



30th international  
symposium  
on ALS/MND

# 30th international symposium on ALS/MND

## Perth Australia

4 – 6 December 2019

# Programme

Hosts:

MND Australia, in partnership with MND WA



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



motor neurone disease  
association

## 30th international symposium on ALS/MND

### Organiser of the Symposium:



**Motor Neurone Disease Association**  
10-15 Notre Dame Mews, Northampton NN1 2BG, UK  
Tel: (-) 44 1604 250505  
Email: [symposium@mndassociation.org](mailto:symposium@mndassociation.org)  
Website: [www.mndassociation.org](http://www.mndassociation.org)

### CME Accreditation

The 30th International Symposium on ALS/MND has been approved by the Federation of the Royal College of Physicians of the United Kingdom for 18 category 1 (external) CPD credit(s).

### Hosts for the Symposium:



**MND Australia**  
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The **30th International Symposium on ALS/MND, Perth, Australia, 4 December 2019 – 6 December 2019** has been accredited by the European Accreditation Council for Continuing Medical Education (EACCME®) with 13 European CME credits (ECMEC®s). Each medical specialist should claim only those hours of credit that he/she actually spent in the educational activity.

Through an agreement between the Union Européenne des Médecins Spécialistes and the American Medical Association, physicians may convert EACCME® credits to an equivalent number of AMA PRA Category 1 Credits™. Information on the process to convert EACCME® credit to AMA credit can be found at [www.ama-assn.org/education/earn-credit-participation-international-activities](http://www.ama-assn.org/education/earn-credit-participation-international-activities).

Live educational activities, occurring outside of Canada, recognised by the UEMS-EACCME® for ECMEC®s are deemed to be Accredited Group Learning Activities (Section 1) as defined by the Maintenance of Certification Program of the Royal College of Physicians and Surgeons of Canada.

### Held in co-operation with:



INTERNATIONAL ALLIANCE  
OF ALS/MND ASSOCIATIONS

### The International Alliance of ALS/MND Associations

Email: [alliance@als-mnd.org](mailto:alliance@als-mnd.org)  
Website: [www.alsmndlalliance.org](http://www.alsmndlalliance.org)

## Welcome

On behalf of MND Australia and MND Western Australia, I extend a warm welcome to delegates of the 30th International Symposium on ALS/MND, the 27th meeting of the International Alliance of ALS/MND Associations and the 17th Allied Professionals Forum. Much has changed over the last 30 years. The number of people attending the Symposium and the number of platform and poster presentations have increased dramatically, demonstrating a sustained growth in interest and investment in ALS/MND research globally. The breadth of meetings has also grown in response to the urgent need to improve care and support and accelerate access to clinical trials as the search for an end to ALS/MND continues.

MND Australia and its members, the State MND Associations, form the only national network focused on improving the lives of all Australians living with motor neurone disease (MND). For over 35 years this national network has helped increase understanding of the disease and advocate for improvements in its treatment and care to ensure people living with MND have the best quality-of-life possible. Investment in research is a priority, and through the MND Research Institute of Australia, the research arm of MND Australia, over the last 32 years more than \$32 million donated by the Australian community has been invested in research with the greatest chance of realising our collective vision of a world without MND.

This year's program clearly demonstrates the fruits of investment in research, the commitment to improving the lives of people living with ALS/MND and the depth and breadth of research efforts globally. Now is a time of great hope for the ALS/MND community and it is with this in mind that we extend a special welcome to people living with ALS/MND to the meetings in Perth and look forward to their contributions, questions and insights.

The International Symposium on ALS/MND is quite unique in bringing together researchers, clinicians, health professionals, ALS/MND associations and people living with ALS/MND. We hope that the Perth sunshine and Aussie hospitality will foster further collaborations, sharing of knowledge, innovations and new ideas. We hope that you will be inspired and motivated to continue the work you do to improve the lives of people living with ALS/MND and to share what you have learned with your colleagues on your return home. Together we will defeat ALS/MND.

Perth is Australia's sunniest city and easy to get around so please enjoy your time with us and take time out of the busy schedule to explore and experience all Perth, and its surrounds, has to offer.

### **Carol Birks**

CEO, MND Australia

## Foreword

A warm welcome to the 30th International Symposium on ALS/MND, in the wonderful city of Perth, for what has become the most important event of the year for researchers and clinicians working in the field of motor neuron disease.

The most notable scientific discovery to have come out of Western Australia is the famous Nobel Prize winning observation by Barry Marshall and Robin Warren that peptic ulcers are caused by a bacterium, *H pylori*. The story demonstrates the importance of how radical new hypotheses can change medicine, but also the importance of rigorous scientific methods to overcome established orthodoxy. Finding effective treatments for ALS/MND is a challenging problem but we are making progress through science.

Each year the Program Committee has the difficult task of selecting invited speakers and choosing platform presentations from the submitted abstracts. The good news is that the task gets more difficult each year, because the quality of scientific work in ALS/MND is increasing dramatically. Nowhere is this more evident than in Australia, a country that consistently achieves more platform presentations than would be expected on the basis of population size.

Many areas of medicine have progressed to a phase of personalised therapeutics, where treatment is based on stratification by individual risk factors. At this meeting we have plenary sessions on design of precision DNA-based therapies and platform presentations on antisense and viral mediated reduction in SOD1 levels, demonstrating that precision medicine is now beginning in ALS. There are also several sessions highlighting the key developments in care that are changing practice and improving the wellbeing of people living with the disease. As always, the Symposium provides renewed hope to bring home to our clinics and laboratories.

### **Kevin Talbot**

Programme Committee Chair



# PROGRAMME

**Wednesday 4 December 2019**

**SESSION1**

RIVERSIDE THEATRE

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**JOINT OPENING SESSION**

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*Chairs: S Light (UK) K Talbot (UK)*

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**08.30 – 08.35**

Welcome – *S Light (UK) and K Talbot (UK)*

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**08.35 – 08.45**

Welcome from Host Association/Welcome to Country ceremony  
*D Ali/O Whalley*

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**08.45 – 09.20**

**C1** Glymphatic system dysfunction as a driver of protein mis-aggregation in neurodegenerative disease  
*J Iliff (USA)*

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**09.20 – 09.55**

**C2** The biomarker challenge: What is it and are we nearly there?  
*M Turner (UK)*

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**09.55 – 10.05**

International Alliance Humanitarian Award

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**10.05 – 10.25**

IPG Award and winner's research presentation

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**10.30 – 11.00**

REFRESHMENTS, NETWORKING AND EXHIBITORS:  
Riverside Theatre Foyer

**SESSION 2A**

BELLEVUE BALLROOM 2

**PROTEOSTASIS / PROTEOTOXICITY**

*Chairs: J Atkin (Australia) J Robertson (Canada)*

**11.00 – 11.30**

**C3** The fine balance of proteostasis and its implications for ALS  
*J Yerbury (Australia)*

**11.30 – 11.45**

**C4** Cytoplasmic TDP-43 alters the solubility and abundance of numerous proteins in ALS/FTD pathology  
*T Hedl (Australia)*

**11.45 – 12.00**

**C5** Monitoring autophagy dynamics in motor neurons in vivo using a novel reporter mouse model of ALS shows early impairment of autophagy flux  
*N Perera (Australia)*

**12.00 – 12.15**

**C6** Selective clearance of misfolded SOD1 delays disease onset in animal models of ALS  
*J Kong (Canada)*

**12.15 – 12.45**

**C7** Non-protein amino acids and neurodegenerative disease  
*K Rodgers (Australia)*

**SESSION 2B**

RIVERSIDE THEATRE

**CLINICAL TRIALS**

*Chairs: L van den Berg (Netherlands)  
D Rowe (Australia)*

**11.00 – 11.30**

**C8** The ALS Platform Trial: Design considerations and statistical efficiencies  
*B Saville (USA) and S Paganoni (USA)*

**11.30 – 11.50**

**C9** A phase Ib/Ila Open Label trial of MN-166 (ibudilast) in ALS: A biomarker endpoint-based clinical trial  
*S Babu (USA)*

**11.50 – 12.10**

**C10** Modulation of innate immunity by MSC-NTF cells (NurOwn®) correlates with ALS clinical outcomes  
*R Kern (Israel)*

**12.10 – 12.30**

**C11** Safety, PK, PD and exploratory efficacy in single and multiple dose study of a SOD1 antisense oligonucleotide (tofersen) in participants with ALS  
*T Miller (USA)*

**SESSION 2C**

MEETING ROOMS 1-3

**CLINICAL ELECTROPHYSIOLOGY**

*Chairs: S Vucic (Australia)  
M de Carvalho (Portugal)*

**11.00 – 11.15**

**C12** Novel threshold tracking TMS assessment of trancallosal inhibition identifies potential mechanism for interhemispheric disease spread  
*M van den Bos (Australia)*

**11.15 – 11.30**

**C13** Cortical hyperexcitability and cognitive dysfunction in ALS  
*M Higashihara (Australia)*

**11.30 – 11.45**

**C14** Spinal hyperexcitability in ALS is extrinsic to motoneurons  
*V Marchand-Pauvert (France)*

**11.45 – 12.00**

**C15** Asymmetry of cortical dysfunction identifies regions of onset and relates to clinical heterogeneity in ALS  
*T Dharmadasa (Australia)*

**12.00 – 12.15**

**C16** Neurophysiological index is associated with the survival of patients with ALS  
*B Cao (China)*

**12.15 – 12.30**

**C17** The split hand in ALS: A possible role for the neuromuscular junction  
*M de Carvalho (Portugal)*

**12.30 – 12.45**

**C18** The rise and fall of fasciculations in amyotrophic lateral sclerosis  
*J Bashford (UK)*

**12.30 – 14.00**

LUNCH AND NETWORKING: Bellevue Ballroom 1/Bellevue Foyer

EXHIBITORS: Riverside Theatre Foyer

**SESSION 3A**  
 BELLEVUE BALLROOM 2

**SYNAPTIC  
PATHOLOGY**

Chairs: L Greensmith (UK)  
T Dickson (Australia)

**14.00 – 14.30**

**C19** Neuromuscular degeneration in ALS and SMA  
*H Nishimune (USA)*

**14.30 – 14.50**

**C20** Modeling ALS: Human neuromuscular junctions in a dish  
*K Dittlau (Belgium)*

**14.50 – 15.10**

**C21** Loss of c9orf72: A problem at the synapse?  
*J Robertson (Canada)*

**15.10 – 15.30**

**C22** Can oestrogen protect against the synaptic plasticity deficits that underlie motor cortex dysfunction in ALS?  
*C Blizzard (Australia)*

**SESSION 3B**  
 RIVERSIDE THEATRE

**DEMOGRAPHICS AND  
CLINICAL FEATURES**

Chairs: C Armon (Israel) A Chio (Italy)

**14.00 – 14.30**

**C23** ALS: Phenotypes, demographics and clinical management in Asia  
*N Shahrizaila (Malaysia)*

**14.30 – 14.50**

**C24** Prognosis and clinical features of ALS patients in Japan  
*N Atsuta (Japan)*

**14.50 – 15.10**

**C25** ALS in Latin America: A population-based incidence study in three countries  
*O Hardiman (Ireland)*

**15.10 – 15.30**

**C26** How frequent are pauses in ALS progression? Results from a population-based cohort  
*A Calvo (Italy)*

**SESSION 3C**  
 MEETING ROOMS 1-3

**GENOMICS**

Chairs: N Wray (Australia) A Al-Chalabi (UK)

**14.00 – 14.30**

**C27** Future directions in ALS genomics  
*N Wray (Australia)*

**14.30 – 14.45**

**C28** GWAS in ALS identifies novel loci and insight into the genetic architecture  
*J Veldink (Netherlands)*

**14.45 – 15.00**

**C29** Genome-wide meta-analysis identifies new loci associated with ALS and links body mass index with disease genetics  
*A Iacoangeli (UK)*

**15.00 – 15.15**

**C30** Unbiased genome-wide screen identifies new ALS risk variants within gene-regulatory elements  
*J Cooper-Knock (UK)*

**15.15 – 15.30**

**C31** Computational efficient method to detect genetic interactions associated with age of onset in an ALS genome-wide association study  
*J Gui (USA)*

**15.30 – 16.00** REFRESHMENTS, NETWORKING AND EXHIBITORS: Riverside Theatre Foyer

# Welcome to Perth



**SESSION 4A**

BELLEVUE BALLROOM 2

**THERAPEUTIC STRATEGIES**

*Chairs: L Van Den Bosch (Belgium)  
L Brujin (UK)*

**16.00 – 16.30**

**C32** Designer DNA drug therapy for human neurodegenerative disease  
*D Cleveland (USA)*

**16.30 – 17.00**

**C33** iPSC-derived models for genetic and compound screening in ALS  
*S Finkbeiner (USA)*

**17.00 – 17.15**

**C34** An AI drug discovery case study establishing new neuroprotective compounds for treating ALS  
*R Paul (UK)*

**17.15 – 17.30**

**C35** Machine learning to accelerate drug discovery: A novel small molecule rescues ALS phenotypes in preclinical models  
*I Choi (USA)*

**17.30 – 17.45**

**C36** Relevance of the therapeutic agent CuATSM to sporadic ALS  
*P Crouch (Australia)*

**SESSION 4B**

RIVERSIDE THEATRE

**NON-MOTOR SYMPTOMS**

*Chairs: G Mora (Italy) R Radakovic UK*

**16.00 – 16.30**

**C37** Pseudobulbar affect in ALS: Not (only) a laughing matter  
*E Pioro (USA)*

**16.30 – 16.50**

**C38** Psychosis risk states in FTD-MND  
*A Wilcox (UK)*

**16.50 – 17.10**

**C39** Progression of cognitive and behavioural impairment in early ALS  
*J Raaphorst (Netherlands)*

**17.10 – 17.25**

**C40** Association between oculomotor dysfunction and cognitive impairment in ALS  
*N Ticozzi (Italy)*

**17.25 – 17.40**

**C41** Cognitive dysfunction as an endophenotype in C9orf72 positive ALS  
*M Ryan (Ireland)*

**SESSION 4C**

MEETING ROOMS 1-3

**EMERGING TISSUE BIOMARKERS**

*Chairs: J Costa (Portugal) R Bowser (USA)*

**16.00 – 16.20**

**C42** Disease progression in ALS: Cell senescence and metabolism in the driving seat  
*A Malaspina (UK)*

**16.20 – 16.40**

**C43** Myeloid cells and complement c5aR1 are associated with clinical features in ALS patients  
*T Woodruff (Australia)*

**16.40 – 17.00**

**C44** Chitotriosidase level and activity rises in the pre- and early symptomatic phases of ALS  
*A Thompson (UK)*

**17.00 – 17.20**

**C45** Isolation and enrichment of neural-derived exosomes from plasma of ALS patients and characterisation of their miRNAome using next-generation sequencing  
*R Dunlop (USA)*

**17.20 – 17.40**

**C46** Identification of new plasma-based biomarkers for ALS by RNA sequencing of leukocytes  
*Y Chen (China)*

**18.00 – 20.30** GLOBAL WALK TO D'FEET FOLLOWED BY BARBECUE RECEPTION



## Thursday 5 December 2019

### SESSION 5A

BELLEVUE BALLROOM 2

#### TDP-43

*Chairs: C Shaw (UK) P Wong (USA)*

##### 08.30 – 08.50

**C47** Validation of TDP-43 splicing repression as a therapeutic target for ALS-FTD  
*P Wong (USA)*

##### 08.50 – 09.10

**C48** Targeting the nucleocytoplasmic transport machinery: Why does TDP-43 mislocalise?  
*M Morsch (Australia)*

##### 09.10 – 09.30

**C49** TDP-43 triggers mitochondrial DNA release to activate cGAS/STING in ALS  
*A Yu (Australia)*

##### 09.30 – 09.45

**C50** Detection and quantification of pathological C-terminal TDP-43 fragments in post mortem brain tissue  
*E Feneberg (UK)*

