multiple-ascending dose study to evaluate the safety and tolerability of QRL-201 in amyotrophic lateral sclerosis

Ms Kristiana Salmon

CLT-09 The Integrated Stress Response is modulated by eIF2B agonist DNL343: Results from Preclinical, Phase 1 Healthy Subject and Interim Phase 1b ALS Patient Studies

Dr Linus Sun

**DSP-01 WITHDRAWN** 

DSP-02 Changes in objective temporal speech measures during ALS-FRS-R plateau phases Dr Liziane Bouvier

**DSP-03 Longitudinal Changes to Communicative Participation in ALS** Dr Kathryn Connaghan

**DSP-04 Approaches to Evaluating Speech Severity of Remote Speech Recordings** Dr Abigail Haenssler

DSP-05 The ALS CURE Project 2022 Roadmap to Cure ALS : an approach to enable international research collaboration Mr Mike Piscotty

DSP-06 Exploring the analytical validity of the Winterlight assessment app – a novel, home-based speech assessment tool – in ALS

Dr Leif Simmatis

COG-01 Making Sense:An online Meaning Centred Psychological intervention for individuals with MND

Mrs Sandra de Moree

COG-02 Clinicoanatomical substrates of selfish behaviour in amyotrophic lateral sclerosis – an observational cohort study Professor Dorothee Lule COG-03 A novel EEG paradigm to quantify the cortical network dysfunction underpinning verbal fluency impairments in ALS Ms Serena Plaitano

CMS-01 IMPACT ALS EUROPE SURVEY -People living with ALS in Europe Miss Éilís Conroy

CMS-02Understanding family experiences of inherited MND: qualitative interview study and new resource on healthtalk.org

Miss Jade Howard

CMS-03 A multi-centre evaluation of post-gastrostomy management in motor neuron disease (PostGas) to understand why patients often experience poor nutritional outcomes following placement of a feeding tube Miss Lucy Musson

CMS-04 Critical Resources for Underserved Veterans with ALS -Combating Deserts of Care Mrs Mandi Bailey

CMS-05 CBAS: Ongoing Data Collection & Validation

Professor Laura Ball

CMS-06 At-Home Telespirometry (AHT) In Amyotrophic Lateral Sclerosis (ALS) Implementation Science: Evidence-Based Development of Vital Capacity (VC) Measurement Frequency Algorithm for Clinical Testing

Professor Benjamin Rix Brooks

#### **CMS-07 WITHDRAWN**

CMS-08 Proximity to UCSF ALS Satellite Clinics Increases Rural Patient Access and Clinic Visit Frequency, But Disparities Remain

Dr Jill Goslinga

CMS-09 Equipment for Living with ALS: Results from the ALS Focus Mobility Survey

Dr Sarah Parvanta

### Wednesday 7 December 2022

### LIVE POSTER SESSION B

#### 12.15 - 13.45 GMT

EPI-05 A Review of the Efficacy of **Edaravone and the Factors That Influence its Response in Patients With Amyotrophic Lateral Sclerosis** Miss Maleesha Jayasinghe

#### **EPI-06 WITHDRAWN**

EPI-07 'He's been brave for me and I've been brave for him' meaning in life and personal satisfaction as positive aspects for ALS caregivers – A longitudinal mixed methods analysis

Miss Éilís Conroy

**EPI-08 Exposure to electromagnetic** fields does not modify neither the onset age nor the disease progression in ALS patients

Dr Margherita Daviddi

#### **GEN-09 WITHDRAWN**

GEN-10 Study into a possible common founder effect for the SOD1 p.V48A mutation amongst Malaysian Chinese and Mainland Chinese ALS patients Miss Suzanna Edgar

GEN-11 Comprehensive genetic study of genes that alter NAD+ levels in Australian sporadic amyotrophic lateral sclerosis Dr Jennifer Fifita

**GEN-12 Prediction of pathogenicity of** ALS-causing gene missense variants using structural information predicted by AlphaFold2

Mr Yuya Hatano

GEN-13 Sex-stratified analysis of ~133k samples identifies novel associations with Amyotrophic Lateral Sclerosis Dr Ross Byrne

GEN-14 Whole-genome sequencing reveals that variants in the IL18RAP 3'UTR protect against ALS Dr Chen Eitan

**GEN-15 Genetic analysis of ALS in Russia** Miss Alina Korbut

**GEN-16** Genome-wide association study of rare variants in ERV-elements of ALS patients: age at onset of ALS may be regulated by MLT1B ERV-element. Dr Alexey Shatunov

IVT-07 Dysregulation of SFPQ in motor neuron disease Dr Alison Hogan

IVT-08 Dysregulation of cellular energy metabolism and axonal transport in TDP-43 M337V mESC-derived motor neurons Miss Emily Carroll

IVT-09 TBK1 kinase inhibition is associated with cell autonomous microglial dysfunction Miss Uroosa Chughtai

IVT-10 Mechanistic insights into the cause of DNA damage accumulation in cells bearing TDP-43 and FUS cytoplasmic inclusions

