Full programme (not including satellites)

Wednesday 1st June

08.30 to 10.30	Registration
10.30 to 12.30	**Session 1: Presymptomatic Disease** Chairs: **Sharon Abrahams, Orla Hardiman** Speakers: **Leonard van den Berg**, University Medical Center Utrecht – The Netherlands, and Chair of ENCALS; Introduction **Siddharthan Chandran**, University of Edinburgh – Scotland; "From Bench to Bedside" Invited Speaker: **Jonathan Rohrer**, University College London – UK; "GENFI and the pathway to clinical trials in genetic frontotemporal dementia" Platforms: 1. Pre-symptomatic mild cognitive and behavioural impairment in ALS-frontotemporal spectrum disorder (ALS-FTSD): A conceptual framework. **Caroline McHutchison**, University of Edinburgh – Scotland 2. EEG changes in cognitive networks in asymptomatic C9orf72 repeat expansion carriers. **Stefan Dukic**, University Medical Center Utrecht – The Netherlands 3. Distinct neural signatures of pulvinar in C9orf72 ALS mutation carriers. **Anna Nigri**, Foundation IRCCS Neurological Institute Carlo Besta – Italy 4. Neuropsychological endophenotypes in first- and second-degree relatives of people with ALS. **Emmet Costello**, Trinity Biomedical Sciences Institute – Ireland

13.30 to 15.00	**Session 2: Imaging and Biomarkers** Chairs: **Philip van Damme, Adriano Chio** Invited Speaker: **Frederica Agosta**, San Raffaele Scientific Institute – Italy; "Neuroimaging in ALS: facing the challenges of an elusive disease" Platforms: 5. Neuroimaging correlates of domain-specific cognitive deficits in amyotrophic lateral sclerosis. **Abram Nitert**, University Medical Center Utrecht – The Netherlands 6. ALS subgroups based on EEG measures recorded during sustained attention to response task performance. **Vlad Sirenko**, Trinity College Dublin – Ireland 7. Brain sensorimotor integration after focal muscle-tendon vibration in amyotrophic lateral sclerosis. **Arnaud s, Sorbonne** Université – France 8. Diagnostic utility of nerve excitability tests in ALS. **Diederik Stikvoort Garcia**, University Medical Center Utrecht – The Netherlands
15.30 to 17.15	**Session 3: Neuropsychology/ Neuropathology** Chairs: **Nigel Leigh, Sharon Abrahams** Invited Speaker: **Niall Pender**, Trinity College Dublin / Beaumont Hospital Dublin – Ireland; "The Clinical Neuropsychology of ALS" Platforms: 9. Examining the nature of phonemic verbal fluency in the Familial ALS cognitive endophenotype. **Colm Peelo**, Trinity College Dublin – Ireland 10. Self-perceived quality of life and cognitive and behavioural impairment in ALS, UEA/EMC. **Ratko Radakovic**, University of East Anglia – UK 11. Patients with amyotrophic lateral sclerosis and cognitive deficits are impaired in recognizing negative facial emotions. **Nathalie Braun**, Kantonsspital St. Gallen Muskelzentrum - Switzerland 12. Motor, cognitive and behavioral profiles of C9orf72-related amyotrophic lateral sclerosis. **Nicola Ticozzi**, Istituto Auxologico Italiano IRCCS – Italy 13. Synaptic proteomics reveal distinct molecular signatures of cognitive change and C9orf72 repeat expansion in the human ALS cortex. **Chris Henstridge**, University of Dundee – UK 14. NanoString molecular barcoding of patient tissue identifies molecular signatures of clinical heterogeneity in C9orf72-ALS. **Olivia Rifai**, University of Edinburgh – Scotland

Thursday 2nd June

08.30 to 10.30

Session 4: Genomics

Chairs: **Chris McDermott**, Monica Povedano

Invited Speaker: **Jan Veldink**, University Medical Center Utrecht – The Netherlands; "Genetics in ALS: is bigger always better?"