##### 09.45 – 10.00

**C51** Super-resolution characterisation of TDP-43 aggregation in ALS  
*O Kantelberg (UK)*

### SESSION 5B

RIVERSIDE THEATRE

#### CARER AND FAMILY SUPPORT

*Chairs: M O'Brien (UK) S Aoun (Australia)*

##### 08.30 – 09.00

**C52** Supporting MND family carers from diagnosis to bereavement: The palliative approach to care  
*S Aoun (Australia)*

##### 09.00 – 09.30

**C53** Research and support for young caregivers in families with ALS  
*M Kavanaugh (USA)*

##### 09.30 – 09.45

**C54** When the shared journey ends: The enduring impact of ALS on bereaved caregivers  
*M Galvin (Ireland)*

##### 09.45 – 10.00

**C55** Individual quality of life among spousal ALS patient-caregiver dyads  
*M Galvin (Ireland)*

### SESSION 5C

MEETING ROOMS 1-3

#### THE SPECTRUM OF MOTOR NEURON DISORDERS

*Chairs: P Shaw (UK) H Mitsumoto (USA)*

##### 08.30 – 09.00

**C56** Lessons from SBMA: Pathophysiology, clinical characteristics and treatment strategies  
*G Sobue (Japan)*

##### 09.00 – 09.30

**C57** ALS-PDC of the Kii Peninsula, Japan: Clinical and neuropathological features and epidemiology  
*S Kuzuhara (Japan)*

##### 09.30 – 09.45

**C58** Genotype phenotype correlation and longitudinal study of hereditary spastic paraparesis and primary lateral sclerosis  
*J Statland (USA)*

##### 09.45 – 10.00

**C59** Infant with MND caused by homozygosity for a SOD1 mutation and no SOD1 enzymatic activity: Implications for clinical trials to depress the level of SOD1  
*P Andersen (Sweden)*

**10.00 – 10.30** REFRESHMENTS, NETWORKING AND EXHIBITORS: Riverside Theatre Foyer/Pavilion 1



LOCATION: PAVILION 1

**POSTER SESSION A: 10.30 – 12.30****10.30 – 11.30****Theme 1 (EPI):** Epidemiology and informatics**Theme 3 (IVT):** In vitro experimental models**Theme 5 (HCB):** Human cell biology and pathology**Theme 7 (TST):** Pre-clinical therapeutic strategies**Theme 9 (CLT):** Clinical trials and trial design**Theme 11 (COG):** Cognitive and psychological assessment and support**Theme 13 (CMS):** Clinical management and support**Theme CP:** Care practice**11.30 – 12.30****Theme 2 (GEN):** Genetics and genomics**Theme 4 (IVV):** In vivo experimental models**Theme 6 (BIO):** Tissue biomarkers**Theme 8 (IMG):** Clinical imaging and electrophysiology**Theme 10 (DSP):**

Disease stratification and phenotyping of patients

**Theme 12 (RNM):** Respiratory and nutritional management**Theme WP:** Biomedical and clinical work in progress

**12.30 – 14.00** LUNCH AND NETWORKING: Bellevue Ballroom 1/Bellevue Foyer  
EXHIBITORS: Riverside Theatre Foyer

**SESSION 6A**  
BELLEVUE BALLROOM 2**HUMAN CELL BIOLOGY AND PATHOLOGY**

Chairs: M-L Rogers (Australia) J Prehn (Ireland)

**14.00 – 14.30****C60** Investigating the role of endogenous retroviruses in ALS  
A Nath (USA)**14.30 – 14.45****C61** Antiviral immune response as a trigger of FUS proteinopathy in ALS  
H An (UK)**14.45 – 15.00****C62** Distinct autoimmune antibody responses towards TDP-43 in ALS  
T Brudek (Denmark)**15.00 – 15.15****C63** Cell autonomous dysfunction of brain pericytes in ALS: Impaired regulation on interleukin-6 by TDP-43  
E Scotter (New Zealand)**15.15 – 15.30****C64** Defective phagocytic function is associated with rapid progression in ALS patients  
S Kim (Korea)**SESSION 6B**  
RIVERSIDE THEATRE**PALLIATIVE CARE**

Chairs: D Oliver (UK) S Feldman (USA)

**14.00 – 14.40****C65** Palliative care and healthcare utilization at the end of life in people with ALS  
L Deliens (Belgium)**14.40 – 15.10****C66** Positive impact of physical exercise on ALS patients' QoL and effective state  
D Lulé (Germany) TBC**15.10 – 15.30****C67** A trial of suprascapular nerve block for shoulder pain in motor neuron disease  
P Allcroft (Australia)**SESSION 6C**  
MEETING ROOMS 1-3**NEUROIMAGING**

Chairs: N Atassi (USA) P-F Pradat (France)

**14.00 – 14.30****C68** Is ALS a network disease?  
J Grosskreutz (Germany)**14.30 – 14.50****C69** Disruptions in cortical structures and pathways precede the development of ALS in asymptomatic C9orf72 familial ALS  
N Geevasinga (Australia)**14.50 – 15.10****C70** Spinal cord MRI for early detection of presymptomatic pathology in C9orf72 mutation carriers: A longitudinal neuroimaging study  
G Querin (France)**15.10 – 15.30****C71** 'Next generation' cervical cord MRI in ALS reveals marked atrophy and corticospinal tract degeneration  
P Nestor (Australia)

**15.30 – 16.00** REFRESHMENTS, NETWORKING AND EXHIBITORS Riverside Theatre Foyer

**SESSION 7A**

BELLEVUE BALLROOM 2

**GENETICS**

*Chairs: G Nicholson (Australia) P Andersen (Sweden)*

**16.00 – 16.20**

**C72** Association of a poly-T structural variant within the SCAF4 gene and ALS  
*J Pytte (Australia)*

**16.20 – 16.40**

**C73** Intronic structural variant within stathmin 2 gene (STMN2) impacts disease duration in sporadic ALS  
*F Theunissen (Australia)*

**16.40 – 17.00**

**C74** Correlating survival by SOD1 variant in global ALS cohort identifies variants with a strong effect on prognosis  
*S Opie-Martin (UK)*

**17.00 – 17.20**

**C75** Novel software 'TRIBES' enables distant relationship and disease variant discovery in Australian ALS  
*N Twine (Australia)*

**17.20 – 17.40**

**C76** Relatedness mapping and IBD analysis of Australian sporadic ALS/FTD identifies distantly related sALS cases with a mutation in FIG4 and implicates two new genome-wide loci linked to sALS and FTD  
*K Williams (Australia)*

**17.40 - 17.50**

**Late breaking news:** Exome sequencing in ALS implicates a novel gene, DNAJC7, encoding a heat-shock protein  
*A Iacoangeli (UK)*

**SESSION 7B**

RIVERSIDE THEATRE

**IMPROVING CARE PRACTICE**

*Chairs: M Ogino (Japan)  
 M Galvin (Ireland)*

**16.00 – 16.30**

**C77** Progressive neurological diseases: Modelling care  
*S Mathers (Australia)*

**16.30 – 16.45**

**C78** Breaking the news of the MND diagnosis: The gap between standards and actual practice  
*S Aoun (Australia)*

**16.45 – 17.00**

**C79** Development of the MiND Toolkit for management of cognitive and behavioural impairment in MND: A modified Delphi method  
*R Radakovic (UK)*

**17.00 – 17.15**

**C80** Terminal Care in patients with MND: A clinical audit of inpatient and community patients  
*A Kulkarni (Australia)*

**17.15 – 17.30**

**C81** Telehealth provides meaningful contributions to patient care in ALS  
*K Atkins (Australia)*

**17.30 – 17.45**

**C82** The impact of mental health on acute health service provision in MND: A big data study  
*J Trollor (Australia)*

**SESSION 7C**

MEETING ROOMS 1-3

**BIOENERGETICS AND METABOLISM**

*Chairs: J-P Loeffler (France)  
 S Ngo (Australia)*

**16.00 – 16.15**

**C83** Metabolic rewiring in ALS  
*D Zarnescu (USA)*

**16.15 – 16.30**

**C84** Manipulation of bioenergetic pathways in motor neuron diseases  
*H Chaytor (UK)*

**16.30 – 16.45**

**C85** Mitochondrial bioenergetic profile in platelets as a biomarker for ALS  
*M Kazamel (USA)*

**16.45 – 17.00**

**C86** Altered skeletal muscle glucose-fatty acid flux in ALS  
*S Kirk (Australia)*

**17.00 – 17.15**

**C87** Metabolic dysfunction in MND: A 31-phosphorous magnetic resonance spectroscopy study  
*M Sassani (UK)*

**17.15 – 17.30**

**C88** Brain metabolic correlates of King's staging system in ALS: A 18F-FDG-PET study  
*A Chio (Italy)*

**17.30 – 17.45**

**C89** Lipids, apolipoproteins and prognosis of ALS  
*C Ingre (Sweden)*

LOCATION: PAVILION 1

**POSTER SESSION B: 18.00 – 20.00****18.00 – 19.00**

All themes

**19.00 – 20.00**

Free flow for all delegates around posters

## Friday 6 December 2019

### SESSION 8A

BELLEVUE BALLROOM 2

#### DISEASE MODELS

*Chairs: L Ferraiuolo (UK) B Turner (Australia)*

**08.30 – 09.00**

**C90** Mouse models of ALS: Past, present and future  
*B Turner (Australia)*

**09.00 – 09.15**

**C91** Longitudinal quantitative proteomics reveals distinct biochemical signatures of cortical and spinal cord neurodegeneration and recovery in TDP-43 transgenic mice  
*A Walker (Australia)*

**09.15 – 09.30**

**C92** Golgi dysfunction is an early event associated with TDP-43 pathology formation in ALS  
*B Berning (Australia)*

**09.30 – 09.45**

**C93** Antioxidant drugs reveal the potential for patient stratification in motor neuron disease  
*C Allen (UK)*

**09.45 – 10.00**

**C94** Intrathecal AAV9-SOD1-shRNA administration for ALS  
*P Allred (USA)*

### SESSION 8B

RIVERSIDE THEATRE

#### DYSPHAGIA AND NUTRITIONAL MANAGEMENT

*Chairs: F Steyn (Australia) C McDermott (UK)*

**08.30 – 08.50**

**C95** Loss of appetite is associated with a loss of weight and fat mass in patients with ALS  
*F Steyn (Australia)*

**08.50 – 09.10**

**C96** Dietary intake in patients with MND: Assessment relative to disease severity and resting energy expenditure  
*V Chachay (Australia)*

**09.10 – 09.30**

**C97** A multidisciplinary pilot study to trial the feasibility and effect of swallowing exercises and diet among people with ALS  
*V Flood (Australia)*

**09.30 – 09.50**

**C98** Validation of the Physiologic Risk Index for Swallowing Impairment (PRISM) in ALS  
*E Plowman (USA)*

**10.00 – 10.30** REFRESHMENTS, NETWORKING AND EXHIBITORS: Riverside Theatre Foyer

**Join us in  
Montreal,  
Canada**

9-11 December 2020



## SESSION 9A

BELLEVUE BALLROOM 2

### IMMUNITY AND INFLAMMATION

Chairs: K Yamanaka (Japan) L Barbeito (Uruguay)

**10.30 – 10.50**

**C99** The role of innate and acquired immunity in neuroinflammation of ALS mice  
K Yamanaka (Japan)

**10.50 – 11.10**

**C100** Immunosuppressive functions of M2 macrophages derived from iPSC of ALS patients  
W Zhao (USA)

**11.10 – 11.30**

**C101** MCP1-CCR2 and neuroinflammation in the ALS motor cortex with TDP-43 pathology  
H Ozdinler (USA)

**11.30 – 11.50**

**C102** Microglial galectin-3 in the spinal white matter is a key molecule for motor neuron degeneration in ALS  
S Hayashi (Japan)

**11.50 – 12.10**

**C103** Using patient-derived microglia to investigate neuroinflammation in MND and provide a platform suitable for patient-specific drug screening  
H Quek (Australia)

**12.10 – 12.30**

**C104** A nanoparticle-based strategy for treating neuroinflammation in MND  
A Wright (Australia)

## SESSION 9B

RIVERSIDE THEATRE

### RESPIRATORY SUPPORT

Chairs: J Andrews (USA) E Pioro (USA)

**10.30 – 11.00**

**C105** The multidimensional nature of respiratory failure in ALS  
C Morélot-Panzini (France)

**11.00 – 11.30**

**C106** The management of disordered breathing in MND  
D Berlowitz (Australia)

**11.30 – 11.50**

**C107** Slow vital capacity as a prognostic factor in ALS: A population-based study  
A Calvo (Italy)

**11.50 – 12.10**

**C108** A feasibility study of an ambulatory non-invasive ventilation set-up model using intelligent volume assured pressure support mode in MND  
W Chow (Australia)

**12.10 – 12.30**

**C109** The physiological effects of a single session of lung volume recruitment in people with MND  
N Sheers (Australia)

**12.30 – 14.00** LUNCH AND NETWORKING: Bellevue Ballroom 1/Bellevue Foyer  
EXHIBITORS: Riverside Theatre Foyer

## SESSION 10 RIVERSIDE THEATRE

### JOINT CLOSING SESSION

Chairs: K Talbot (UK) M Kiernan (Australia)

**14.00 – 14.05**

Poster Prizes Announcement

**14.15 – 14.50**

**C110** The dawn of brain computer interfaces  
T Oxley (Australia)

**15.10 – 15.20**

Late breaking news

**14.05 – 14.15**

Invitation to Montreal 2020

**14.50 – 15.10**

Healey Center Award

# Poster sessions

## THEME 1

### Epidemiology and informatics

**EPI-01 Applying Mendelian Randomization methods to investigating risk factors and causes for ALS: promises and pitfalls**

Armon C

#### EPI-02 WITHDRAWN

**EPI-03 Determining environmental risk factors for ALS using large claims and environmental pollutant databases**

Miller C, Arndt T, Russo P, Shukla O, Merrill C, Agnese W, Apple S, Bradley W, Stommel E, Andrew A, Shi X, Butt T, Guetti B, Harrison A

**EPI-04 Cyanobacterial exposure and neurodegenerative disease at autopsy**

DeWitt J, Butt T, Rueckert J, Buskey A, Martindale R, Andrew A, Shi X, Peipert D, Bradley W, Stommel E

**EPI-05 Residential history of volatile solvent exposure and ALS risk: an interdisciplinary GIS-based spatiotemporal approach**

Andrew A S, Shi X, Guetti B, Butt T, Piepart D, Pioro E, Stommel E, Bradley W

**EPI-06 Incidence of MND/ALS in the Western Cape province of South Africa**

Henning F, Heckmann J, Naidu K, Vlok L, Chetty S, Cross H, Marin B

**EPI-07 Epidemiological study of MND in India**

Sane H, Varghese R, Pradhan R, Paranjape A, Badhe P, Gokulchandran N, Sharma A

#### EPI-08 WITHDRAWN

**EPI-09 MND Register for England, Wales and Northern Ireland**

Opie-Martin S, Ossher L, Bredin A, Kulka A, Kelly K, Talbot K, Al-Chalabi A

**EPI-10 Machine learning model using insurance claims data to help predict future ALS diagnosis**

Grabowsky T, Miller C, Folta T, Shukla O, Merrill C, Agnese W

**EPI-11 Googling global burden of motor neuron diseases**

Phan T G, Beare R, Srikanth V, Ma H

**EPI-12 Assessing the impact of genetic information on the accuracy of machine learning based survival analyses in ALS**

Bowles H, Iacoangeli A, Liang J, Al-Khleifat A, Opie-Martin S, Dobson R J, Newhouse S J, Al-Chalabi A

**EPI-13 Evidence for generalizability of edaravone efficacy using a novel machine-learning (ML) risk-based analysis tool**

Brooks B R, Pioro E P, Schactman M, Beaulieu D, Taylor A A, Keymer M, Agnese W, Perdrizet J, Apple S, Ennist D L

**EPI-14 Health utility decline with advancing FT9 stage of ALS: an application to health economic evaluation of hypothetical treatment**

