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**Mechanistic insights into the pathophysiology of ALS**

**26-28 May 2021**

*****Hosted by the UK Dementia Research Institute on Zoom,
All times in British Summer Time (BST)*

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| **Wednesday 26 May** |
| **11:00 – 11.05** | Welcome address |
| **11:05 – 11:45** | **Ludo Van Den Bosch (VIB-KU Leuven, Belgium)***The role of HDAC6 in axonal degeneration and regeneration* |
| **11:45 – 12:15** | **Emanuele Buratti (International Centre for Genetic Engineering and Biotechnology, Italy)***Cross-comparison of hnRNP controlled transcripts identifies NOS1AP as a new target in ALS pathology* |
| **12:15 – 12:45** | **Eva Hedlund (Stockholm University, Sweden)***Decoding motor neuron vulnerability and resilience in ALS* |
| **12:45 – 13:00** | Break (15 minutes) |
| **13:00 – 13:15** | Rapid fire talks (to be selected from submitted abstracts) |
| **13:15 – 13:30** | **Sebastian Lewandowski (Karolinska Institute, Sweden)** *Early perivascular fibroblast activity precedes the onset of ALS neurodegeneration* |
| **13:30 – 14:00** | **Laura Ranum (University of Florida, USA)***Targeting RAN proteins rescues ALS/FTD in C9orf72 BAC mice* |
| **14:00 – 14:15** | **Alyssa Coyne (John Hopkins University, School of Medicine, USA)***Nuclear accumulation of CHMP7 initiates NPC injury and subsequent TDP-43 dysfunction in sporadic and familial ALS* |
| **14:15 – 14:30** | **Kurt De Vos (University of Sheffield, UK)***Loss of TMEM106B increases C9ALS/FTD DPR proteins by disrupting lysosome positioning and autophagy* |
| **14:30 – 14:45** | Break (15 minutes) |
| **14:45 – 15:15** | **Adrian Isaacs (UK Dementia Research Institute at UCL, UK)***C9orf72 FTD/ALS disease mechanisms* |
| **15:15 – 15:20** | **Conclusion of the day** |

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| **Thursday 27 May** |
| **11:00 – 11:05** | Welcome address |
| **11:05 – 11:45** | **Dorothee Dormann (Johannes Gutenberg University and Institute of Molecular Biology Mainz, Germany)***Regulation of ALS/FTD-linked proteins by nuclear import receptors and post-translational modifications* |
| **11:45 – 12:15** | **Hideyuki Okano (Keio University, Japan)***iPSCs-based drug development for ALS and clinical trial* |
| **12:15 – 12:45** | **Laura Ferraiuolo (University of Sheffield, UK)***Patient stratification approaches based on drug response using patient-derived cells* |
| **12:45 – 13:00** | Break (15 minutes) |
| **13:00 – 13:15** | Rapid fire talks *(to be selected from submitted abstracts)* |
| **13:15 – 13:30** | ***Alessandro Rosa* (Sapienza University of Rome, Italy)***RNA-binding protein network alteration causes axonal phenotypes in FUS ALS mutant motoneurons* |
| **13:30 – 14:00** | **Aaron Gitler (Stanford University, USA)***New TDP-43 targets* |
| **14:00 – 14:30** | **Clotilde Lagier-Tourenne (Massachusetts General Hospital & Harvard Medical School, USA)***Disruption of RNA metabolism in ALS/FTD and emerging therapeutic strategies* |
| **14:30 – 14:45** | Break (15 minutes) |
| **14:45 – 15:00** | Rapid fire talks *(to be selected from submitted abstracts)* |
| **15:00 – 15:15** | **Daniel Jutzi (UK Dementia Research Institute at King’s College London, UK)***Aberrant FUS-U1 snRNA interactions in FUS-linked ALS* |
| **15:15 – 15:30** | **Nicol Birsa (UCL Queen Square Institute of Neurology, UK)***Protein translation and LLPS impairments in ALS-FUS motor neurons* |
| **15:30 – 15:35** | Conclusion of the day |

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| **Friday 28 May** |
| **11:00 – 11:05** | Welcome address |
| **11:05 – 11:45** | **Séverine Boillée (Paris Brain Institute, France)***Microglia/macrophage contribution to ALS* |
| **11:45 – 12:15** | **Luc Dupuis (University of Strasbourg, France)***Role of FUS in synaptic gene expression: from the neuromuscular junction to the central nervous system* |
| **12:15 – 12:45** | **Rickie Patani (UCL & Francis Crick Institute, UK)***Decoding cell type-specific molecular aberrations in ALS using human stem cell models* |
| **12:45 – 13:00** | Break (15 minutes) |
| **13:00 – 13:15** | Rapid fire talks *(to be selected from submitted abstracts)* |
| **13:15 – 13:30** | **Karthik Krishnamurthy (Thomas Jefferson University, USA)***Aberrant expression of the axon guidance cue netrin-1 by SOD1-ALS astrocytes contributes to motor neuron dysfunction in models of SOD1-ALS* |
| **13:30 – 14:00** | **Chris Shaw (UK Dementia Research Institute at King’s College London, UK)***Genetic therapies for ALS: Are we there yet?* |
| **14:00 – 14:30** | **Magdalini Polymenidou (University of Zürich, Switzerland)***Synaptic accumulation of FUS triggers early misregulation of synaptic RNAs in ALS-FUS mice* |
| **14:30 – 14:45** | Break (15 minutes) |
| **14:45 – 15:00** | **Ilary Allodi (University of Copenhagen, DK)***Locomotor deficits in a mouse model of ALS are paralleled by loss of Engrailed-1 interneurons* |
| **15:00 – 15:30** | **Alain Prochiantz (Collège de France, France)***EN1 homeoprotein transcription factor is a direct non-cell autonomous survival factor for spinal cord α-Motoneurons* |
| **15:30 – 15:35** | Conclusion of the meeting |