Miss Stefania Modafferi

IVT-11 Characterization of iPSCmotoneurons from a HSP patient with a novel mutation in KIF5A N-terminal region

Dr Serena Santangelo

IVV-08 Synergistic activity of a nervemuscle, multimodal combination therapy in mutant SOD1 mice

Professor Peter Crouch

IVV-09 Longer telomeres are present in a mouse model of amyotrophic lateral sclerosis displaying TDP-43 pathological features

Dr Anna Konopka

IVV-10 The Serum Response Factor (SRF) regulates motoneuron vulnerability in ALS through the regulation of autophagy flux

Miss Natalie Yashoda Dikwella

IVV-11 The increased expression of neuregulin-1 type III in neurons in double transgenic mice does not improve ALSrelated pathology or extend the lifespan of SOD1G93A mice

Dr Sara Hernández

IVV-12 Exploring the clinical relevance of compound muscle action potential data in the SOD1G93A mouse

Mr Scott McKinnon

IVV-13 Axonal transport of diverse cargoes in fast and slow α-motor neurons in multiple ALS mice Dr Andrew Tosolini

HCB-07 Evaluating models of Motor Neuron Disease molecule by molecule. Dr Dezerae Cox

HCB-08 Immunological characterization of spinal cord organoids derived from sALS patients Dr Matteo Bordoni

HCB-09 ALS/FTD-associated C9orf72 C4G2 repeat RNA binds to FARS protein and affect the rate of phenylalaninetRNA aminoacylation Ms Urša Čerček

HCB-10 RNA G-quadruplex structures regulate stress granule dynamics Mr Yehuda-Matan Danino

**HCB-11** Towards a quantitative protein atlas of human iMN and their extracellular vesicles Dr Raquel Mesquita-Ribeiro

HCB-12 HuD gain-of-function leads to disruption of the neuromuscular junction and apoptosis in FUS-ALS iPSC-derived muscle-nerve co-cultures Professor Alessandro Rosa

#### **HCB-13 WITHDRAWN**

BIO-05 The first evaluation of calretinin in the spinal axons encompassed in anterolateral funiculi outside the corticospinal tract and cerebrospinal fluids of ALS patients Dr Shintaro Hyashi

#### **BIO-06 WITHDRAWN**

**BIO-07 Benchmarking Proteome of Extracellular Vesicles From Plasma Of** fALS-Associated SOD1 Mutant Patients And SOD1 Mutant Healthy Controls Dr Aslihan Gunel

BIO-08 First evidence of altered Gas6-Axl signaling and correlation between sAXL blood levels and clinical decline in ALS Dr Marco Peviani

BIO-09 Premorbid primary care blood biomarker alterations support a metabolic prodrome in amyotrophic lateral sclerosis

Dr Alexander Thompson

TST-07 Inhibition of glucosylceramides improves cell viability and TDP-43 mislocalisation in ALS iPSC-LMNs Miss Leanne Jiang

TST-08 Investigating novel gene therapy approaches to target RAN-translation of pathological C9ORF72 repeat transcripts in ALS/FTD

Miss Bridget Benson

TST-09 The identification of SCFV'S that bind to TDP-43 and prevent its induced aggregation as a potential therapy for ALS

Professor Hélène Blasco

TST-10 The feeding behaviour of Amyotrophic Lateral Sclerosis mousemodels is modulated by the Ca2+activated KCa3.1 channels

Mrs Germana Cocozza

TST-11 Novel functionalized nanoparticles targeted to 18KDa translocator protein (TSPO) to track and modulate neuroinflammation in animal models of familial Amyotrophic Lateral Sclerosis

Mr Andrea Gazzano

TST-12 Effects of therapeutic hypothermia in an animal model of Amyotrophic Lateral Sclerosis. Dr Laura Pasetto

TST-13 VecTabs<sup>®</sup> and advanced human in-vitro models: targeting TDP-43 aggregates and oxidized phosphocholines in Amyotrophic Lateral Sclerosis

Dr Andreia Duarte

#### **IMG-04 WITHDRAWN**

IMG-05 A monocentric case series of Hirayama's disease: clinical, neurophysiological, imaging features and differences with Amyotrophic Lateral Sclerosis.

Dr Sara Cabras

IMG-06 Clinical utility of structural and diffusion brainstem analysis in predicting bulbar and respiratory dysfunction. Dr Mohammed Khamaysa

**CLT-10 EPI-589 early Phase 2 Investigatorinitiated Clinical trial for ALS (EPIC-ALS)** Dr Shotaro Haji

**CLT-11 TUDCA as disease modifier for ALS: time will tell truth** Dr Antoniangela Cocco CLT-12 First in human phase 1/2a study evaluating the safety and efficacy of the Akt activator, IPL344 in ALS (NCT03652805, NCT03755167). Professor Marc Gotkine

CLT-13 Design of an international, phase 3, randomized, placebo-controlled trial with daily oral edaravone (fnp122) in als: the adore study