Thakore N J, Pioro E P

**EPI-15 Functional impairment and survival prediction in ALS patients: a probabilistic model of disease progression**

Chio A, Zandonà A, Daberdaku S, Vasta R, Nefussy B, Tavazzi E, Lunetta C, Mora G, Mandrioli J, Grisan E, Gotkine M, Calvo A, Moglia C, Drory V, Di Camillo B

**EPI-16 Possibility of needle EMG for prognostic prediction of ALS in the analysis of the Japanese National Registry for intractable and rare diseases**

Sato Y, Kanatani Y

**EPI-17 The effect of comorbidities in ALS prognosis and disease progression: results from ALS Natural History Consortium**

Lunetta C, Arcila-Londono X, Walk D, Vota S, Sherman A, Goslin K, Hayat G, Newman D, Steijlen K, Wymer J, Olney N, Somers M, Yu H, Jones J, Gandhi N, Faulconer K, La T, Tarlarini C, Gerardi F, Macklin E

**EPI-18 Longitudinal data collection of combined clinic cohorts for improved understanding of ALS natural history**

Lunetta C, Arcila-Londono X, Walk D, Vota S, Sherman A, Goslin K, Hayat G, Newman D, Steijlen K, Wymer J, Olney N, Somers M, Yu H, Jones J, Gandhi N, Faulconer K, La T, Tarlarini C, Gerardi F, Macklin E

**EPI-19 Retaining voice identity in ALS/MND patients by new generation Voice-Output Communication Aids (VOCAs)**

Chopra R, Sane H

## THEME 2

### Genetics and genomics

**GEN-01 The ALS GAP Program: paving the way for genetic characterization of ALS in the clinic**

Rogggenbuck J, Doyle C, Lincoln T, Glass J

**GEN-02 A clinical tool to determine the probability of a person with ALS having a positive gene test, given their age of onset**

Mehta P R, Jones A R, Iacoangeli A, Lewis C M, Morgan S, Pittman A, Morrison K E, Shaw P J, Shaw C E, Al-Chalabi A

**GEN-03 Implication of rare variants in causative genes for Charcot-Marie-Tooth disease in patients clinically diagnosed as ALS**

Hama Y, Date H, Ishiura H, Mitsui J, Doi K, Yoshimura J, Morishita S, Tsuji S, Mizusawa H, Takahashi Y

**GEN-04 Shared genetic architecture between ALS, bipolar disorder and cognitive function**

Byrne R P, Doherty M A, Hengeveld J C, Kelly C, van Rheenen W, Veldink J H, Hardiman O, McLaughlin R L

**GEN-05 Advantages of Southern blot to overcome repeat-primed PCR limits in the molecular diagnosis a C9ORF72-mutated FTD patient**

Chudinova A V, Heitz C, Pattyn A, Thouvenot E, Raoul C, Lumbroso S, Mouzat K

**GEN-06 Targeted long-read sequencing of C9orf72 repeat detects repeat length, sequence composition and methylation status in ALS patients**

Parker M D, Wyles M, Heath P, Cooper-Knock J, Ashok V, Thornton Z A, Wang D, Kirby J, Shaw P

**GEN-07 Investigation of a novel genomic structural variant of NEK1 in ALS**

Jiang L, Pytte J, Theunissen F, Flynn L, Fletcher S, Anderton R, Mastaglia F, Rodger J, Harvey A, Needham M, Akkari A

**GEN-08 The elucidation of disease mechanisms underlying MND/familial ALS caused by a novel gene mutation**

Salman M, Rother A, Topp S, Smith B, Shaw C, de Belleroche J

**GEN-09 Exome analysis in 54 autopsied Japanese sporadic ALS patients**

Ishihara T, Hatano Y, Tada M, Kakita A, Onodera O

**GEN-10 Genetic and functional analysis of the KIF5A variants in Japanese patients with sporadic ALS**

Nakamura R, Atsuta N, Tohnai G, Nakatouchi M, Hayashi N, Katsuno M, Izumi Y, Taniguchi A, Hattori N, Morita M, Kano O, Kuwabara S, Oda M, Abe K, Mizoguchi K, Kaji R, Sobue G

**GEN-11 A novel splicing variant of ANXA11 in Japanese sporadic ALS patients**

Hatano Y, Ishihara T, Tada M, Kakita A, Onodera O

**GEN-12 ANXA11 Mutations are commonly associated with patients with sporadic ALS of Chinese origin**

Zou Z, Feng S-Y, Feng S-M, Huang H-P

**GEN-13 Novel mutations in the cargo binding and stalk domains of KIF5A link to patients with ALS**

Zou Z, Feng S, Feng S, Huang H-P

**GEN-14 Familial flail leg ALS caused by PFN1 mutation**

Zou Z-Y, Yu J-T, Chen D-D, Huang H-P

**GEN-15 Exploration of genetic architecture in Chinese ALS patients**

Zhang K, Shen D, Tai H, Liu S, Sun X, Wang Z, Yang X, Liu Q, Li X, Guan Y, Liu M, Zhang X, Cui L

**GEN-16 Mutation analysis of causative genes for ALS in southern China**

Wang J L, Li Z, Huang L, Yuan Y C, Li W Z, Ni J, Hu Y T, Shen L, Tang B S

**GEN-17 Mutation analysis of the GLT8D1 and ARPP21 genes in ALS patients in mainland China**

Wang J, Li W, Liu Z, Sun W, Yuan Y, Hu Y, Ni J, Jiao B, Fang L, Li J, Shen L, Tang B

**GEN-18 Genotype characteristics of patients with ALS in eastern China**

Zhao H, Niu Q

**GEN-19 Screening of the OPTN mutations in Chinese ALS patients**

Yang L, Cheng Y, Jia X, Liu X, Liu M, Cui L, Li X

**GEN-20 ALS-associated TBK1 variant p.G175S is unable to phosphorylate p62 serine residue 403 and fails to promote NF- $\kappa$ B signalling**  
Foster A D, Rea S

**GEN-21 SQSTM1 variants in familial ALS patients**

Yilmaz R, Müller K, Brenner D, Borck G, Volk A E, Hermann A, Meitinger T, Strom T M, Ludolph A C, Andersen P M, Weishaupt J H

**GEN-22 Investigating the genetics of ALS in a multi-ethnic Malaysian cohort**

Edgar S, Ahmad-Annar A, Ellis M, Aziz N A A, Goh K-J, Loh E C, Latif L A, Capelle D, Kennerson M L, Shahrizaila N

**GEN-23 First de novo SOD1 pathogenic variant in Korean patient with sporadic ALS**

Kim Y-E, Oh K-W, Park J, Ki C-S, Kim S H

**GEN-24 Identity by descent analysis of Australian SOD1 mutation carriers links sporadic ALS cases to ALS families and uncovers founder events**

Henden L, Twine N A, Szul P, McCann E P, Nicholson G A, Rowe D B, Kiernan M, Bauer D C, Blair I P, Williams K L

**GEN-25 Genetic variation in known ALS genes is prevalent among Australian sporadic ALS cases**

McCann E P, Fifita J A, Henden L, Grima N, Twine N A, Bauer D C, Kiernan M, Nicholson G A, Rowe D B, Williams K L, Blair I P

**GEN-26 Frequency and methylation status of active L1s in ALS**

Pfaff A L, Lopez A I, Bubb V J, Iacangeli A, Smith B, Schumann G G, Koks S, Al-Chalabi A, Quinn J P

**GEN-27 Genetic origin of ALS: a somatic or germline process?**

Borrego-Hernández D, Rodal-González I, Martín-Hondarza A, Pastor-López A B, García-Salamero G, González-Alvarez V, Lucas-Gómez B, Cordero-Vázquez P, Martín-Casanueva M A, Esteban-Pérez J, Rábano A, García-Redondo A

**GEN-28 Using Next Generation Sequencing and functional analysis to discover novel ALS genes and variants**

Fat S C M, Fifita J A, McCann E P, Yang S, Williams K, Twine N, Bauer D, Rowe D, Nicholson G A, Blair I

**GEN-29 Partitioning the genetic architecture of ALS**

Broce I J, Nillo R M, Fan C C, Olney N T, Lomen-Hoerth C, Finkbeiner S, Atassi N, Cudkowics M E, Paganoni S, Yokoyama J S, van Rheenen W, Veldink J H, Al-Chalabi A, Andreassen O A, Dale A M, Seeley W, Sugrue L, Ofori-Kuragu A, Miller B L, Desikan R S

**GEN-30 Molecular basis of upper motor neuron degeneration**

Ozdinler H

**IVT-02 High-content screening platform to screen potential Rab1 therapeutic candidates to treat MND**

Mehta P, Parakh S, Vidal M, Jagaraj S J, Shahheydari H, Abbott B, Laird A, Atkin J

**IVT-03 ALS2 along with a novel ALS2 interacting protein rab30 regulates morphological integrity and functions of the golgi apparatus**

Otomo A, Onodera W, Murakoshi S, Matsui K, Sato K, Mitsui S, Ono S, Fukuda M, Hadano S

**IVT-04 Sorting cells with ALS-associated protein aggregation phenotypes for genome-wide CRISPR screening**

Gil R S, Venturato J, Hedl T, Zhao Q, Edson J, Walker A K

**IVT-05 Development and validation of Cas9 neuroblastoma stable cell lines for CRISPR knockout and activation studies of the mechanisms involved in MND pathogenesis**  
Venturato J, Gil R S, Walker A K

**IVT-06 CRISPR-Cas9 mediated introduction of the TDP-43 A382T mutation produces iPSC-derived motor neurons with ALS-like pathology**  
Tracey T J, Ovchinnikov D A, Wolvetang E J, Ngo S T

**IVT-07 Optimisation of a method for the direct reprogramming of patient fibroblasts into lower motor neurons**

Tracey T J, Abernathy T G, Steyn F J, McCombe P A, Henderson R D, Ovchinnikov D A, Yoo A S, Wolvetang E J, Ngo S T

**IVT-08 The impact of Ryanodine receptor on organelle function in iPSC-derived ALS motor neurons**

Vlad B, Tadic V, Gaur N, Stubendorff B, Witte O W, Hermann A, Grosskreutz J

**IVT-09 Pathogenic and functional effects of the F115C Matrin 3 mutation in motor neurons derived from patient iPSCs**

Medina D X, Dominick M, Bowser R

**IVT-10 Mutant protein aggregates, mitochondrial impairment, and calcium dysregulation in motor neurons derived from induced pluripotent stem cell lines of Chinese family ALS patients carrying SOD1 mutations**  
Deng M, Liu W

**IVT-11 MSC-NTF differentiation increases the neurotrophic effects of MSC cells: live imaging analysis**

Semo J, Kaspi H, Abramov N, Lebovits C, Gothelf Y, Aricha R, Kern R

**IVT-12 Elucidating mechanisms of TDP-43 toxicity in embryonic stem cell derived motor neurons**

Carroll E, Gordon D, Candalija A, Talbot K

**IVT-13 Concurrent hypomorphic cytoplasmic dynein and reduced TBK1 function exacerbate formation of stress granules and p62 protein aggregates and compromise their autophagic clearance**

Gourabi M H, Simoes F A, Hafezparast M

**IVT-14 TDP-43 mutation affects stress granule assembly and disassembly in NSC34 motor neurons**

Ding Q, Ng D, Hilliard M A, Wolvetang E, Noakes P G

**IVT-15 TIA1 interacts with mutant SOD1 and affects stress granule dynamics in ALS**  
Jeon G S, Yang J W, Lee K-W, Sung J-J

**IVT-16 Neuropeptide Y reduces cell death in vitro via a cleaved caspase 3 dependent mechanism in SOD1G93A model of ALS**  
Clark C M, Clark R M, Dickson T

**IVT-17 Promotion of the maturation of SOD1-FALS mutants using small molecules**  
Shephard V, McAlary L, Wright G SA, Yerbury J J

**IVT-18 Layer V Pyramidal Neurons with synaptic hyper-excitability show altered TrkB receptor signaling in the SOD1G93A mouse model of ALS**  
Pradhan J, Nakes P G, Bellingham M C

**IVT-19 Pharmacological autophagy induction causes reductions in the levels of FUSP52L and amelioration of SOD1A4V aggregation in cell culture ALS models**  
Lambert-Smith I A, Tym M, Luu L, Watchon M, Yerbury J J, Don E K, Laird A S

**IVT-20 Increased levels of a key UPS protein reduce mutant SOD1 toxicity but have no effect on mutant SOD1 aggregation**  
Lambert-Smith I A, Farrarwell N E, McAlary L, Vine K L, Ecroyd H, Saunders D N, Yerbury J J

**IVT-21 Ubiquitin homeostasis is disrupted in ALS**  
Farrarwell N E, Lambert-Smith I A, Vine K L, Saunders D N, Yerbury J J

**IVT-22 Cyclin F interacts with autophagy protein sequestosome-1/p62 elucidating a novel molecular mechanism**

Davidson J M, Cheng F, Rayner S L, Chung R S, Lee A

**IVT-23 EPI-589, a novel redox-active protectant against oxidative cell injury and poly glycine-alanine aggregation in cellular models of ALS-related pathophysiology**

Motodate R, Matsumoto Y, Nashida T, Goto N, Yamana Y, Miyoshi K, Kondo Y, Tanaka T, Isobe Y, Yamanaka M, Trimmer J, Ishiyama T

**IVT-24 Neuronal toxicity and mitochondrial protection in ALS-related Sigma-1 receptor missense mutation**

Fukunaga K, Fukunaga R, Shinoda Y

**IVT-25 A microdevice-based method for quantifying endolysosomal and mitochondrial axonal transport in neuron derived from a mouse model of ALS**

Otomo A, Kushida T, Ishida T, Araki R, Sato K, Mitsui S, Ono S, Kimura H, Hadano S

**IVT-26 Single copy expression of mutant TDP-43 increases microglial reactivity and motor neuron vulnerability to inflammatory stimuli**  
Clark J, Kelfkens M, Geist E, Gray E, Gordon D, Talbot K

**IVT-27 Inflammatory response to TDP-43 knockdown in human leptomeningeal tissue**  
Naidoo S, Scotter E L, Dragunow M

### THEME 3

#### In vitro experimental models

**IVT-01 A functional pipeline for the validation of novel ALS candidate genes**

Yang S, Wu S, Fifita J, McCann E, Fat S C M, Galper J, Freckleton S, Zhang K Y, Blair I P

**IVT-28 Impaired NHEJ repair in ALS is associated with TDP-43 mutations**

Konopka A, Whelan D R, Perri E, Shahheydari H, Jamali S, Toth R P, Parakh S, Mehta P, Vidal M, Ragagnin A MG, Khizhnyak I, Jagaraj C J, Shadfar S, Bel T D M, Walker A, Atkin J D

**IVT-29 WITHDRAWN****IVT-30 SQSTM1/p62 expression induces TDP-43 cytoplasmic mislocalisation and cleavage**

Foster A, Clunis C, Lee A, Rea S L

**IVT-31 Exosomes secreted by diseased ALS cerebral cortex include messages to modulate disease progression**

Ozdinler H, Gautam M, Xie E, Brent J, Thaxton S

**IVT-32 ALS-associated mutations in the TDP-43 low-complexity domain have variable effects on its liquid-liquid phase separation properties**

McAlary L, Heydari T, Hoggarth J, Peng X, Cashman N R, Plotkin S S

**IVT-33 Using fluorescence microscopy techniques to structurally characterise TDP-43 inclusions present in a mammalian cell model**

Matharu N S, Hanspal I M A, Ng1 J S W, Wilson M R, Yerbury J J, Kaminski C F, Dobson C M, Kaminski-Schierle G S, Kumita J R

**IVT-34 Characterisation of the role of Annexin A11 in ALS biology using two complementary in vitro models**

Patel R, Glennon E, Vance C, Smith B

**IVT-35 ANXA11 mutations in ALS cause dysregulation of calcium homeostasis and stress**

Lim S M, Nahm M, Kim Y-E, Park J, Noh M-Y, Lee S, Oh K-W, Kim S H

**IVT-36 Neuronal expression and function of C-type lectin domain family 4 Member C, CLEC4C**

Nahm M, Lim S M, Noh M-Y, Kim Y-E, Oh K-W, Kim S H

**IVT-37 The TGF-beta induced muscle fibrosis and wasting in myoblast as in ALS mouse might be inhibited by pirfenidone**