Platforms:

15. Whole-genome sequencing reveals that variants in the Interleukin 18 Receptor Accessory Protein 3'UTR protect against ALS. **Chen Eitan**, Weizmann Institute of Science – Israel
16. Identifying genetic subtypes of amyotrophic lateral sclerosis using latent class analysis. **Thomas Spargo**, King's College London – UK
17. Phenotype analysis of FUS mutations in ALS. **Andrea Calvo**, University of Turin – Italy
18. Genetic architecture of primary lateral sclerosis. **Munishikha Kalia**, Kings College London – UK
19. Genetic data of 10,996 ALS patients and 7,403 controls shows that missense variants in the tail domain of NEFH increase the risk of ALS. **Heather Marriott**, King's College London – UK
20. Dissecting the pathogenic role of Ataxin-2 repeat expansions in ALS. **Marta Cañizares Luna**, University Medical Center Utrecht – The Netherlands

10.10- 10.30

Rapid Fire Posters (1/2)

Chair: **Philippe Corcia**

Presentations:

21. Understanding Disease Trajectory in Amyotrophic Lateral Sclerosis. **Ahmad Al Khleifat**, King's College London – UK
22. Developing a systematic framework to identify, evaluate and report evidence for drug selection in motor neuron disease clinical trials. **Charis Wong**, University of Edinburgh – Scotland
23. Genome-wide assessment of genetic modifiers in ALS progression. **Ramona Zwamborn**, Utrecht University Medical Center – The Netherlands
24. The Sustained Attention to Response Task evokes sensorimotor beta ERD/ERS and enables quantification of motor and cognitive pathophysiology. **Roisin McMackin**, Trinity College Dublin - Ireland
25. Profiling brain morphologic features of motor neuron disease caused by TARDBP mutations: an MRI-based study. **Alma Ghirelli**, IRCCS San Raffaele Scientific Institute – Italy
26. Dysregulation of extracellular vesicle formation and release in astrocytes from ALS patients. **Andre Varcianna**, University of Sheffield – UK

Thursday 2nd June

11.00 to 12.30

Session 5: Cell & Molecular Biology (1/2)

Chairs: **Janine Kirby**, **Ludo van den Bosch**

Invited Speaker: **Anne Bertolotti**, Cambridge University – United Kingdom; Boosting protein quality control: "A possible therapeutic strategy for neurodegenerative diseases"

Platforms:

27. ALS-causing KIF5A mutant proteins form aggregates. **Rüstem Yilmaz**, Heidelberg University – Germany
28. HNRNPK counteracts DNA damage as part of RNA toxicity in C9orf72 ALS/FTD. **Elke Braems**, KU Leuven – Belgium
29. Updates on seeding studies: SOD1 prions transmit aggregation and fatal ALS-like disease – Introducing Strain C. **Elaheh Ekhtiari Bidhendi**, Umeå University - Sweden
30. Defective cyclophilin A induces TDP-43 proteinopathy: implications for amyotrophic lateral sclerosis and frontotemporal dementia. **Laura Pasetto**, Istituto di Ricerche Farmacologiche Mario Negri IRCCS – Italy
31. iPSC-derived motor neurons from C9orf72 ALS/FTD-patients display defects in lysosomal function and homeostasis. **Jimmy Beckers**, KU Leuven – Belgium

13.30 to 14.55

Session 6: Cell and Molecular Biology (2/2)

Chairs: **Siddharthan Chandran**, **Helene de Blasco**

Invited Speaker: **Hemali Phatnani**, Columbia University – USA; "Using Spatial Genomics to study the Central Nervous System in Health and Disease"

Platforms:

32. Different cellular environments shape TDP-43 function with implications in neuronal and muscle diseases. **Emanuele Buratti**, International Centre for Genetic Engineering and Biotechnology – Italy
33. Why should we care about astrocytes in a motor neuron disease? **Katarina Stoklund Dittlau**, KU Leuven – Belgium
34. Cell-autonomous immune dysfunction driven by disrupted autophagy in C9orf72-ALS microglia contributes to neurodegeneration. **Poulomi Banerjee**, University of Edinburgh – Scotland
35. Astrocyte-induced DNA damage as a mechanism of motor neuron death in ALS. **Jannigje Kok**, University of Sheffield – United Kingdom
36. Involvement of inhibitory neurons in amyotrophic lateral sclerosis and frontotemporal dementia linked to Fused in Sarcoma protein. **Félicie Lorenc**, University of Strasbourg - France