Dr Ruben van Eijk

CLT-14 Remote monitoring of accelerometry for amyotrophic lateral sclerosis detects differences in disease progression and survival

Mr JWJ van Unnik

CLT-15 Shifting the Paradigm – PrimeC: A Potential Disease-Modifying Treatment for ALS Driven by Novel Biomarkers Measuring Mechanism of Action Dr Shiran Zimri

#### **DSP-07 WITHDRAWN**

DSP-08 Protein structure based FUS mutational subtypes are linked with subcellular mislocalization and age at onset in amyotrophic lateral sclerosis Mr Wanli Yang

DSP-09 When rare gets rarer: five reports of association between ALS and other uncommon conditions from a large cohort of patients in Central Italy Dr Francesca Bianchi

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**DSP-10 A patients with ALS and TBK1 loss of function mutation: a case report.** Dr Lorenzo Fontanelli

DSP-11 Phenotype-specific gene expression patterns identification through transcriptome-phenotype investigation in unmutated sporadic ALS patients

Dr Maria Garofalo

DSP-12 Traditional Methods of Quantifying Upper Limb Function in ALS and the Need for Objective Solutions Mr Conor Hayden

DSP-13 Defining mild cognitive and behavioural impairment in presymptomatic ALS/FTD Dr Caroline McHutchison

DSP-14 Relevance of respiratory tests in determining respiratory involvement in ALS

Dr Susana Pinto

**COG-04 Premorbid psychological resilience is protective against cognitive deterioration in motor neuron diseases** Dr Pilar Maria Ferraro

**COG-05 Quantifying network dysfunction underpinning social cognition impairments in ALS with EEG** Dr Roisin McMackin

**COG-06 Experiences, coping strategies** and support needs of family caregivers of **ALS-patients post-bereavement** Mrs Sabina Pujic

COG-07 Cognition in the Course of ALS – a Meta-Analysis Mrs Julia Finsel

CMS-10 Utility of three-dimensional (3D) motion capture in monitoring disease progression in amyotrophic lateral sclerosis (ALS) Ms Akiko Goto

**CMS-11 Robot-assisted training using HAL ameliorates gait ability in patients with amyotrophic lateral sclerosis** Miss Harumi Morioka

CMS-12 Satisfaction with healthassociated support system in Germany and Poland from the perspective of patients with amyotrophic lateral sclerosis, their caregivers and physicians Dr Krzysztof Barć

**CMS-13 Paraneoplastic motor neuron disease with anti-CV2 antibodies** Sra Maria Dolores Calabria Gallego

**CMS-14 The role of maximal voluntary ventilation in evaluating respiratory function of ALS patients** Professor Mamede Alves De Carvalho

CMS-15 Making her end of life her own: further reflections on supporting a loved one with motor neurone disease Dr Denise Harris

CMS-16 Feasibility and acceptability of the real-world implementation of Telehealth in Motor Neuron Disease (TiM) service in two specialist MND centres. Dr Liam Knox

CMS-17 'Gastrostomy Tube: Is it for Me?' Development and pilot testing of a webbased patient decision aid for people with motor neurone disease considering a gastrostomy tube (DiAMoND Study) Dr Sally Wheelwright

13.45 - 14.00 BREAK

### **SESSION 3**

### **METABOLISM**

Chairs: B Dickie (UK) A AI-Chalabi (UK) **PARALLEL SESSION** 14.00 - 14.25 **C06** Investigating the role of hypermetabolism in *S Ngo (Australia)* 14.00 - 14.45 ALS Sponsored Parallel Session 1 (non-CME) 14.25 - 14.40 **C07** Dysregulated energy metabolism related to J He (China) worse prognosis in ALS 14.40 - 14.55 **C08** An 18F-FDG-PET study exploring the F Di Pede (Italy) metabolic signature of pure bulbar and pure spinal ALS 14.55 - 15.10 Discussion/Questions

15.10 – 15.40 NETWORKING EVENT

### SESSION 4

CELL BAS	SED MODELS		
Chairs: G Tyzak (	UK) N Cole (UK)		PARALLEL SESSION
15.40 – 16.05	<b>C09</b> iPSC-derived models for compound screening and the identification of new therapeutic strategies	K Roet (USA)	<b>15.40 – 16.25</b> Sponsored Parallel Session 2 (non-CME)
16.05 – 16.20	<b>C10</b> Sporadic ALS motor neurons recapitulate neuronal vulnerability	B Turner (Australia)	
16.20 – 16.35	<b>C11</b> Reduced C9orf72 expression exacerbates poly-GR toxicity in patient iPSC-derived motor neurons	T Dane (USA)	
16.35 – 16.50	Discussion/Questions		

16.50 – 17.05 BREAK

**C12** Identifying risk factors using Mendelian

C13 Traumatic Brain Injury as a risk factor for

C14 Mild motor impairment and prodromal

### **SESSION 5**

17.05 - 17.25

17.25 - 17.45

17.45 - 18.05

18.05 - 18.20

### **DISEASE PRESENTATION AND RISK**

neurodegenerative disease

Discussion/Questions

Randomization

Chairs: A Thompson (UK) A Al-Chalabi (UK)

markers

### PARALLEL SESSION

17.05 – 17.50
Sponsored Parallel
Session 3 (non-CME)