Kwon Y N, Lee D Y, Baek H, Min Y G, Choi S-J, Ban J-J, Jeon G S, Hong Y-H, Sung J-J, Lee K-W

**IVT-38 In vitro measurement of molecular vibrational spectra of TDP25-based protein aggregates in culture cells**

Nagashima Y, Iwata A, Toda T

**IVT-39 A novel mutation of endoplasmic reticulum protein PDZD8 in the progression of ALS**

Li C, Chen Y, Chen X, Wei Q, Cao B, Shang H

**THEME 4****In vivo experimental models****IVV-01 Use of Zebrafish for the functional validation of ALS-associated genetic variants**

Chudinova A V, Rossel M, Vergunst A, Le-Masson G, Camu W, Raoul C, Lumbroso S, Mouzat K

**IVV-02 In vivo analysis of variant pathogenicity for the validation of novel MND linked genes**

Hogan A, Fifita J, Yang S, McCann E, Grima N, Blair I P

**IVV-03 An inducible Zebrafish model of sporadic neurodegenerative disease**

Don E K, Horte E, Hogan A L, Formella I, Morsch M, Lucus C W, Winnick C G, Scherer N, Vidal-Itriago A, Chow S, Chung R S, Nicholson G A, Cole N J, Laird A S

**IVV-04 Investigating synaptic dysfunction in a C9orf72 loss of function Zebrafish model**

Butti Z, Giacomotto J, Patten K

**IVV-05 Heterozygous TBK1 deletion in a TARDBP mutant mouse model**

Sieverding K, Ulmer J, Bruno C, Satoh T, Akira S, Tsao W, Wong P, Ludolph A C, Brenner D, Weishaupt J H

**IVV-06 Characterization of mice with heterozygous TBK1 deletion**

Bruno C, Sieverding K, Freischmidt A, Brenner D, Ludolph A C, Weishaupt J H

**IVV-07 TDP-43 mutant lacking its C-terminal domain induces age-dependent motor dysfunction in mice**

Watanabe S, Nishino K, Murata Y, Oiwa K, Yamanaka K

**IVV-08 Elevated expression of the DNA/RNA-binding protein FUS in astrocytes induces reactivity and spinal motor neuron death in vivo**

McAvoy K, Jensen B K, Heinsinger N, Mayer K, Westergard T, Lepore A C, Haeusler A, Trott D, Pasinelli P

**IVV-09 Establishing a novel mouse model of sporadic ALS using a chemogenetic approach**

Haidar M, Cuic B, Rytova V, Luikinga S, Turner B

**IVV-10 Hyperexcitable layer 5 pyramidal neurons in a model of cortical TDP-43 mislocalisation**

Dyer M S, Lewis K E, Dickson T C, Woodhouse A, Blizzard C A

**IVV-11 Beneficial effects of EPI-589, a novel redox-active protectant against oxidative stress, on motor function, spinal motor neuron pathology and biomarkers of oxidative stress and neurodegeneration in wobbler mouse MND**

Nashida T, Matsumoto Y, Motodate R, Goto N, Yamana Y, Nakada S, Kitaichi M, Fujii Y, Tani N, Matsushita H, Yamanaka M, Ishiyama T

**IVV-12 Co-administration of EPI-589, a novel redox-active protectant against oxidative stress, and riluzole remarkably enhanced the efficacy of either drug for motor function deterioration in wobbler mouse MND**

Matsumoto Y, Nashida T, Motodate R, Goto N, Yamana Y, Fujii Y, Tani N, Matsushita H, Yamanaka M, Ishiyama T

**IVV-13 Investigating the role of oxidative stress in spinal motor neurons in a zebrafish model of ALS**

Scherer N M, Formella I, Vidal-Itriago A, Don E K, Svahn A J, Graeber M B, Chung R S, Morsch M

**IVV-14 The pathogenesis of chlorovirus infection in SOD1G93A transgenic mice and clinical implications for ALS**

Pattee G L, Petro T M, Dunnigan D D, Agarkova I, Van Etten J L

**IVV-15 A protective role for complement C3aR activation in ALS**

Lee J D, McDonald T S, Woodruff T M

**IVV-16 Microglia specific and disease associated protein signature in ALS**

Barreto-Núñez R, Béland L-C, Boutej H, Barbeito L, Kriz J

**IVV-17 In vivo study of microglial-mediated clearance of disease-related TDP-43 aggregates**

Vidal-Itriago A, Svahn A J, Scherer N M, Radford R A, Don E K, Chung R, Graeber M, Morsch M

**IVV-18 Oral glutathione administration rescues neurons by reduced neuroinflammation in Alzheimer's mice**

Fukunaga R, Izumi H, Sato K, Fukunaga K

**IVV-19 Motor neuron morphogenesis is controlled by phosphoinositol signalling to the actin cytoskeleton**

Buttigieg D, Jacquier A, Tian Z, Blanchard S, Barad M, Gentien D, De La Grange P, Medina I, Bohl D, Erb M, Santini J, Haase G

**IVV-20 Enhancing glycosphingolipid metabolism improves motor function in mutant TDP-43 mice**

Luikinga S J, Henrikes A, Ngo S T, Loeffler J P, Spedding M, Turner B J

**IVV-21 In vivo dampening the metabotropic glutamate receptor 5 activity normalizes the reactive phenotype of spinal cord astrocytes isolated from late-symptomatic SOD1G93A mice**

Bonifacino T, Milanese M, Torazza C, Provenzano F, Ravera S, Usai C, Bonanno G

**IVV-22 Defective glucose metabolism in the brain and spinal cord of the SOD1G93A mouse model of MND**

Tefera T W, Bartlett K, Tran S S, Hodson M P, Ngo S T, Borges K

**IVV-23 Impaired glucose handling contributes to skeletal muscle pathology in the SOD1G93A mouse model of MND**

McDonald T S, Woodruff T M, Lee J D

**IVV-24 Resistant neuromuscular junction of extraocular muscle in ALS: a comparative analysis with vulnerable muscle in ALS mouse model**

Provost F, Nadeau R, Arbour D, Gauthier M-S, Lavallée-Adam M, Coulombe B, Robitaille R

**IVV-25 Transmission of human mutant SOD1 across the synapse involves exocytosis**

Cashman N R

**IVV-26 Transmissibility of SOD1 prion strains between mice expressing different mutant human SOD1s**

Brannstrom T, Bidhendi E E, Anderson P M, Marklund S L

**IVV-27 Identifying novel roles for Protein Disulfide Isomerase (PDI) in ALS**

Shadfar S, Shahheydari H, Parakh S, Laird A S, Atkin J

**IVV-28 BDNF-regulation of in vivo axonal transport is impaired in SOD1G93A mice**  
Tosolini A P, Sleigh J N, Surana S, Cahalan S D, Negro S, Schiavo G

#### IVV-29 WITHDRAWN

**IVV-30 Interplay of necroptotic and ferroptotic motor neuron death in ALS**  
Wang T, Perera N, Murphy J, Turner B

**IVV-31 Comparison study of the anatomical redistribution of essential metals in the embryonic and adult CNS of SOD1 overexpressing mice**  
Kysenius K, Hilton J B, Paul B, Hare D J, Crouch P J

**IVV-32 Does mislocalised TDP-43 in excitatory neurons of the motor cortex cause ALS-like pathology in the spinal cord?**  
Reale L A, Lewis K EA, Dyer M S, Handley E E, Dickson T C, Blizzard C A

**IVV-33 Investigating the role of TDP-43 recruitment to stress granules in a mouse model of ALS/FTD**  
Wardley G FB

**IVV-34 Modeling gene-toxicant interactions in ALS using regulatory elements as novel risk factors**  
Morrice J R, Smith M, Hancock R EW, Gregory-Evans C Y, Shaw C A

**IVV-35 Investigation of HSF1 gene therapy in a mouse model of ALS/FTD**  
McLoughlin C J

**IVV-36 Enhanced survival of a transplanted MSC sheet on the mouse cerebrum**  
Honda N, Watanabe Y, Nakanishi M, Tokuoka Y, Terashima T, Katagi M, Hanajima R

**IVV-37 Network aberrations in iTDP-43A315T mouse model of ALS/FTD**  
van Hummel A, Ittner L M, Bi M, Chieng B, Müller J, Ke Y D

**IVV-38 Investigating NPY receptors in SOD1G93A transgenic mice and human ALS tissue - pathogenic and therapeutic relevance of the endogenous Neuropeptide Y system**  
Clark R M, J A Hoyle, Clark C M, Lewis K EA, Blizzard C A, Dickson T C

**IVV-39 Role of C9orf72 in inflammation and neurodegeneration**  
McCauley M E, O'Rourke J, Bell S, Valencia V, Markman J, Ardit M, Ho R, Yanez A, Jefferies C, Baloh R H

**IVV-40 Gain of toxicity from ALS/FTD-linked repeat expansions in C9ORF72 mouse model**  
Arias N, Mueller S, Shaw C

**IVV-41 Converging pathological mechanisms in ALS-FTD: RNA dysregulation in a endosomal dysfunction model of ALS-FTD**  
Fort L, Sweeney S T

**IVV-42 tRNA modification defects in mouse models of ALS**  
Hogg M C, Richter F M, Woods I, Helm M, Prehn JHM

**IVV-43 Longitudinal transcriptomic analysis of altered pathways in a CHMP2Bintron5-based model of ALS-FTD**

Waegaert R, Dirrig-Grosch S, Keime C, Henriques A, Loeffler J-P, René F

Suzuki N, Akiyama T, Ishikawa M, Kawada J, Fujii T, Mitsuzawa S, Ikeda K, Funayama Ryo, Nakayama K, Fujishima F, Mitsuhashi H, Warita H, Okano H, Aoki M

#### HCB-13 The pathogenic role of a new FUS isoform in ALS

Vidal M, Ragagnin A MG, Lee A, Heng B, Sundaramoorthy V, Chung R, Guillemin G J, Atkin J D

**HCB-14 Assessing TDP-43 and RNA dynamics: patient induced pluripotent stem cell derived motor neurons as a tool for investigating Annexin A11 associated ALS**  
Hedges E C

#### HCB-15 A multicentric approach to monocyte alterations in ALS

Brockmann S J, Ribon M, Masrori P, Bhatia D, Brenner D, Hesters A, Ludolph A C, Danzer K M, Salachas F, Van Damme P, Boillée S, Weishaupt J H

#### HCB-16 Mutations in the glycosyltransferase domain of GLT8D1 are associated with familial ALS

Moll T A, Cooper-Knock J, Hautbergue G, Ramesh T, Higginbottom A, Castelli L, Kirby J, Shaw P

#### HCB-17 Premature polyadenylation and loss of the neuronal stathmin-2 inhibits axonal regeneration in TDP-43-dependent neurodegeneration

Melamed Z, Lopez-Erauskin J, Baughn M, Zhang O, Drenner K, Sun Y, Freyermuth F, Wu D, Bennett F, Rigo F, Da Cruz S, Ravits J, Cleveland D, Lagier-Tourenne , C

#### HCB-18 Phosphorylation state of ALS2/ALSIN alters its intracellular localization and endosome dynamics

Shimakura K, Sato K, Mitsui S, Ono S, Otomo A, Hadano S

#### HCB-19 Genetic and immunopathological analysis of CHCHD10 in Australian ALS and FTD

Yang S, McCann E P, Fifita J A, Grima N, Galper J, Mehta P, Freckleton S, Zhang KY, Hogan A, Chan S, Henden L, Williams K, Twine N, Bauer D, Kwok J, Halliday G, Kiernan M, Rowe D B, Nicholson G A, Walker A K, Blair I P

#### HCB-20 WITHDRAWN

#### HCB-21 Predicting disease-specific spinal motor neurons and glia in sporadic ALS

Song F, Dachet F, Liu J, Ravits J

#### HCB-22 Elemental and structural characterisation of SOD1 aggregates in the human ALS spinal cord

Genoud S, Jones M WM, Hare D J, Double K L

#### HCB-23 Abnormal accumulation of citrullinated proteins in ALS/PDC of the Kii peninsula of Japan

Kokubo Y, Morimoto S, Sasaki R, Kuzuhara S, Ishigami A

#### HCB-24 RNA trafficking is disrupted in oligodendrocytes in the motor cortex white matter in ALS patients

Barton S K, Gregory J M, McDade K, Turner B J, Smith C, Chandran S

## THEME 5

### Human cell biology and pathology

**HCB-01 ALS: a complex disease needs a validated early diagnostic tool**

Giesecker A, Hillert R, Krusche A, Zacher K H

**HCB-02 A large-scale library of patient derived iPSC lines to accelerate ALS research**

Bye C, Daniszewski M, Qian E, Lim K, Liang H, Needham M, Fletcher S, Mathers S, Pebay A, Hewitt A, Turner B

**HCB-03 Developing cell lines from distinct ALS/MND disease presentations: understanding disease heterogeneity**

Rosenfeld J, Shi Y, Hung S-T, Rocha G, Lin S, Linares G, Staats K A, Seah C, Wang Y, Chickering M, Lai J, Sagare A P, Zlokovic B V, Ichida J K

**HCB-04 Modelling cortical motor neuron pathology in induced pluripotent stem cell derived neurons from patients with ALS**  
Chen Z S, Dafinca R, Patani R, Talbot K

**HCB-05 Vesicle trafficking pathways are dysregulated in C9orf72 iPSC-derived motor neurons**

Candalija A, Vahsen B, Barbagallo P, Scaber J, Dafinca R, Webber C, Haase G, Talbot K

**HCB-06 Expression of poly(GA) and poly(PR) in iPSC-derived motor neurons induces ALS-related phenotypes via different pathways**  
Barbagallo P, Candalija A, Dafinca R, Talbot K

**HCB-07 Novel method to detect DNA damage in ALS**

Brocardo M G, Rizos H, Atkin J

**HCB-08 C9orf72 is an actin binding protein involved in regulation of actin dynamics**

Jagaraj C J, Sundaramoorthy V, Walker A K, Atkin J D

**HCB-09 Nucleocytoplasmic transport defects are induced by mutant cyclin F in ALS/FTD**

Atkin J D, Ragagnin A MG, Shadfar S, Vidal M, Lorenc F, Sundaramoorthy V

**HCB-10 Investigating changes in neuronal excitability using iPSC-derived motor neurons from ALS patients**

Do-Ha D, Sanz Munoz S, Stevens C H, Cabral-da-Silva M C, Bax M, Balez R, Kalajdzic P, Lisowski L, Nicholson G, Yang S, Blair I, Engel M, Buskila Y, Ooi L

**HCB-11 Sequestration of RNA helicase DHX30 from mitochondrion is linked to mitochondrial dysfunction in ALS-FUS**

Hikiami R, Minamiyama S, Asada M, Wada H, Shodai A, Morimura S, Ayaki T, Takahashi R, Urushitani M

**HCB-12 Elucidating axonal pathology of fused in sarcoma (FUS)-mutant ALS motor neurons using microfluidic devices**

**HCB-25 CNS-derived extracellular vesicles from superoxide dismutase 1 (SOD1) G93A ALS mice originate from astrocytes and neurons and carry misfolded SOD1**

Silverman J M, McAlary L, Mackenzie I R, Foster L F, Cashman N R

**HCB-26 Non-genetic origins of SOD1 protein misfolding in post mortem familial and sporadic ALS**

Trist B G, Cottam V, Genoud S, Roudeau S, Fifita J A, Carmona A, Blair I P, Ortega R, Hare D, Double K L

**HCB-27 Copper malfunction is a pervasive feature of the sporadic MND spinal cord**

Hilton J B, Kysenius K J, Liddell J R, Rautengarten C, Mercer S W, McLean C A, Hare D J, Roberts B R, White A R, Crouch P J

**THEME 6****Tissue biomarkers****BIO-01 Is the problem within the gut instead of the brain? Impairment of dietary glutamate metabolism identified in patients with ALS**