15.20 to 17.00

Session 7: Drug Discovery

Chairs: **Kevin Talbot**, **Susanna Petri**

Invited Speaker: **Neil Carragher**, University of Edinburgh – Scotland; "Advancing drug discovery, in challenging areas of unmet medical need, through high content phenotypic and pathway profiling"

Platforms:

37. Modulation of TDP-43 by TTBK1 inhibitors: A new therapeutic approach for Amyotrophic Lateral Sclerosis and others TDP-43-pathies. **Ana Martinez**, Centro de Investigaciones Biológicas-CSIC - Spain

16.00- 16.20

Rapid Fire Posters (2/2)

Chair: **Kevin Talbot, Suzanna Petri**

Presentations:

38. In ALS dysfunction of nucleoporin 107 impairs autophagy contributing to TDP-43 aggregation. **Manuel Portero-Otin**, IRBLleida-Universitat de Lleida – Spain
39. Senescent astrocytes drive neurodegeneration via extracellular vesicles in ALS-FTD. **Manuela Basso**, University of Trento - Italy
40. Dynamic Expression Profiles of Stressed iPSC-MNs by Translating Ribosome Affinity Purification (TRAP) from C9orf72-ALS Patients. **Yinyan Xu**, University of Oxford – UK
41. Aging-dependent activity impairments of human C9orf72-mutant motor neurons are accompanied by aberrant transcriptional programs. **Alberto Catanese**, Ulm University School of Medicine - Germany
42. Using optogenetics to model activity-dependent neurodegeneration in amyotrophic lateral sclerosis. **Lucy Farrimond**, University of Oxford – UK
43. ALS/FTD-associated C9orf72 C4G2 repeat RNA disrupts phenylalanine tRNA aminoacylation. **Boris Rogelj**, Jožef Stefan Institute – Slovenia

16:20 to 17:00

Session 8: Debate

Chair: **Orla Hardiman**

Invited Speakers: **Mary Porteous**, University of Edinburgh – Scotland
Michael van Es, University Medical Center Utrecht – The Netherlands

Theme: Should we be doing whole genome sequencing routinely in ALS?

Friday 3rd June

9.00 to 10.30

Session 9: Clinical Trials

Chairs: **Leonard van den Berg, Angela Genge**

Invited Speaker: **Ruben van Eijk**, University Medical Center Utrecht – The Netherlands; "Current trends and considerations for ALS Clinical Trials"

Platforms:

44. Futility monitoring in clinical trials for amyotrophic lateral sclerosis: saving time, resources and accelerating clinical development. **Jordi van Unnik**, University Medical Center Utrecht – The Netherlands
45. Phase 2 clinical trial of Rapamycin for Amyotrophic Lateral Sclerosis. **Jessica Mandrioli**, University of Modena and Reggio Emilia - Italy
46. Results from the Phase 1 Trial and Open Label Extension Evaluating BIIB078 in Adults with C9orf72-ALS. **Leonard van den Berg**, University Medical Center Utrecht – The Netherlands
47. Targeting pathological transcriptional variants in C9orf72-associated amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD): Initial results from the ongoing FOCUS-C9 clinical trial. **Michael A. Panzara**, Wave Life Sciences – USA
48. Evaluating the Efficacy and Safety of Tofersen in Adults with ALS and a SOD1 Mutation: Results from the Phase 3 VALOR Trial and Open-Label Extension. **Tim Miller**, Washington University School of Medicine – USA

11:00 to 11.45

Session 10: Young Investigator Award Session

Chairs: **Ammar Al Chalabi, Caroline Ingre**
Speakers: Awardee & Two Runners Up

11.45 to 12.45

Session 11: Disease Models

Chairs: **Jochen Weishaupt, Eva Hedlund**

Platforms:

49. Early reversible structural and functional impairments of excitatory synapses on ALS motoneurons. **Daniel Zytnicki**, Université Paris Cité - France
50. Meta-analysis of ALS astrocytes reveals multi-omic signatures of inflammatory reactive states. **Oliver J. Ziff**, University College London – UK
51. Human iPSC derived neuromuscular assembloid model to study neuromuscular junction degeneration in Amyotrophic lateral sclerosis. **Andrea Salzinger**, University of Edinburgh - Scotland
52. Alterations in the expression pattern of specific HERV-K copies is associated with Amyotrophic Lateral Sclerosis. **Laura Moreno-Martinez**, University of Zaragoza – Spain