Gabriel Benois



G Davey Smith

M Benatar (USA)

C Tartaglia

(Canada)

(UK)

### Thursday 8 December 2022

### **SESSION 6**

### **GENETICS AND GENOMICS**

Chairs: C Vance (UK) A Al-Chalabi (UK)

<b>C15</b> Decoding gene regulatory networks in ALS	J Kirby (UK)	<b>14.00 – 14.45</b> Sponsored Parallel Session 4 (non-CME)
<b>C16</b> Unsupervised hierarchical clustering of postmortem motor cortex expression data identifies distinct molecular phenotypes which replicate the major mechanisms of ALS pathogenesis	H Marriott (UK)	
<b>C17</b> Interactions between genetic modifiers of survival in ALS: a population based study	A Chio (Italy)	-
Discussion/Questions		-
	<ul> <li>C16 Unsupervised hierarchical clustering of postmortem motor cortex expression data identifies distinct molecular phenotypes which replicate the major mechanisms of ALS pathogenesis</li> <li>C17 Interactions between genetic modifiers of survival in ALS: a population based study</li> </ul>	C16 Unsupervised hierarchical clustering of postmortem motor cortex expression data identifies distinct molecular phenotypes which replicate the major mechanisms of ALS pathogenesis <i>H Marriott (UK)</i> C17 Interactions between genetic modifiers of survival in ALS: a population based study <i>A Chio (Italy)</i>

**PARALLEL SESSION** 

#### 15.10 – 15.25 BREAK

### **SESSION 7**

### **RNA AND PROTEIN PROCESSING**

Chairs: M Hallegger (UK) N Cole (UK)

15.25 – 15.50	<b>C18</b> Protein chaperones as a therapeutic target for ALS	H Durham (Canada)
15.50 – 16.15	<b>C19</b> MicroRNA dysregulation in motor neuron disease	E Hornstein (Israel)
16.15 – 16.30	<b>C20</b> Diminished miRNA activity is associated with aberrant cytoplasmic intron retention in ALS pathogenesis	H Crerar (UK)
16.30 – 16.45	Discussion/Questions	

16.45 – 17.00 BREAK

### LIVE POSTER SESSION C

#### 17.00 - 18.30 GMT

EPI-09 Kidney Function (eGFR MDRD) in **ALS Patients** 

Mr Matteo Locatelli

**EPI-10 Poliomyelitis Survivors in the Time** of a Covid-19 Pandemic

Miss Jeyce Nogueira

**EPI-11 Estimates of C9orf72 Statistics** drawn from prior publications, including estimated Life Expectancy for Genetic **Carriers, Estimated Incidence of C9orf72** ALS and FTD Diagnoses, Estimated **Genetic Carrier and At Risk Population** and others.

Ms Jean Swidler

EPI-12 Early initiation of riluzole may improve absolute survival in amyotrophic lateral sclerosis

Dr Nimish Thakore

**GEN-17 Fine-tuning a molecular** diagnosis algorithm for patients with ALS and FTD

Dr Daniel Borrego Hernández

#### GEN-18 Exploring the role of HERV-K integrations in amyotrophic lateral sclerosis

Mr Harry Bowles

#### GEN-19 SOD1 D91A variant in the southernmost tip of Europe: a heterozygous ALS patient resident on the island of Gozo

Professor Ruben Cauchi

GEN-20 Allele genotype of a SINE-VNTR-Alu (SVA) retrotransposon correlates with differential gene expression at the MAPT locus

Mr Alexander Fröhlich

GEN-21 Utilising genetic data and quality of life metrics within the TONiC initiative to better predict patient outcome, progression and severity in ALS Dr Ben Middlehurst

**GEN-22** Targeted long-read sequencing of C9orf72 in blood of patients with ALS Mr Evan Udine

#### **GEN-23 WITHDRAWN**

IVT-12 iPSC-derived motor and sensory neurons have distinct molecular profiles but share axonal vulnerability due to the C9orf72 hexanucleotide repeat expansion Dr Jakub Scaber

IVT-13 Characterization of PPIA/ **EMMPRIN** pathway in the peripheral nervous system of SOD1G93A model of **Amyotrophic Lateral Sclerosis** Miss Marion Boyer

**IVT-14 Using PRMT Inhibition to Abrogate Dipeptide Repeat Protein Toxicity in** C9orf72-Mediated ALS: A Proteomics Assessment of Post-Translational **Modifications** 

Ms Anna Gill

IVT-15 Traumatic injury exacerbates neuropathology in C9orf72 ALS/FTD motor neurons Mr. Fric Martin

**IVT-16 Effects of arginine methylation** state on the subcellular localization of C9orf72 repeat expansion associated toxic dipeptide repeat proteins. Ms Kaly Mueller

IVV-14 Cytoplasmic accumulation of FUS in adult neurons prevents establishment of social memory but preserves motor function