Garnaas K R, Kittelsrud J, Behnke M

**BIO-02 Vitamin levels in patients with ALS in central south China**

Wang J, Sun W, Liu Z, Yuan Y, Ni J, Li W, Hu Y, Jiao B, Fang L, Li J, Shen L, Tang B

**BIO-03 The vitamin D activator, CYP27B1, is a novel muscle biomarker of ALS disease progression**

Si Y, Kazamel M, Kwon Y, Lee I, Bamman M, Wiggins D, Zhou S, King P H

**BIO-04 Longitudinal analysis of CSF biomarkers for ALS**

Vu L, An J, Garcia-Mansfield K, David-Dirgo V, Sharma R, Pirrotte P, Bowser R

**BIO-05 Phosphoneurofilament heavy chain and IgG N-glycosylation as cerebrospinal fluid biomarkers for ALS**

Costa J, Streich L, Pinto S, Laborinho-Pronto A, Nitzm T, Conradt H S, de Carvalho M

**BIO-06 A panel of three ratios of four molecules as cerebrospinal fluid biomarker for ALS in a Brazilian cohort**

Maximino J R, Busser F D, Jorge F M H, Gomes H R, Fortini I, Palmisano G, Chadi G

**BIO-07 Significance of TDP-43, NfL, and tau in plasma and CSF as diagnostic and prognostic biomarkers of ALS**

Ohmichi T, Kasai T, Tatebe H, Kojima Y, Noto Y, Tsuji Y, Shinomoto M, Mizuno T, Tokuda T

**BIO-08 Biomarkers in cerebrospinal fluid for diagnosis and prognosis in ALS**

Rosen H, Constantinescu R, Axelsson M, Mitre B, Nilsson G, Zetterberg H

**BIO-09 Significant out-of-sample classification from methylome-wide association study of ALS**

Nabais M F

**BIO-10 Characterising the role of SARM1 in ALS axon degeneration**

Perry S E, Atkinson R A K, Collins J M, King A E

**BIO-11 Biomarker analysis in oral levosimendan phase 2 clinical trial LEVALS**

Dickens A M, Sarapohja T, Serkkola E, Garratt C, Holmström K M, on behalf of the LEVALS study group

**BIO-12 Chitinases as markers of neuroinflammation in ALS**

Gaur N, Perner C, Perner F, Vlad B, Steinbach R, Heidel F, Duduskar S, Huss E, Metzner K, Witte O W, Prell T, Grosskreutz J

**BIO-13 C-Reactive protein and disease aggressiveness and progression in ALS: retrospective application of the D50 model**

Gaur N, Vlad B, Witte O W, Prell T, Grosskreutz J

**BIO-14 Exploration of N-Alkylated Carbazole Translocator Protein (TSPO) ligands for detection of glial activation**

Cheng H W A, Sokias R, Werry E L, Ittner L M, Reekie T A, Kassiou M

**BIO-15 Increased Interleukin-6 levels in the astrocyte-derived exosomes of sporadic ALS patients**

Chen Y, Xia K, Fan D

**BIO-16 Cardiac troponins as biomarkers in ALS**

Castro-Gomez S, Mirandola S R, Radermacher B, Binder J, Heneka M T, Weydt P

**BIO-17 WITHDRAWN****THEME 7****Pre-clinical therapeutic strategies****TST-01 The potential of neurofilaments analysis using dry-blood and plasma spots**

Lombardi V, Carassiti D, Giovannoni G, Lu C-H, Adiutori R, Malaspina A

**TST-02 UCHL1 is necessary and sufficient for maintaining cytoarchitectural integrity of upper motor neurons**

Ozdinler H, Genc B, Jara J, Kocak N, Zhu Y

**TST-03 Incorporation of upper motor neurons in drug discovery efforts**

Ozdinler H

**TST-04 Ambroxol hydrochloride improves motor functions and extend survival in a mouse model of familial ALS**

Bouscary A, Quessada C, Mosbach A, Callizot N, Spedding M, Loeffler J-P, Henriques A

**TST-05 Investigating the therapeutic potential of miR-23a inhibition in TDP-43ΔNLS ALS mice**

Tsitsikou S, Foletta V, Della Gatta P, Gerlinger Romero F, Wallace M, Walker A, Russell A

**TST-06 Targeting ATXN2 with artificial microRNA as gene therapy for sporadic ALS**

Freckleton S E, Lee Y, Shaw C E

**TST-07 Investigating antisense oligonucleotide therapeutics for ALS**

Mejzini R, Flynn L L, Fletcher S, Wilton S D, Akkari A

**TST-08 Pre-clinical development of a genetic therapy for SOD1 ALS**

Flynn L, Tomas D, Barton S, Pitout I L, Swanson T, Fletcher S, Metz C, Wilton S D, Turner B J, Akkari A

**TST-09 Antisense oligonucleotide modulation of selected RNA-binding proteins to reduce the severity of SMA**

Pitout I L, Flynn L L, Fletcher S, Wilton S D

**TST-10 WITHDRAWN****TST-11 Cu<sup>II</sup>(ATSM) potently inhibits neuronal ferroptosis: implications for ALS pathogenesis**

Bush A I, Southon A, Crouch P, Donnelly P, Barnham K

**TST-12 Ferroptotic stress induces neurotoxic astrocyte activation in ALS: A potential therapeutic target for CuATSM**

Liddell J R, Hilton J B, Kysenius K, Mercer S, McLean C A, Donnelly P S, Szostak K, Lam L, Roberts B R, Hare D J, White A R, Bush A I, Crouch P J

**TST-13 A novel cell transplantation therapy for familial ALS using oligodendrocyte precursor cells expressing scFv specific for misfolded SOD1**

Minamiyama S, Sakai M, Yamaguchi Y, Hikami R, Tamaki Y, Shodai A, Makino A, Maki T, Tomonaga K, Takahashi R, Urushitani M

**TST-14 The identification of scFv biomolecules that bind to TDP-43 and prevent its induced aggregation as a potential therapy for ALS**

Hergesheimer R C, Chami A, Vourc'h P, Andres C, Corcia P, Martineau P, Reis de Assis D, Lanznaster D, Blasco H

**TST-15 Therapeutic potential of the Bornavirus X protein and X-derived peptide in ALS**

Chevallier S, Leger C, Cabelguen J-M, Delignat Lavaud B, Irubetagoyena P, Klonjkowski B, Gonzalez-Dunia D, Szelechowski M, Le Masson G

**TST-16 The homeoprotein transcription factor ENGRAILED 1 modulates motor neuron physiology and survival**

Vargas Abonce S E, Leboeuf M, Prochiantz A, Moya K L

**TST-17 Targeting ALS cortical excitability dysfunction through nasal delivery of neuropeptide Y**

Lewis K EA, Clark R M, Chuckowree J A, Hoyle J A, Blizzard C A, Dickson T C

**TST-18 Development of autophagy-inducing peptides as a potential therapy for MND**

Amin A, Perrera N, Turner B, Shabanpoor F

**TST-19 Developing new cell models and approaches to study the role of TDP-43 and autophagy in MND**

Keating S S J, San Gil R, Walker A K

**TST-20 WITHDRAWN****TST-21 A systematic review of the role of stem cells in pre-clinical models of ALS/MND**

Sane H, Pradhan R, Paranjape A, Kulkarni P, Varghese R, Badhe P, Gokulchandran N, Sharma A

**TST-22 Degeneration of ALS mouse neuromuscular junctions by a loss of synapse organizer and a treatment using human mesenchymal stem cells**

Badawi Y, Matsuda T, Tungtur S, Tanaka T, Soder R, Silva K, Shigemoto K, Yoshida T, Barohn R, Nishimune H

**TST-23 Combined bone marrow transplantation therapy of MNCs and growth factor expressing-MSCs for ALS**

Terashima T, Kobashi S, Watanabe Y, Nakanishi M, Honda N, Katagi M, Nakae Y, Ohashi N, Urushitani M, Kojima H

**TST-24 Immunogenes for targeted neurotrophic factor gene delivery to motor neurons *in vivo***

Rogers M-L, Subramaniam C, Haidar M, Turner B

**TST-25 Subcutaneous infusion of next generation neurotrophic factor MANF delays disease onset and increases survival in a SOD1 murine model of ALS**

Beckett L, Voutilainen M H, Saarma M

**TST-26 New strategy for blood-brain barrier crossing and brain disease therapy**

Shi B

**TST-27 Pre-clinical test of a gene therapy approach for familial ALS with SOD1 mutations**

Marais T, Cohen-Tannoudji M, Astord S, Biferi M-G

**TST-28 Viral strategies for pre-clinical testing in the TDP-43ΔNLS mouse model of MND**

Brown-Wright H, Chui K, Walker A

**TST-29 Treatment with HDAC inhibitors is protective for a transgenic zebrafish model of neurodegenerative disease**

Watchon M, Luu L, Lee A, Lambert-Smith I, Tym M, Don E K, Yuan K, Suddull H, De Luca A, Chung R, Nicholson G A, Laird A S

**TST-30 Restoration of histone acetylation ameliorates disease and metabolic abnormalities in a FUS mouse model of ALS**

Rossaert E, Pollari E, Jaspers T, Van Helleputte L, Jarpe M, Van Damme P, De Bock K, Moisse M, Van Den Bosch L

**TST-31 Understanding the molecular mechanism and finding a therapeutic solution: Structural studies of TDP-43 and Copper-Zinc Superoxide Dismutase**

Watanabe T, Wright G, Antonyuk S, Chantadul V, Amporndanai K, O'Neil P, Hasnain S

**TST-32 Engineering and enhancing the therapeutic potential of intrabodies targeting pTDP-43**

Fernandes A, Shaw C

**TST-33 Targeting of the regulatory balance between b-oxidation and glycolysis improves outcomes in the SOD1G86R mouse model of ALS**

Quessada C, Bouscary A, Ferri A, Valle C, Ngo ST, Loeffler J-P, Rene F

**TST-34 Pharmacological modulation of hypermetabolism: a promising therapeutic strategy in ALS**

Valle C, Scaricamazza S, Salvatori I, Giacovazzo G, Proietti D, Ciriminna G, René F, Loeffler J-P, Steyn F J, Ngo ST, Madaro L, Coccurello R, Ferraro E, Ferri A

**ST-35 Redox-enhancing nanocatalysis improves motor neuron survival *in vitro* and SOD1 mouse motor function and survival *in vivo***

Ho K S, Zhang Z, Richard J-P, Hotchkiss M T, Lee W, Mortenson P, Steinmetz L, Taga A, Merzlakoff M, Dorfman A R, Maragakis N J, Mortenson M G

**TST-36 Arimoclomol is neuroprotective and ameliorates both ALS and FTD pathology in mutant VCP mice and patient iPSC motor neurons**

Ahmed M, Harley J, Spicer C, Patani R, Greensmith L

**TST-37 Ca<sup>2+</sup>-activated K<sup>+</sup> channels modulate microglia affecting motor neuron survival in hSOD1G93A mice**

Cocozza G, D'Alessandro G, Garofalo S, Limatola C

**TST-38 CD34+ microglia precursors as therapeutic targets in ALS**

Kovacs M, Trias E, King P H, Si Y, Kwon Y, Beckman J S, Ibarburu S, Hermine O, Barbeito L

**TST-39 Post-paralysis treatment with the synthetic immunomodulator EOLO4 abrogates neuroinflammation and prolongs survival in a model of inherited ALS**

Ibarburu S, Trias E, Ingold M, Rodriguez-Duarte J, Galliussi G, López V, Kovacs M, Varela V, Baththyán C, Barbeito L

**TST-40 Identification of cellular targets of masitinib along the CNS motor pathways in ALS**

Trias E, King P H, Si Y, Kwon Y, Kovacs M, Ibarburu S, Beckman J S, Hermine O, Barbeito L

**TST-41 AI-led drug discovery identifies nilotinib as a lead compound for ALS**

Markus N, Stopford M, Myszcynska M, Richardson P, Rackham M, Mead R, Ferraiuolo L

**TST-42 Combination of acamprosate and baclofen: a potential new therapy for ALS**

Boussicault L, Laffaire J, Schmitt P, Rinaudo P, Callizot N, Cholet N, Nabirochkin S, Hajj R, Cohen D

**TST-43 Mechanism of action of the cardiovascular drug Levosimendan in the management of ALS**

Holmström K M, Pollesello P, Garratt C

## THEME 8

### Clinical imaging and electrophysiology

**IMG-01 The spectrum of involuntary movements in patients with MND: a cross-sectional study**

Vogelnik K, Alfonso R P, Koritnik B, Klavžar P, Leonardis L, Grošelj L D, Zidar J, Kojović M

**IMG-02 Impact of stimulus duration on detecting enlarged motor units in the compound muscle action potential scan**

Sleutjes B THM, Ruisch J, Goedee H S, van den Berg L H, Franssen H

**IMG-03 Reassessment of split-leg signs in ALS: differential involvement of the extensor digitorum brevis and abductor hallucis muscles**

Wang Z-L, Cui L, Liu M, Zhang K, Liu S, Ding Q, Hu Y

**IMG-04 Split-hand index for ALS: an F-wave study**

Wang Z-L, Cui L, Liu M, Zhang K, Liu S, Ding Q, Hu Y

**IMG-05 The split hand sign in spinal and bulbar muscular atrophy**

Shibuya K, Misawa S, Sekiguchi Y, Beppu M, Amino H, Tsuneyama A, Suzuki Y-I, Suichi T, Nakamura K, Kuwabara S

**IMG-06 Split finger syndrome in ALS**

Sono M, Takahashi K

**IMG-07 Relationship between EMG-detected fasciculation potentials and ultrasound-detected fasciculations in ALS: a prospective cohort study**

Bokuda K, Shimizu T, Morishima R, Kimura H, Kawata A, Nakayama Y, Isozaki E

**IMG-08 Fasciculations demonstrate daytime consistency in ALS**

Bashford J, Masood U, Wickham A, Iniesta R, Drakakis M, Boutelle M, Mills K, Shaw C

**IMG-09 Fasciculation analysis reveals a novel adverse predictor of survival in ALS**

Wannop K, Bashford J, Mills K, Shaw C

**IMG-10 Distribution patterns of fasciculations in ALS: a ultrasonographic study**

Suzuki Y-I, Shibuya K, Misawa S, Sekiguchi Y, Suichi T, Tsuneyama A, Nakamura K, Kano H, Kuwabara S

**IMG-11 Ectopic impulse generation in peripheral nerve hyperexcitability syndromes and ALS**

Noto Y-I, Simon N G, Selby A, Garg N, Vucic S, Kiernan M C

**IMG-12 Longitudinal assessment of individual muscle changes in MND**

Jenkins T M, Alix J JP, Fingret J, Esmail T, Hoggard N, Baster K, McDermott C J, Shaw P J, Wilkinson I D

**IMG-13 Assessment of upper motor neuron in ALS and MSA using CMCT**

Otsuka J, Shiota Y, Hamada M, Toda T

**IMG-14 MND with malignancy: a pathophysiological distinct entity of MND?**

Higashihara M, Menon P, Geevasinga N, Van den Bos M AJ, Kiernan M C, Vucic S

**IMG-15 Longitudinal magnetoencephalography in early stage ALS and behavioural variant of FTD: peak frequencies and relative power in six frequency bands**

Govaarts R, Beeldman E, Fraschini M, Pijnenburg Y AL, de Visser M, Stam C J, Raaphorst J, Hillebrand A

**IMG-16 Assessing cortico-muscular communication patterns in MND**

Coffey A, Bista S, Fasano A, Buxo T, Dukic S, McMackin R, Haverin M, Lowery M, Carson R, Nasseroleslami B, Hardiman O

**IMG-17 Developing biomarkers of focal network disruptions in ALS using threshold-tracking transcranial magnetic stimulation**

McMackin R, Fasano A, Tadjine Y, Mitchell M, Heverin M, Nasseroleslami B, Carson R, Hardiman O

**IMG-18 Quantifying cognitive and motor preparatory dysfunction in ALS using EEG and electrical source imaging**

McMackin R, Dukic S, Chipika R, Buxo T, Fasano A, Heverin M, Reilly R, Pender N, Bede P, Muthuraman M, Nasseroleslami B, Hardiman O