Dr Luc Dupuis

IVV-15 Imaging the spinal cord neurodegeneration of the TDP-43-A315T ALS mouse model: relationship between MRI and TDP-43 aggregates Dr Débora Lanznaster

IVV-16 Alteration of the neuromuscular junction and modifications of muscle metabolism in response to neuron-restricted expression of the **CHMP2BINTRON5** mutant in a mouse model of ALS-FTD syndrome Dr Frederique Rene

**IVV-17 Development of a zebrafish** Stathmin-2 (STMN2) knockout model

Mr Tyler Gurberg

IVV-18 C57BL/6J-Ighmbp2EM6 displays respiratory and neuromuscular characteristics of very severe SMARD1 Miss Sarah Holbrook

IVV-19 Is ALS onset accelerated by Chloroviruses and delayed by antiviral immune responses? Dr Gary Pattee

**HCB-14 Alterations in the interactome** of wild-type TDP-43 and mutant TDP-43 lacking nuclear localization signal Ms Jerneja Nimac

HCB-15 Crosstalk between NEAT1\_1 IncRNA and TDP-43 in ALS Dr Tatyana Shelkovnikova

HCB-16 Dynamic Expression Profiles of Stressed iPSC-MNs from C9orf72-ALS Patients by Translating Ribosome Affinity Purification (TRAP) Dr Yinyan Xu

HCB-17 Discovery of novel small molecule Cu(II) complexes using a genome edited iPSc-based phenotypic screening platform Dr Kyle Denton

HCB-18 SIRT1 is associated with the alleviation of DPR pathology in C9orf72associated disease Ms Sophie Imhof

HCB-19 Stabilizing Microtubules Rescues Disrupted Nucleocytoplasmic Transport in Loss-of-Function Models of the ALS Gene NEK1

Dr Jacob Mann

HCB-20 Proteome-Wide Degradation Dynamics in ALS SOD1 Patient Neurons **Reveals Disrupted VCP Homeostasis** Dr Konstantinos Tsioras

**BIO-10 Integrated deep proteomics** of cerebrospinal fluid for biomarker identification in ALS Dr Elizabeth Dellar

**BIO-11 Inflammasome genes expression** as biomarkers in ALS and FTD patients Miss Laura Expósito Blázquez

BIO-12 Characterisation of the role of the gut-brain axis in the pathophysiology and progression of motor neuron disease Miss Iman Geelani Khwaja

**BIO-13 Erythrocyte membrane properties** in patients with ALS Dr Ana Catarina Pronto Laborinho

#### **BIO-14 WITHDRAWN**

TST-14 A Retrospective Analysis of **Dietetic Input Received by Patients** with Motor Neurone Disease (MND) and Dietetic Workload when Caring for the MND Population across Varied National Health Service Settings 2019/2020 Ms Justvna Reinert

TST-15 Engrailed-1 homeoprotein is a non-cell autonomous neurotrophic factor for motoneurons

Dr Stephanie Elizabeth Vargas Abonce

TST-16 QRL-201: A Stathmin-2 spliceswitching antisense oligonucleotide for the treatment of ALS Ms Taylor Gray

**TST-17 Preclinical Characterization** of Prosetin, A Novel, Brain-Penetrant, **Clinical-Stage MAP4K Inhibitor for** the Treatment of Amyotrophic Lateral Sclerosis

Dr Emily Lowry

#### TST-18 Targeting the NFkb pathway as a therapeutic strategy for stabilizing dendritic structure in an ALS-FUS mouse model

Mrs Mari Carmen Pelaez

**TST-19 Hydrogen Peroxide and Amyotrophic Lateral Sclerosis: From Biochemistry to Pathophysiology** Mr Nitesh Sanghai

TST-20 Targeting neuregulin1 on microglia to slow or stop ALS Professor Fei Song

TST-21 Development of TDP-43 inhibitors for the treatment of ALS, FTD, and Alzheimer's disease Dr Chengliang Zhang

IMG-07 Dysfunction of cortical inhibitory

interneurons In Amyotrophic Lateral **Sclerosis** 

Mrs Cristina Benetton

IMG-08 Role of brain 18Fluorodeoxyglucose-Positron-Emission Tomography as survival predictor in **Amyotrophic Lateral Sclerosis** Dr Antonio Canosa

IMG-09 Brain sensorimotor integration after focal muscle-tendon vibrations in amyotrophic lateral sclerosis Mr Arnaud Preuilh

CLT-16 Interim Analysis of the Radicava/ Edaravone Findings in Biomarkers from ALS (REFINE-ALS) Study Dr James Berry

CLT-17 Using Active Digital Phenotyping to Quantify Function and Speech in **Amyotrophic Lateral Sclerosis (ALS)** Dr Katherine Burke

**CLT-18 The HEALEY ALS Platform Trial: Results of Regimens A-D** Dr Merit Cudkowicz

CLT-19 Phase 3, Open-Label, Multicenter Safety Study of Oral Edaravone in **Patients With Amyotrophic Lateral** Sclerosis (MT-1186-A01): 48-Week Results Dr Angela Genge

**CLT-20** Operationalizing meaningful participant engagement in ALS research: Early lessons from CAPTURE ALS Dr Wendy Johnston

CLT-21 Using asynchronous online focus groups to improve access to research for people affected by amyotrophic lateral sclerosis (ALS): A methodological exploration

Ms Westerly Luth

CLT-22 Phase 3b, Multicenter, Randomized, Double-Blind, Parallel-Group Study Evaluating Efficacy and Safety of Oral Edaravone Administered **Over 48 Weeks in Patients with Amyotrophic Lateral Sclerosis (MT-**1186-A02)

Dr Alejandro Salah

**CLT-23 ENCALS Predictive Survival Model** in FORTITUDE-ALS

**Dr Tyrell Simkins** 

CLT-24 Update on an International Phase **3 Trial Evaluating Sodium Phenylbutyrate** and Taurursodiol in Amyotrophic Lateral Sclerosis (PHOENIX)

Professor Leonard van den Berg

**DSP-15 WITHDRAWN** 

DSP-16 Interaction of gender and onset site in the progression of ALS - A multivariate disease modelling approach Ms Juliette Ortholand

DSP-17 Evaluating nQ as an objective biomarker to assess fine motor impairment in people with ALS Alison P. Clark

DSP-18 Wearable sensors for tracking activity patterns in patients with motor neuron disease Dr Andrew Geronimo

**DSP-19 Cepstral/Spectral Measures** of Dysphonia in Amyotrophic Lateral **Sclerosis** Mr Marc Maffei

**DSP-20 ALS Precision Medicine Program: Cohort Overview of a Comprehensive Natural History and Translational Research Study for the Identifcation of** ALS Therapeutic Targets, Biomarkers, and **Targetable Patient Subsets** Mr Alan Premasiri

DSP-21 At-Home Telespirometry (AHT) in **Amyotrophic Lateral Sclerosis** Dr Eufrosina Young

#### **COG-08 WITHDRAWN**

COG-09 Screening for visuospatial abilities in amyotrophic lateral sclerosis: a pilot study using the Battery for Visuospatial Abilities (BVA) Dr Minoo Sharbafshaaer

COG-10 People of Color living with and impacted by ALS and Community **Created Intervention** Miss Katrina Byrd

#### **COG-11 WITHDRAWN**

CMS-18 Food Consumption of Individuals with Amyotrophic Lateral Sclerosis Ms Maria Luisa do Nascimento Felipe

CMS-19 Patient perspectives on digital healthcare technology in care and clinical trials for motor neuron disease: An international survey Mr Jochem Helleman

CMS-20 Bringing health care closer to patients - two years of the home care ALS programme in Slovenia Dr Blaž Koritnik

CMS-21 A Telemedicine Approach to Goals of Care Discussions in Motor Neuron Disease

Mrs Paula Brockenbrough

CMS-22 ALS Community Support Assessment to Improve Online Access to Information and Resources in Italy Mr Silverio Conte

CMS-23 Educommunication for food and nutrition in Amyotrophic Lateral Sclerosis Sra Karla Coutinho

CMS-24 At-Home Telespirometry (AHT) in Amyotrophic Lateral Sclerosis (ALS): **Digital Health Technology and Quality** Control

Dr Bhavya Narapureddy

CMS-25 Eligibility, prescription, and dispensation of riluzole and Radicava(R) for newly diagnosed people with Amyotrophic Lateral Sclerosis (ALS): a single site retrospective chart review Ms Kelsey Tymkow

### Friday 9 December 2022

### LIVE POSTER SESSION D

#### 12.15 – 13. GMT

**EPI-13 Time interval between age at** retirement from soccer and the onset of **ALS in the Spanish league: a literature** review and meta-analysis. Dr Josep Gamez

EPI-14 The influence of agricultural areas on ALS risk and phenotype: a populationbased study

Dr Umberto Manera

EPI-15 Presymptomatic geographical distribution of patients with Amyotrophic Lateral Sclerosis: a population-based cluster analysis.

Dr Rosario Vasta

GEN-24 Reduced-penetrance Huntington's disease-causing alleles with 39 CAG trinucleotide repeats could be a genetic factor of amyotrophic lateral sclerosis

Dr Kangyang Jih

GEN-25 Discovering novel MND causal mutations through comprehensive assessment of complex genomic variant types

Dr Emily McCann

#### **GEN-26 WITHDRAWN**

GEN-27 Does familiarity for neurodegenerative diseases influence amyotrophic lateral sclerosis presentation? An exploratory clinical study.