**IMG-19 Can MRI enable an earlier or more certain diagnosis of ALS? A multi-modal quantitative MRI-based diagnostic biomarker**

Welton T, Maller J J, Lebel M, Tan E T, Rowe D B, Grieve S M

**IMG-20 Subcortical abnormality is a prominent indicator of evolving cortical dysfunction in ALS**

Tu S, Kiernan M C, Turner M R

**IMG-21 Tract pathology in ALS correlates with the D50 disease progression model**

Steinbach R, Voss A, Gaur N, Mayer T E, Witte O W, Grosskreutz J

**IMG-22 Clinical correlation of MRI-based brain morphometry findings in motor and non-motor areas in ALS**

Barć K, Piątkowska-Jankó E, Maj E, Kuźma-Kozakiewicz M

**IMG-23 Cortical thickness, white matter graph network, and 18F-FDG PET reveal abnormal brain structure and function in ALS-FTD: neuronopathy or axonopathy?**

Pioro E P, Rajagopalan V

**IMG-24 Dystrophy pattern of motor cortex in ALS patients according to onset type**

Shen D, Hou B, Liu S, Zhang K, Yang X, Liu M, Cui L

**IMG-25 Gait initiation and motor preparation are impaired in ALS patients**

Abidi M, de Marco G, Couillardre A, Feron M, Mseddi E, Termoz N, Querin G, Bede P, Pradat P F

**THEME 9****Clinical trials and trial design****CLT-01 The NEALS Consortium – a collaborative research organization**

Lincoln T C, Allen C

**CLT-02 Understanding and incorporating the patient and caregiver perspective in clinical trials**

Proulx L, Madsen A, Daniel S, McKenzie M, McDougall F, Cantrell C, Hains A, Cho W

**CLT-03 The symptoms and impacts of MND/ALS from the patient perspective**

McDougall F, McKenzie M, Lewis S, DeMuro-Mercon C, Kerchner G, Cho W, Hains A, Heiman-Patterson T

**CLT-04 Engaging ALS research ambassadors to help design the REFINE-ALS biomarker study**

Berry J, Bedlack R, Mathews D, Agnese W, Apple S

**CLT-05 A platform trial for ALS: innovative trial design to drive ALS science and accelerate the path to effective treatments**

Paganoni S, Berry J, Shefner J, Andrews J, Chew S, Quintana M, Broglie K, Saville B, Macklin E, Vestrucchi M, Meurer W, Chase M, Pothier L, Harkey B, Gladden C, Berry S, Cudkowicz M

**CLT-06 Increasing the efficiency of ALS clinical trials using machine learning**

Taylor A A, Beaulieu D, Cuerdo J, Pierce D, Conklin A, Keymer M, Ennist D L

**CLT-07 The influence of clinical study inclusion criteria on baseline characteristics and disease progression in ALS**

Katz J, Perdrizet J, Apple S, Zhang J, Lu P, Agnese W

**CLT-08 Date of onset as an indicator and predictor of data quality in clinical research**

Sherman A, Sinani E, Cudkowicz M

**CLT-09 Tracking ALS progression in clinical trials with a mobile app measuring speech**

Hahn S, Stegmann G, Berisha V, Liss J M, Cockroft B M, Malik F I, Meng L, Rudnicki S, Wolff A A, Shefner J

**CLT-10 The impact of frequent sampling of new and established outcome measures: results of the ALS-at-home study**

Shefner J M, Shelton K, Qi K, Liss J, Berisha V, Rutkove S

**CLT-11 Remote longitudinal assessment of functional MND burden in a large United States registry**

Statland J, Karanovich A, Wuu J, Herbelin L, Barohn R J, Benatar M, on behalf of the CReATE Consortium3

**CLT-12 Blood-brain barrier disruption is associated with poor survival in ALS**

Vlad B, Prell T, Gaur N, Dreger M, Witte O W, Grosskreutz J

**CLT-13 Selecting appropriate outcome measures for ALS clinical trials**

Arjunji R, Glowienka E, Maru B, Wiesner T, Meriggioli M, Dabbous O

**CLT-14 Optimizing the ALSFRS-R as a clinical trial endpoint**

de Jongh A D, van Eijk R P, van den Berg L H

**CLT-15 Overhauling clinical trials for ALS: the Lighthouse II trial design**

van Eijk R PA, Roes K CB, Nikolakopoulos S, Gold J, van den Berg L H

**CLT-16 Design of the Phase 3, randomised, placebo-controlled trial of oral Arimoclomol in ALS: ORARIALS-01**

Sundgreen C, Blaettler T, Bennett R, Rom D, Wuu J, Andersen P M, Benatar M

**CLT-17 TUDCA ALS: A novel clinical trial design for disease progression**

Cocco A, Tornese P, Albanese A

**CLT-18 Baseline characteristics and status update of REFALS: A phase 3 study comparing oral levosimendan to placebo in patients with ALS**

Cudkowicz M, Genge A, Maragakis N, Petri S, van den Berg L, Aho V, Garratt C, Sarapohja T, Al-Chalabi A

**CLT-19 Study AB19001, a confirmatory, pivotal phase 3 trial of masitinib in ALS**

Genge A, Ludolph A

**CLT-20 Utilization of durable medical equipment in FORTITUDE-ALS**

Rudnicki S, Andrews J A, Genge A, Jackson C, Lechtzin N, Miller T M, Cockroft B M, Malik F I, Meng L, Wei J, Wolff A A, Shefner J

**CLT-21 Responder and subgroup analyses for FORTITUDE-ALS, a phase 2 trial of Reldesemtiv in patients with ALS**

Shefner J, Andrews J A, Genge A, Jackson C, Lechtzin N, Miller T M, Cockroft B M, Malik F I, Meng L, Wei J, Wolff A A, Rudnicki S

**CLT-22 Quality of life and depression measurements in FORTITUDE-ALS**

Rudnicki S, Andrews J A, Genge A, Jackson C, Lechtzin N, Miller T M, Cockroft B M, Malik F I, Meng L, Wei J, Wolff A A, Shefner J

**CLT-23 Impact of ALSFRS-R progression rates on outcome measures in FORTITUDE-ALS**

Rudnicki S, Andrews J A, Genge A, Jackson C, Lechtzin N, Miller T M, Cockroft B M, Malik F I, Meng L, Wei J, Wolff A A, Shefner J

**CLT-24 Clinical trial design for a Phase II, randomized, placebo-controlled trial of AMX0035 in ALS (CENTAUR)**

Paganoni S, Cohen J B, Klee J B, Leslie K L, Yeramian P

**CLT-25 Interaction (nonuniformity) of ALS progression and the efficacy of MN-166 (ibudilast)**

Matsuda K, Iwaki Y, Makhay M, Dojillo J, Yasui S

**CLT-26 Oral levosimendan for ALS: pharmacokinetic considerations**

Ahtola-Sätilä T, Sarapohja T, Aho V V, Kivikko M

**CLT-27 Estimate of an Acthar® Gel Treatment effect in ALS patients using virtual controls**

Beaulieu D, Cuerdo J, Taylor A A, VanMeter S, Zhao E, Keymer M, Ennist D L

**CLT-28 Safety and biological efficacy of narrow-band UVB phototherapy in ALS**

Vucic S C, Menon P, Fernandez-Péñas P, Byrne S, Turner B, Booth D R, Stewar G J, Fewings N, Gatt P, Vucic S

**CLT-29 Regulation of motor neuron neurotrophic molecules in autologous ALS bone marrow mesenchymal stem cells of the clinical trial NTC02917681 in relation to other cell types and tissues**

Gilio J M, Jorge G C, Maximino J R, Brofman P RS, Bydlowski S P, Senegaglia A C, Rebelatto C LK, Daga D R, Chadi G

**CLT-30 Clinical effects of two intrathecal infusions of autologous mesenchymal stem cells in ALS: a phase 1/2 clinical trial in a cohort of Brazilian patients**

Chadi G, Jorge F M H, Busser F D, Maximino J R, Lage L APC, Pereira J, Paiva W S, Callegari D, Carvalho C RR, Brofman P RS, Senegaglia A C, Rebelatto C LK, Daga D R

**CLT-31 Involvement of complement system in mesenchymal stem cell therapy for ALS**

Chadi G, Maximino J R, Reis A LG, Chouman I H, Busser F D, Palmisano G, Brofman P RS, Senegaglia A C, Rebelatto C LK, Daga D R

**CLT-32 Repeated intrathecal allogeneic bone marrow-derived mesenchymal stromal cells in ALS: investigator initiated trial**

Oh K-W, Noh M-Y, Kwon M-S, Park J, Lee S, Kim K S, Kim S H

**CLT-33 Can modulation of the gut microbiota confer disease-modifying benefit in ALS? Results of an open label, prospective trial of RaphaLX™ probiotic compound in ALS patients**  
Garnaas K R, Kittelsrud J, Behnke M

**CLT-34 A phase 1b, open-label, dose-escalation, safety and pharmacokinetic study of IC14, an antibody to CD14, in MND**  
Henderson R D, Heggie S, Thorpe K, Singh G, Appleby M W, Agosti J M, Crowe D T, Ziegelaar B, Redlich G L, McCombe P A

**CLT-35 Design of the Australian multicenter, randomized, double-blind, placebo-controlled study of CuATSM for treatment of ALS**  
Noel K, Curran J, Rowe D, Mathers S

**CLT-36 Bioequivalence of CuATSM capsule and oral suspension formulations: advancing CuATSM to randomized clinical trials in ALS**  
Woodworth J, Kuo J, Fitzpatrick S, Lappin R, Rosenfeld C S, Noel K

**CLT-37 Efficacy of edaravone in Korean ALS patients: open pilot study**  
Park J-S, Park J-M

**CLT-38 Surveillance of using a novel, free-radical scavenger, edaravone, to investigate a survival effect for ALS patients in Japan (SUNRISE Japan): interim report**  
Sobue G, Hirai M, Yuki S, Ishizaki K, Nakamura H

**CLT-39 Retaining physical function in ALS with edaravone: post hoc analysis of pivotal study MCI186-19**

Palumbo J, Apple S, Agnese W, Hirai M, Yoneoka T, Takahashi F, Takei K, Endo M

**CLT-40 Radicava/Edaravone findings in biomarkers from ALS (REFINE ALS): interim analysis**

Berry J, Brooks B, Genge A, Heiman-Patterson T, Apple S, Benatar M, Bowser R, Cudkowicz M, Gooch C, Shefner J, Apple S, Agnese W, Merrill C, Nelson S

**CLT-41 The impact of renal or hepatic impairment on the pharmacokinetics of edaravone after intravenous infusion**

Nakamaru Y, Kakubari M, Yoshida K, Akimoto M, Kondou K

**CLT-42 Effect of intravenous infusion of edaravone on QT/QTc interval**

Shimizu H, Inouke S, Endo M, Nakamaru Y, Yoshida K, Natori T, Kakubari M, Akimoto M, Kondo K

## THEME 10

### Disease stratification and phenotyping of patients

**DSP-01 Barriers to the diagnosis of MND – a South Australian study**  
Sharrad D F, Schultz D W

**DSP-02 Validation study of clinical diagnosis of ALS: The Brain Bank for Aging Research (BBAR) project**

Matsubara T, Izumi Y, Miyamoto R, Oda M, Nodera H, Higashihara M, Sengoku R, Oki R, Fujita K, Kawarai T, Watanabe C, Saito Y, Kaji R, Murayama S

**DSP-03 Investigating hidden Gelsolin amyloidosis which mimick slowly progressing MND using genetic test**

Park J, Kim Y-E, Lee S, Oh K-W, Nahm M Y, Il Jang D, Ki C-S, Kim S H

**DSP-04 Improvements in the definition of biomarkers for Spinal Muscular Atrophy (SMA) type III and IV: a multimodal longitudinal study**  
Querin G, Hogrel J-Y, Debs R, Marchand-Pauvert V, Stojkovic T, Behin A, Laforet P, Salachas F, Bede P, Lenglet T, Pradat P-F

**DSP-05 On-the-spot assessment of venous creatinine as a marker for change in fat-free mass and disease progression in patients with MND**

Ngo S T, Holdom K J, Lucia D, van Eijk R PA, van den Berg L H, McCombe P A, Henderson R D, Steyn F J

**DSP-06 The world according to the D50 model of ALS progression**

Grosskreutz J, Al-Chalabi A, van den Berg L, de Carvalho M, Chio A, Corcia P, Couratier P, van Damme P, Grehl T, Ingre C, Kiernan M, Koritnik B, Kuźma-Kozakiewicz M, Meyer T, Neuwirth C, Petri S, Talman P, Uysal H, Veldink J, Weber M, Wicks P, Zidar J

**DSP-07 Significant events during ALS progression according to the D50 model in the ONWebDUALS cohort**

Grosskreutz J, de Carvalho M, Fartmann F, Gromicho M, Kuźma-Kozakiewicz M, Petri S, Stubendorff B, Szacka K, Uysal H

**DSP-08 Structural and functional implications of cortical dysfunction on clinical disease progression in ALS**

Dharmadasa T, Wang C, Simon N G, Howells J, Kiernan M C

**DSP-09 Rapid reprogramming method differentiates CuATSM responders/nonresponders from ALS patient population**

Dennys-Rivers C, Zhang X, Rodrigo R, Estevez A, Kaspar B, Beckman J, Franco M C, Meyer K

**DSP-10 A personalized medicine approach for ALS/MND**

Rosenfeld J, Dey D, Yu S F, Macieik A, Selvarajah S, Paulvannan D, Lingappa V R

**DSP-11 The clinical phenotype of motor neuron diseases in Bangladesh**

Jenkins T M, Bandmann O, Hoque A, Alam B, Chowdhury J, Shaw P J, Mohammad Q D

**DSP-12 The Demographic and clinical characteristics of ALS in Malaysia**

Abdul-Aziz N A, Loh E C, Goh K-J, Latif L A, Capelle D, Shahrizaila N

**DSP-13 The genotype and phenotype spectrum of familial ALS in China**

Yang X, Zhang K, Liu S, Shen D, Liu Q, Liu M, Cui L

**DSP-14 Tracking bulbar impairment using the Beiwe smartphone app**

Connaghan K, Green J, Paganoni S, Chan J, Weber H, Collins E, Richburg B, Eshghi M, Onnela JP, Berry J

**DSP-15 Foreign Accent Syndrome (FAS) and ALS: a case report**

Sierra H N M, Borges R M, Frabasile L M, Alves P C L, Neves J W C, dos Santos Salvioni C C, Oliveira A S B, Oda A L

**DSP-16 What matters most to patients with ALS: initial validation of the ALS Health Index (ALS-HI), a multi-faceted patient reported outcome measure**

Zizzi C, Wagner E, Benatar M, Heatwole C

**DSP-17 Early treatment effects of Riluzole in ALS-MND 2: Isometric strength improvements in sentinel muscles**

Brooks B R, Braverman E K, Desai U G, Jalali N, Dawson W B, Bockenek W L

## THEME 11

### Cognitive and psychological assessment and support

**COG-01 Phenotypic variation in ALS-FTD and effect on survival**

Ahmed R M, Devenney E M, Strikwerda-Brown C, Hodges J R, Piguet O, Kiernan M C

**COG-02 Cognitive and behavioural changes predict poorer survival in ALS**

Nguyen C H, Caga J, Highton-Williamson E, Kiernan M C, Huynh W

**COG-03 Cognitive reserve as a mediator of cognitive decline in ALS**

Costello E, Ryan M, Pender N, Hardiman O

**COG-04 Do cognitive or behavioural problems explain satisfactory QoL in ALS?**

Lulé D, Schrempf T, Uttner I, Ludolph A C

**COG-05 Respiratory impairment is associated with cognitive dysfunction in ALS**

Huynh W, Sharplin L E, Caga J, Highton-Williamson E, Kiernan M C

**COG-06 Cognition and behavior in Japanese ALS patients**

Watanabe Y, Adachi T, Takigawa H, Hanajima R, ALS-FTD-Q-J Research Group

**COG-07 Clinical usefulness of neuropsychological scales for evaluating cognitive impairment in Japanese patients with ALS**