Dr Marta Cillerai

**GEN-28 Neuropeptide signalling** processes perturbed in postmortem motor cortex of ALS patients Dr Renata Kabiljo

GEN-29 Facilitating Template Structure

Selection for in Silico Anaylses of ALS Proteins – A Comprehensive Dataset of All Available Experimentally Solved Wildtype and ALS Mutant Protein Structures

Miss Deborah Ness

**GEN-30 WITHDRAWN** 

IVT-17 Developing a molecular platform for the rapid functional study of novel oligogenic MND candidate genes in vitro Mx Sharlynn Wu

IVT-18 Human in vitro models of TDP-43 proteinopathy for drug screening approaches

Miss Valeria Casiraghi

#### IVT-19 DNA Damage Defects are Rescued by Enhancing Chromatin Ubiquitination and DDRNAs Biogenesis in ALS Cellular Model System

Dr Francesca Esposito

IVT-20 TDP-43 dysregulation and STMN-2 mis-splicing upon proteasomal inhibition in potential iPSC-derived neuronal ALS model

Dr Laila Ritsma

IVT-21 Investigating sporadic ALS using human iPSC-derived cells Miss Lisha Ye

IVV-20 Hyperactivity of Purkinje cell and motor deficits in C9orf72 knockout mice Dr Yuning Liu

IVV-21 Comparison of C9orf72 BAC mice on Jackson and Janvier FVB Backgrounds Miss Sophie Badger

IVV-22 FUSDelta14 mutation impairs normal brain development and causes systemic metabolic alterations Dr Silvia Corrochano

IVV-23 Disruption and phagocytosis of afferent synaptic terminals on axotomized spinal cord motor neurons: microglial recruitment and activation of necroptotic pathways

Miss Alaó Gatius

IVV-24 Characterisation of novel mouse models to investigate the involvement of inhibitory neurons in ALS and FTD linked to Fused in Sarcoma

Ms Felicie Lorenc

IVV-25 Re-modelling of the monosynaptic inputs to MCH neurons in SOD1G93A ALS murine model Dr Jelena Scekic-Zahirovic HCB-21 Divergent cerebellar transcriptome in amyotrophic lateral sclerosis cases with greater burden of pTDP-43 neuropathology Ms Natalie Grima

HCB-22 Iron pathways are perturbed and accumulation overlaps with a shifted lipid profile in the CNS of human MND cases

Dr James Hilton

HCB-23 Cell autonomous dysfunction in VCP mutant hiPSC derived microglia Dr Ben Clarke

HCB-24 Investigating the role of neuroinflammation in C9ORF72 ALS using stem cell models Dr Yujing Gao

HCB-25 Single-Cell Proteomics and Lipidomics of Betz and Purkinje Cells in Human Motor Neuron Disease Brain Miss Jasmine Reese

HCB-26 C9ORF72-ALS patient-derived iPSC microglia display pathological features associated with the hexanucleotide repeat expansion and have a pro-inflammatory profile Dr Björn F. Vahsen

**HCB-27 Meta-analysis of ALS astrocytes reveals inflammatory reactive states** Dr Oliver Ziff

BIO-15 TDP-43 accumulations within intramuscular nerve bundles of ALS patients

Dr Takashi Kurashige

**BIO-16 WITHDRAWN** 

BIO-17 Extracellular Vesicle Sphingomyelins as Biomarkers in Amyotrophic Lateral Sclerosis Dr Gavin McCluskey

BIO-18 Investigating the role of autoantibodies against Neurofilaments in neurodegeneration Miss Ellie Sturmey

**BIO-19 WITHDRAWN** 

#### TST-22 Targeting intracellular TDP-43 using SCFV'S vectorized to pegylated spions

Miss Yara Al Ojaimi

**TST-23 Orexin-dependent sleep impairment in mouse models of ALS** Dr Simon J. Guillot

TST-24 Development of a methodology for the bio-synthesis of a novel "druglike" cell-permeable peptide inhibiting the SRSF1-dependent nuclear export of C9ORF72-repeat transcripts in ALS/FTD Mr Avtac Gul

TST-25 A novel "drug-like" therapeutic strategy to inhibit the SRSF1-dependent nuclear export of pathological C9ORF72repeat transcripts in ALS/FTD Dr Ya-Hui Lin

TST-26 The role of TUDCA in Neurodegeneration Mrs Maria Lo Giudice

TST-27 Inhibition of class I histone deacetylases ameliorates TDP-43 pathology in experimental models of ALS Dr Serena Scozzari

IMG-10 Cortical Hyperexcitability Associated Glutamate Abnormality in ALS

Dr Sicong Tu

IMG-11 Home-based electroencephalography in the assessment of cognitive decline in Amyotrophic Lateral Sclerosis and Fronto Temporal Dementia.

Dr Emmet Costello

IMG-12 Noradrenaline deficiency as a driver of cortical hyperexcitability in amyotrophic lateral sclerosis Dr Caroline Rouaux

**IMG-13 Exploring the split** hand phenomenon with the neurophysiological index Dr Cláudia Santos Silva

#### **CLT-25 WITHDRAWN**

**CLT-26 Long term survival of participants** in the mesenchymal stromal stem cells transplantation in amyotrophic lateral sclerosis. Dr Fabiola De Marchi **CLT-27 Evaluation of the usability of the Atalante exoskeleton in the physical therapy of ALS patients** Mrs Ghida Trad

**CLT-28 Estimating resting energy expenditure in people living with Amyotrophic Lateral Sclerosis** Ms Sarah Roscoe

**CLT-29 A pilot phase II study to evaluate the effect of salbutamol on walking capacity in ambulatory ALS patients** Dr Giorgia Querin

**DSP-22 miR-181 in plasma and CSF is a prognostic biomarker for ALS** Dr Iddo Magen

DSP-23 Interest of arterial blood gas parameters as prognostic markers in amyotrophic lateral sclerosis Dr Hugo Alarcan

DSP-24 Brain metabolic changes due to sex differences in Amyotrophic Lateral Sclerosis (ALS): a 18F-FDG-PET study. Dr Andrea Calvo

**DSP-25 The role of peripheral immunity in Amyotrophic Lateral Sclerosis** Dr Maurizio Grassano

DSP-26 The flail-arm syndrome: the influence of phenotypic features Dr Marta Gromicho

DSP-27 ALS and anxiety - a prognostic indicator?

Dr Mariam Kekenadze

DSP-28 Cognitive phenotypes in ALS relate to distinct longitudinal changes of functional 7networks disruption: a resting-state EEG study Ms Marjorie Metzger

DSP-29 Evaluation of smartphonebased cough data in amyotrophic lateral sclerosis as a potential predictor functional disability Mr Pedro Rocha

COG-12 The Italian version of the Hospital Anxiety and Depression Scale for use in Motor Neurone Disease (HADS-MND): detecting mood disorder in a large sample of MND patients

Dr Monica Consonni

#### **COG-13 Apathy in Amyotrophic Lateral Sclerosis, and how it influences trial participation** MSc Juliette Foucher

COG-14 Italian adaptation of the Beaumont Behavioural Inventory (BBI): psychometric properties and clinical usability

Mrs Laura Peotta

COG-15 Stigma experienced by ALS patients and their caregivers: A mixedmethods study

Dr Marion Sommers-Spijkerman

COG-16 A systematic review of factors associated with grief in informal carers of people living with Motor Neurone Disease

Miss Ana Paula Trucco

CMS-26 Effects of e-learning as support communication for people with ALS among students in multiple healthcare disciplines: Assessment of single-session effects through pre- and post-tests. Mr Tekemasa Ishikawa

**CMS-27 Effect of Anti-aging therapies and cellular therapy a case of ALS** Dr Hemangi Sane

#### CMS-28 WITHDRAWN

CMS-29 Trends in the diagnostic delay and pathway for ALS patients across different countries Dr Catarina Campos

CMS-30 When and why to withdraw riluzole?

Sr Jose Santiago Estevez Alonso

**CMS-31 Real life experience with intrathecal tofersen. A case series.** Sra Maria Asunción Herrero Martin

**CMS-32 Serum Chloride as marker and prognostic factor for respiratory failure in Amyotrophic Lateral Sclerosis patients** Dr. Enrico Matteoni

CMS-33 Recording of eye movements: an innovative source of clinical information in ALS

Miss Federica Cozza

13.45 – 14.00 BREAK

### **SESSION 8**

Chairs: A Malasp	pina (UK) and N Cole (UK)		PARALLEL SESSION
14.00 – 14.20	C21 Modelling neuroinflammation in ALS	J Kriz (Canada)	<b>14.00 – 14.45</b> Parallel Session (non-CME)
142.0 – 14.40	<b>C22</b> Suppressing Neuroinflammation: Regulatory T Lymphocyte Immunomodulatory Therapy for ALS	S Appel (USA)	
14.40 – 14.55	<b>C23</b> Molecular signatures of neuroinflammation in patient tissue across sporadic, SOD1 and C9orf72 ALS cohorts	O Rifai (UK)	-
14.55 – 15.10	Discussion/Questions		-

15.10 – 15.25 BREAK

### SESSION 9

### TRIAL DESIGN AND CLINICAL ENDPOINTS

Chairs: L Bruijn (UK) A Al-Chalabi (UK)

15.25 – 15.45	C24 Improving clinical endpoints in therapeutic trials for ALS	R van Eijk (Netherlands)
15.45 – 16.05	C25 Improving ALS clinical trial design	T Ferguson/S Fradette (USA)
16.05 – 16.20	<b>C26</b> Registry of Validated Endpoints in ALS (REVEALS): Results from a multi-centre prospective study of respiratory measures and their clinical meaningfulness	D Meldrum (Ireland)
16.20 – 16.35	Discussion/Questions	

16.35 – 16.50 BREAK

### SESSION 10

### **CLOSING SESSION**

Chairs: B Dickie (UK) A Al-Chalabi (UK)

16.50 – 16.55	Invitation to Basel 2023	
16.55 – 17.25	BrainGate: Clinical trials in intracortical brain- computer interfaces toward the restoration of communication and mobility	
17.25 – 17.35	Discussion/Questions	
17.35 – 17.55	Healey Center Prize and Lalji Family Award	
17.55 – 18.15	Final thoughts and Prize Draw	



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