Nagashima K, Fujita Y, Ikeda M, Ikeda Y

**COG-08 A study of non-motor manifestations in patients with ALS**

Chowdhury A, Biswas A, Pandit A

**COG-09 Early treatment effects of Riluzole in ALS-MND 1: correction of hand grip apraxia in ALS-FTD**

Brooks B R, Braverman E K, Desai U G, Jalali N, Dawson W B, Bockenek W L, Lindblom S S

**COG-10 A systematic review of cognitive screening tools used in MND**

Mayberry E J, McTiffin M H, Tooth C L, McDermott C J

**COG-11 Development of the Online Carer's Questionnaire (OCQ) behaviour screen for MND**

Robinson G, Ceslis A

**COG-12 The role of relationships and social support on the psychological wellbeing of ALS caregivers**

Carney S, Wheeler C, Heverin M, Galvin M, Pender N, Hardiman O

**COG-13 Understanding the needs of ALS caregivers**

Carney S, Galvin M, Pender N, Hardiman O

**COG-14 Augmentative and alternative communication in ALS: a systematic review**

Abbas-Kayano R T, Chadi G

**THEME 12****Respiratory and nutritional management****RNM-01 Weight stability is associated with longer survival in ALS**

Wei Q, Ou R, Chen Y, Chen X, Cao B, Hou Y, Zhang L, Shang H

**RNM-02 Temporal variation of anthropometric parameters and functional scores of patients diagnosed with ALS/MND**

dos S Salvioni C C, Ottoboni M M, Campos M C P, Oda A L, Alves P C L, Sierra H N M, Neves J W C, Frabasile L, Oliveira A S B

**RNM-03 Factors associated with oral nutritional behaviours in people with MND: a systematic review**

Williams I, Norman P, Essat M, Archer R, Coates E, Zarotti M, Clowes M, Beever D, Hackney G, White S, Stavroulakis T, McDermott C, on behalf of the HighCALS group

**RNM-04 Serum albumin as an indicator of gastrostomy in patients with MND/ALS: preliminary results from a single tertiary hospital**

Yang J, Lee K-W, Choi S-J

**RNM-05 Long-term outcomes of gastrostomy in patients with ALS with respiratory compromise**

Choi S-J, Min Y G, Kwon Y N, Yang J, Sung J-J, Lee K-W

**RNM-06 Comparative analysis of the types of alternative feeding route recommended for ALS/MND**

Frabasile L M, Alves P C L, Borges R M, Sierra H N M, Salvioni C C S, Oliveira A S B, Oda A L

**RNM-07 The level of functional hydration assessment according to dysphagia in MND/ALS**

Oda A L, Borges R M, Frabasile L M, Alves P C L, Sierra H N M, Neves J W C, dos Santos Salvioni C C, Oliveira A S B

**RNM-08 The safety of PEG in patients with ALS using non-invasive positive pressure ventilation: a retrospective study**

Tominaga N, Shimizu T, Uchino A, Ogino Y, Ogino M

**RNM-09 Comparison of voluntary and reflex cough effectiveness in health and individuals with ALS**

Gray L T, Locatelli E, Plowman E K

**RNM-10 The impact of respiratory muscle training on cough efficacy and physical function in patients with neuromuscular disease: a systematic review and meta-analysis**

Watson K

**RNM-11 Correlation between forced vital capacity (FVC) and collapse of the upper airways (retropalatal and retrolingual spaces)**

Dorca A, Sisteroli D

**RNM-12 Inspiratory pressurization therapy associated with expiratory muscular training in ALS / MND**

Dorca A, Sisteroli D, Maldaner V

**RNM-13 Sleep-disordered breathing can be asymptomatic in patients with ALS**

Aziz N A A, Ng W J, Loh E C, Capelle D, Goh K-J, Latif L A, Shahrizaila N

**RNM-14 Identifying how many SNIPs to get maxscore in patients with MND**

Fortune J, Meldrum D, Murray D, Fenton L, Tattersall R, Hardiman O

**RNM-15 Symptoms of hypoventilation most sensitive to detect reduced respiratory function in patients with ALS/MND**

Helleman J, Kruitwagen-van Reenen E T, Kruithof W J, Bakers J, Visser-Meily J MA, van den Berg L H, Beelen A J

**RNM-16 Respiratory function and infections in people with MND**

Sheers N, Berlowitz D J, Rochford P, Dirago R, Naughton P, Henderson S, Howard M E

**RNM-17 Implementing home care respiratory protocols in ALS**

Brockenbrough P, Rhodes R, Pearson K, Vota S

**RNM-18 A survey exploring the practice of healthcare professionals in delivering non-invasive ventilation to people with MND in the UK**

Musson L S, Stavroulakis T, Baxter S K, Norman P, O'Brien D, Elliott M, Bianchi S, Kaltsakas G, Hobson E V, McDermott C J

**RNM-19 Factors that influence the tolerance of non-invasive ventilation in ALS: results from single centre in Brazil**

Silva V Z M, Moura M, Franco H, Mateus S, Dorça A

**RNM-20 MND respiratory supports: the impact of non-invasive ventilation on MND patients**

Pradeepan S, Yates N, Vogel N, Paech G

**RNM-21 Diaphragm pacing impairs local myofiber reinnervation in ALS**

Guimarães-Costa R, Niérat M-C, Rivals I, Morélot-Panzini C, Romero N B, Menegaux F, Salachas F, Gonzalez-Bermejo J, Similowski T, Bruneteau G

**RNM-22 Proposition for the assessment of peak cough flow with an oronasal mask in ALS/ MND patients**

Borges R M, Alves P C L, Sierra H N M, Frabasile L M, Neves J W C, dos Santos Salvioni C C, Oliveira A S B, Oda A L

**RNM-23 Clinical criteria for the recommendation of invasive ventilation for MND/ALS patients**

Alves P C L, Borges R M, Frabasile L M, Sierra H N M, Neves J W C, Flores S C, Rocha M S G, dos Santos Salvioni C C, Oliveira A S B, Oda A L

**RNM-24 Amount of publicly available survey information on trends in the number of home-care patients with ALS and invasive TPPV with tracheostomy in Japan: a preliminary survey of prefectural coordinators regarding patients with intractable diseases**

Fukuroku K, Narita Y, Ishikawa T, Nakai M, Matsuda N

**RNM-25 Prognosis of TIV therapy for ALS patients in a multicenter prospective cohort**

Hayashi N, Atsuta N, Yokoi D, Nakamura R, Katsumo M, Izumi Y, Kanai K, Hattori N, Akira T, Morita M, Kano O, Shibuya K, Kuwabara S, Suzuki N, Aoki M, Masaya O, Aiba I, Mizoguchi K, Ishihara T, Onodera O, Ohta Y, Abe K, Kaji R, Sobue G

**RNM-26 Registry of endpoints and validated experiences in ALS (REVEALS): a prospective observational study of respiratory function and morbidity in ALS**

Murray D, Meldrum D, McConnell R, Chio A, Al-Chalabi A, van Den Berg L, Van Damme P, McDermott C, Hardiman O

**RNM-27 The cause of death in pathologically confirmed ALS with mechanical ventilation assist, a retrospective institute-based study**

Komai K, Ishida C, Takahashi K, Tagami A, Motozaki Y, Kawashima A

**THEME 13****Clinical management and support****CMS-01 Genetic testing for familial ALS: insights and challenges**

Crook A, Hogden A, Mumford V, Blair I P, Williams K L, Rowe D B

**CMS-02 Preventing ALS through reproductive genetic testing: costs and complexities**

Crook A, Mumford V, Hogden A, Fell R, Blair I P, Williams K L, Rowe D B

**CMS-03 The importance of the reception performed by ABreLA's social service to ELA patients and their families**

Campos C H M, Cruz F T, Oda A L

**CMS-04 The diagnostic experience in MND: a UK survey of the perspectives of people living with MND**

O'Brien M R, Oliver D, Aoun S, McDermott C J, Kirton J, Pearson E

**CMS-05 Changes in diurnal and nocturnal activity occurs in ALS/MND patients**

Lucia D, McCombe P A, Henderson R D, Steyn F J, Ngo S T

**CMS-06 A survey of healthcare professionals on the measurement of physical functioning in ALS/MND and attitudes to development of technology based measurement and monitoring solutions**

Murray D, Meldrum D, Hayden C, Hardiman O

**CMS-07 Motor symptoms and physical activity assessment in ALS: a systematic review**

Chadi G, de Aquino L M, Silva R, Alves A, Gomide-Çakmak V C

**CMS-08 Are we under dosing our patients with mid-stage disease ALS? When “usual” activities becomes rowing across the Atlantic**

Hayes H A, Alderman A, Gibson S, Bromberg M

**CMS-09 Robot-assisted training using Hybrid Assistive Limb for ALS patients**

Kano O, Murata K, Sugisawa T, Ebina J, Morioka H, Kyuzen M, Sawada M, Hanashiro S, Nagasawa J, Yanagihashi M, Fukuda H, Uchi M, Kawabe K, Ikeda K, Washizawa N, Ebihara S

**CMS-10 The ALS Steering Wheel: A multidisciplinary approach to evaluating driving in ALS**

Berry K, Salmon K, Vitale T, Saunders N, Genge A

**CMS-11 Cessation of driving in individuals with ALS**

Hayes H A, Hu N, Wang X, Leatham J L, Gibson S, Bromberg M

**CMS-12 An investigation into whether board certified neurologists are conscious of supporting continuing employment in their patients**

Ogino M, Eguchi H, Babayev T, Ogino Y, Tutsumi A

**CMS-13 An example of possibility for family members continue working while providing care for ALS/MND patients**

Adachi K, Ishijima K, Kawaguchi Y, Nakajima T

**CMS-14 Information needs and resource preferences in Korean Family caregivers of Patients with ALS**

Chu H S, Son B, Kim S H, Oh J

**CMS-15 Aspiring to minimise aspiration: prevalence and predictors of aspiration in progressive neurological disease**

Klein P, Ferencz N, Jackson N, Keage M

**CMS-16 Providing collars for people with MND: trialling not prescribing**

Gibb R M

**CMS-17 Clinical implications of upper esophageal sphincter restriction in MND**

Cock C, Francis R, Doeltgen S, Omari T

**CMS-18 Interaction between decline of swallowing and cognitive function in MND**

Francis R, Attrill S, Cock C, Doeltgen S

**CMS-19 A clinical bulbar assessment scale for ALS (C-BAS)**

Ball L J, Pattee G L

**CMS-20 Developing a message banking pathway: the Irish experience through multi-agency collaboration**

Doyle L, Fitzsimons C, Jagoe C

**CMS-21 A single adjustment of the scanning-speed of the device affected each participant's feeling and number of letters as an effect of short-term training programs on augmentative and alternative communication to support ALS/MND patients**

Tanaka Y, Narita Y, Ishikawa T, Nakai M, Imura T, Takahashi E, Mizumoto C

**CMS-22 Gross numbers of letters as the effect of a short-term training program for augmentative and alternative communication on health-care students after 6 months**

Ishikawa T, Narita Y, Nakai M, Imura T, Tanaka Y, Takahashi E, Mizumoto C

**CMS-23 Verification of the effect of medical coordinator of intractable disease on the regional medical care network for intractable disease in Japan**

Hotta M, Tanaka Y

**CMS-24 Virtual reality in ALS**

Vota S A, Pearson K, Rhodes R, Brockenbrough P

**CMS-25 Improved survival in ALS patients following intrathecal administration of autologous Bone Marrow Mononuclear Cells (BMMNCs)**

Sane H, Paranjape A, Varghese R, Pradhan R, Nair V, Badhe P, Gokulchandran N, Sharma A

**CMS-26 The effect of riluzole oral film on swallowing safety in individuals with ALS**

Anderson A, DiBiase L, Plowman E, Wymer J, Chapin J, Heller A, Buan C, Jung C, Slatko G

**CMS-27 Nursing driven initiative to increase tolerability and compliance of BHV-0223 novel therapy in ALS patients**

Ranzinger L, Newell-Sturdivant A, Jones J, Brooks B

**CMS-28 Zydis riluzole 40mg oral disintegrating tablet biohaven expanded access program – single center experience**

Jones J, Ranzinger L H, Brooks B R

**CMS-29 Real-world evidence of Radicava® (edaravone) for ALS from a national infusion center database in the United States**

Heiman-Patterson T, Perdrizet J, Apple S, Prosser B, Agnese W

**CMS-30 A preliminary analysis of the feasibility and efficacy of edavarone at a multidisciplinary ALS clinic**

Gray L T, Scarlett F, Pereda M, Locatelli E

**CMS-31 Discussing personalised prognosis in ALS: development of a communication guide**

van Eenennaam R M, Kruihof W, van Es M A, Reenen E K-V, Westeneng H-J, Visser-Meily A, van den Berg L H, Beelen A

**CMS-32 A comprehensive approach to end of life discussions in ALS**

Brockenbrough P, Maldonado J, Gebhardt M, Vota S

## THEME WP

### Biomedical and clinical work in progress

**WP-01 Prevalence of ALS in the United States, 2016**

Mehta P, Larson T, Horton K

**WP-02 Utilizing capture-recapture methodology to estimate the missing ALS prevalent cases in the United States, 2016**

Horton K, Larson T, Mehta P

**WP-03 Critical epochs of environmental exposures and gene-environment-time interactions in ALS**

Bradley W G, Andrew A S, Chio A, D’Ovidio F, Shi X, Pioro E P, Guetti B, Torbick N, Butt T, Traynor B, Gui J, Stommel E W

**WP-04 MND phenotypes and premorbid status in the Trøndelag region, Norway**

Taraldsen M D, Bjørnstadjordet M, Meisingset T W, Geir Bråthen G

**WP-05 Assessing structural- and copy number-variation in MND**

Fifita J A, McCann E P, Williams K L, Chan Moi Fat S, Henden L, Grima N, A Twine N A, Bauer D C, Kiernan M, Nicholson G A, Rowe D B, Blair I P

**WP-06 Long-read sequencing approaches to investigate the contribution of human-specific variable number tandem repeats to ALS susceptibility**

Course M M, Gudsnuuk K, Valdmanis P N

**WP-07 Novel patient-derived 3D in vitro models of microglia to study neuroinflammation in ALS**

Cuni-Lopez C, Quek H, Oikari L E, Stewart R, White A R

**WP-08 NF-κB activation in astroglia in mouse models of ALS**

Kroeger C, Baumann B, Ouali N, Schurr C, Wirth T

**WP-09 Good riddance to bad rubbish: waste disposal in human ALS post-mortem pericytes**

Dunne C M, Mouravlev A, Scotter E L

**WP-10 Investigating TDP-43 mislocalisation using novel human induced pluripotent stem cell models**

Talbot J, Stellon D, Clear S, Atkinson R, Perry S, Hewitt A, King A E, Cook A L

**WP-11 Characterising CYTSB as a potential therapeutic target for organ pathology in SMA**

Munir R, Hunter G

**WP-12 A combined structural, functional and neurochemical MRI signature of motor system excitability in ALS**

Edmond E C, Hinson E L, Clarke W, Talbot K, Stagg C J, Turner M R

**WP-13 Diagnostic utility of the split-hand index in ALS phenotypes**

Hannaford A, Higashihara M, Van den Bos M, Geevasinga N, Vucic S, Menon P

**WP-14 People living with ALS and their caregivers' input into drug development in Europe**

Galvin M, Hardiman O, Heverin M, McDermott C, Laverdiere A, Charpentier B, O’Callaghan L, Bowyer K

**WP-15 WITHDRAWN**

**WP-16 Phase 1/2a, double-blind, placebo-controlled study with an open-label extension of ropinirole hydrochloride extended-release tablets (ROPALS trial based on the iPSC drug repositioning)**

Takahashi S, Morimoto S, Okada K, Daté Y, Ito D, Nakahara J, Okano H

**WP-17 The Japanese early-stage trial of high dose methylcobalamin for ALS (JETALS): Protocol of the phase III trial and validation of the updated Awaji criteria for the diagnosis of early stage ALS**

Oki R, Izumi Y, Fujita K, Nodera H, Yamazaki H, Haji S, Osaki Y, Miyamoto R, Sato Y, Futami A, Maeda K, Takechi K, Sakaguchi S, Nohihara H, Yanagawa H, Kuwabara S, Kaji R, JETALS Collaborators

**WP-18 Clinical characteristics of young-onset ALS in Korean cohort**

Lee S, Park J, Oh K-W, Il Jang D, Hyun Kim S

**WP-19 Machine learning for novel prognosis prediction and ALS patient stratification**

Grollemund V L, Pradat-Peyre J-P, Delbot F, Bede P, Corcia P, Couratier P, Meininger V, Pradat P-F

**WP-20 Intranasal oxytocin for terminal ALS with social interaction deficits**

Fujita K, Shimazu T, Maruki Y

**WP-21 Assessing assistive technology use and needs by individuals with ALS/MND**

Feldman S, Dryden E

**WP-22 The addition of rotational and adjustable flexion components to cervical support**

Feldman S, Goren M, Harris D, Dryden E, Nevasekar N, Ayaz I

**WP-23 The MotOrtose project - development of a motorized upper extremity orthosis for ALS**

Meisingset T, Bråthen G, Lien T

**WP-24 High-throughput screening for the development of novel ALS treatments**

Colicchia V, Hansel C, Porębski B, Häggblad M, Cormann A, Li X, Lidemalm L, Hühn D, Carreras-Puigvert J, Fernández-Capetillo O

## THEME CP

### Care Practice

**CP-01 Challenges and success of rowing across the Atlantic by an individual with ALS**

Hayes H A, Alderman A, Gibson S, Bromberg M

**CP-02 ALS and full-marathon: Response to edaravone treatment**

Yoshino H

**CP-03 A pilot project to determine if standard ayurvedic treatment protocol alters the progression of ALS**

Paul A, James F A, VP J K, Karalam S B

**CP-04 A systematic review of diet and exercise clinical trials among people with ALS**

Zheng E R Y, Lau T, Vucic S, Flood V M

**CP-05 Supporting choice in dysphagia management through naturally thick drinks**

Doyle L, Heraughty N

**CP-06 The impact of gastrointestinal related symptoms on nutrition intake - the role of the dietitian in managing gastrointestinal complications in persons living with MND**

Zandi E, Savvaidis A

**CP-07 Developing a web-based patient decision aid for gastrostomy in MND: the DiAMoND study**

Evill R, Bloomfield S, Erridge C, Foster C, Hardcastle M, Hogden A, Kidd A, Lisiecka D, McDermott C, Morrison K, Recio-Saucedo A, Rickenbach L, White S, Williams P, Wheelwright S

**CP-08 The effect of feeding tube placement on body mass index and ALSFRS-r**

Rhodes R, Brockenbrough P, Pearson K, Vota S, Gwathmey K

**CP-09 Oscillating PeP (O-PeP) devices versus Expiratory Muscle Trainers (EMTs) for lung recruitment in ALS patients prior to NIV**

Heimann-Patterson T, Harris D, Mergner D, Pepper H

**CP-10 Pneumothorax in neuromuscular disease associated with lung volume recruitment and mechanical insufflation-exsufflation**

McDonald L, Berlowitz D J, Howard M E, Rautela L, Chao C, Sheers N

**CP-11 Long-term follow-up for patients undergoing pre-symptomatic genetic testing for C9orf72 and SOD1**

Kinsley L M

**CP-12 Familial ALS and FTD: identifying the need for a new genetic counselling model of care**

Crook A, Rowe D B, Fell R, Jacobs C, Newton-John T, McEwen A

**CP-13 Differentiating needs of informal caregivers of ALS patients across the caregiving course: a systematic review**

Poppe C, Iseli L, Koné I, Elger B S, Wangmo T

**CP-14 Family caregivers in ALS and their vital need for self-care**

Verwey M A

**CP-15 Group interventions for ALS caregivers: a randomised controlled trial protocol**

Burke T, O'Raghallaigh J W, Maguire S, Galvin M, Heverin M, Hardiman O, Pender N

**CP-16 Interventions targeting the psychological well-being of carers of people with MND: a systematic review**

Cafarella P, Effing T, Chur-Hansen A

**CP-17 Efficacy of a group-based mindfulness program for people with MND and their family caregivers**

Gluyas C, Haylock P, Atkins K, Davis M-C, Conroy H, Fisher F, Velissaris S

**CP-18 Animal assisted activity with service dogs "One project" to heal the patients with ALS who have been hospitalized long term**

Ogino Y, Ogino M, Komori T

**CP-19 Mental health support plans for people affected by MND**

Bethell A

**CP-20 The needs and psychological distress of family caregivers after the death of the ALS patient**

Knudsen L F

**CP-21 Support for patients with intractable neurological diseases to select treatment options**

Nagase M

**CP-22 Assessing preference heterogeneity with respect to MND treatment: a discrete choice experiment**

Farrar M A, Street D, Carey K, Kasparian N, De Abreu Lourenco R

**CP-23 The development of a multidisciplinary specialist communication and assistive technology (CAT) clinic within the New South Wales (NSW) public health sector**

Gibb A, Signorelli M, Thornley M

**CP-24 Occupational therapist and the benefits of the online learning environment for clinical intervention for people living with MND**

Brown R, Solomon S

**CP-25 How open-ended questions posed by clients have informed, impacted and evolved an occupational therapy service from inception to now - 5 years on**

Knight R E

**CP-26 How an occupational therapy service has changed and evolved from direct client care provision to consultative service methods, to best meet the needs of people with MND post National Disability Insurance Scheme (NDIS)**

Knight R E

**CP-27 OT for people with MND: adjusting and adapting to rapidly changing function**

Solomon S J

**CP-28 Reflections on the care of people with MND/ALS care over 35 years**

Oliver D J

**CP-29 Four quadrants of care**

Quick L K, Baigent S

**CP-30 Shhh...we don't talk about that!**

Quick L K

**CP-31 Metro South chronic disease MND gastrostomy change service**

Quick L K, Ebzery M

**CP-32 A dying wish: organ donation in MND – ethics law and practice in the Australian context**

Sheahan L, Herz H, Flynn G

## Exhibitors



# Summary of events/locations

## Wednesday 4 December

07.00 – 18.00	Registration International Symposium	Reception Desk	Level 2
07.00 – 19.00	Speaker Room	Meeting Room 12	Level 2
08.30 – 10.30	Symposium Joint Opening Session	Riverside Theatre	Level 2
10.30 – 11.00	Refreshments, Networking and Exhibitors	Riverside Theatre Foyer	Level 2
11.00 – 12.45	Symposium Clinical Session 2A	Riverside Theatre	Level 2
11.00 – 12.30	Symposium Biomedical Session 2B	Bellevue Ballroom 2	Level 3
11.00 – 12.45	Symposium Alternative Session 2C	Meeting Rooms 1-3	Level 2
12.30 – 14.00	Lunch and Networking Exhibitors	Bellevue Ballroom 1/Bellevue Foyer Riverside Theatre Foyer	Level 3 Level 2
12.30 - 14.00	REFALS (closed meeting)	Meeting Room 8	Level 2
14.00 – 15.30	Symposium Clinical Session 3A	Riverside Theatre	Level 2
14.00 – 15.30	Symposium Biomedical Session 3B	Bellevue Ballroom 2	Level 3
14.00 – 15.30	Symposium Alternative Session 3C	Meeting Rooms 1-3	Level 2
15.30 – 16.00	Refreshments, Networking and Exhibitors	Riverside Theatre Foyer	Level 2
16.00 – 17.45	Symposium Clinical Session 4A	Riverside Theatre	Level 2
16.00 – 17.40	Symposium Biomedical Session 4B	Bellevue Ballroom 2	Level 3
16.00 – 17.40	Symposium Alternative Session 4C	Meeting Rooms 1-3	Level 2
18.00 – 20.00	Global Walk to D'Feet and Barbecue Reception	External and Summer Garden	Level 2

## Thursday 5 December

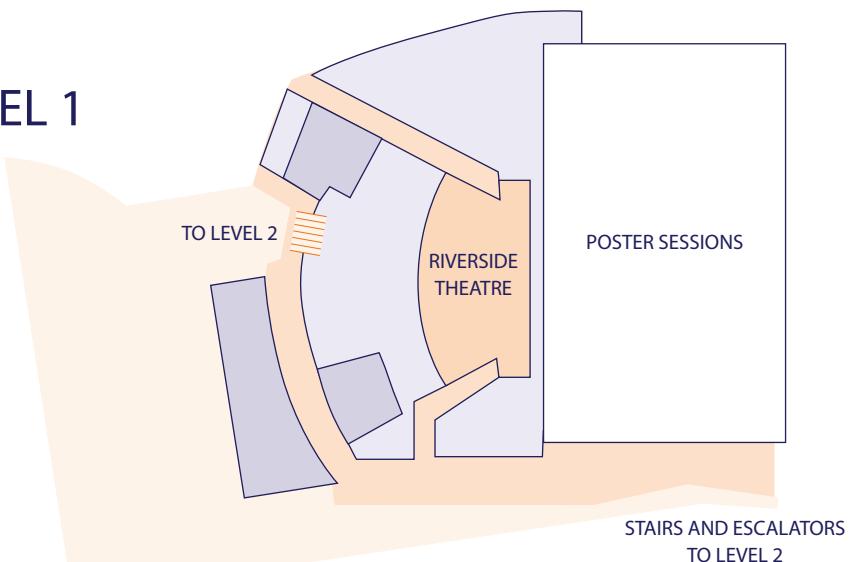
07.00 – 18.00	Registration International Symposium	Reception Desk	Level 2
07.00 – 19.00	Speaker Room	Meeting Room 12	Level 2
07.15 – 08.15	Advancing Innovation in ALS: The Importance of Environment vs Genes in the Development of ALS (Sponsored by Cytokinetics)	River View Room 5	Level 2
08.30 – 10.00	Symposium Clinical Sessions 5A	Riverside Theatre	Level 2
08.30 – 10.00	Symposium Biomedical Session 5B	Bellevue Ballroom 2	Level 3
08.30 – 10.00	Symposium Alternative Session 5C	Meeting Rooms 1-3	Level 2
10.00 – 10.30	Refreshments, Networking and Exhibitors	Riverside Theatre Foyer/Pavilion 1	Level 2/Level 1
10.30 – 12.30	Poster Session A	Pavilion 1	Level 1
12.30 – 14.00	Lunch and Networking Exhibitors	Bellevue Ballroom 1/Bellevue Foyer Riverside Theatre Foyer	Level 3 Level 2
14.00 – 15.30	Symposium Clinical Sessions 6A	Riverside Theatre	Level 2
14.00 – 15.30	Symposium Biomedical Session 6B	Bellevue Ballroom 2	Level 3
14.00 – 15.30	Symposium Alternative Session 6C	Meeting Rooms 1-3	Level 2
15.30 – 16.00	Refreshments, Networking and Exhibitors	Riverside Theatre Foyer	Level 2
16.00 – 17.50	Symposium Clinical Sessions 7A	Riverside Theatre	Level 2
16.00 – 17.45	Symposium Biomedical Session 7B	Bellevue Ballroom 2	Level 3
16.00 – 17.45	Symposium Alternative Session 7C	Meeting Rooms 1-3	Level 2
18.00 – 20.00	Poster Session B	Pavilion 1	Level 1
19.15 – 20.45	BRAIN-MEND consortium meeting (closed meeting)	Meeting Room 10	Level 2

## Friday 6 December

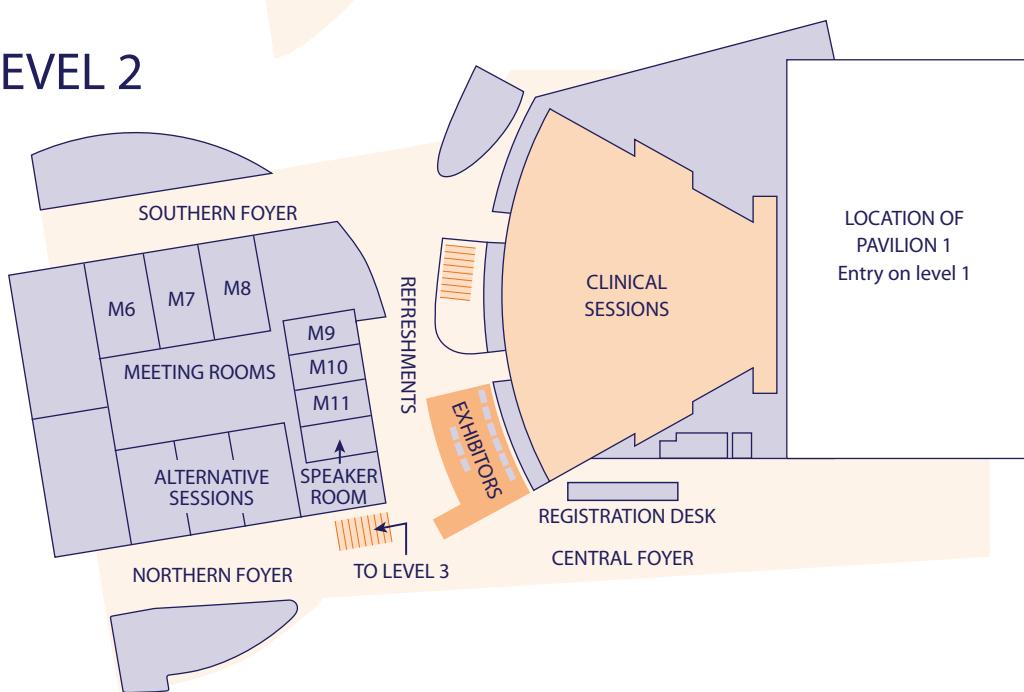
07.00 – 13.30	Speaker Room	Meeting Room 12	Level 2
08.00 – 12.00	Registration International Symposium	Reception Desk	Level 2
08.30 – 10.00	Symposium Clinical Sessions 8A	Riverside Theatre	Level 2
08.30 – 09.50	Symposium Biomedical Session 8B	Bellevue Ballroom 2	Level 3
10.00 – 10.30	Refreshments, Networking and Exhibitors	Riverside Theatre Foyer	Level 3
10.30 – 12.30	Symposium Clinical Sessions 9A	Riverside Theatre	Level 3
10.30 – 12.30	Symposium Biomedical Session 9B	Bellevue Ballroom 2	Level 3
12.30 – 14.00	Lunch and Networking Exhibitors	Bellevue Ballroom 1/Bellevue Foyer Riverside Theatre Foyer	Level 3 Level 2
14.00 – 15.30	Symposium Joint Closing Session	Riverside Theatre	Level 2

## Locations

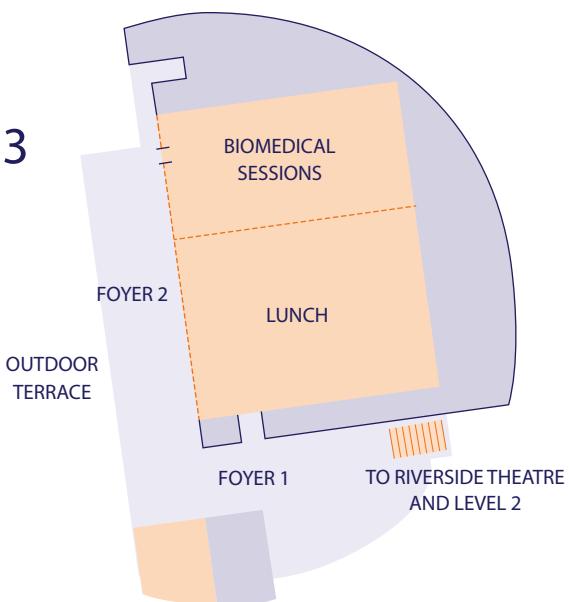
### LEVEL 1



### LEVEL 2



### LEVEL 3



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