



Oral Program

Thursday November 1, 2018

07:30-08:15 Morning Coffee available | *Sunset Pavilion South*

07:30-08:30 Registration | *Room: Sunset Foyer*

Room *Sunset Ballroom*

08:00-08:15 Welcoming Remarks

08:15-09:45 Session 1: Aberrant translation and neurological disease Session Chair: Daniela Zarnescu

08:15-08:45 Ribosome rescue factors and neurological function
Susan Ackerman, *University of California San Diego, USA*

08:45-09:15 Diverse biology of tRNA-derived small non-coding RNAs
Paul J. Anderson, *Brigham and Women's Hospital, Harvard Medical School, USA*

09:15-09:30 Characterization of neuropathological features of miR-218 and exploration of miR-218 as a candidate of biomarker for early diagnosis of ALS
Sandra Diaz-Garcia, *UC San Diego, USA*

09:30-09:45 ALS/FTD-linked mutation in FUS suppresses intra-axonal protein synthesis and drives motor neuron and FTD like-disease without nuclear loss-of-function of FUS
Sandrine Da Cruz, *Ludwig Institute, USA*

09:45-10:15 Refreshment break | *Sunset Pavilion South*

10:15-11:45 Session 2: Consequences of pathological repeat expansions Session Chair: Dieter Edbauer

10:15-10:45 Production and toxicity of Poly(GR) in C9ORF72-ALS/FTD
Fen-Biao Gao, *University of Massachusetts Medical School, USA*

10:45-11:15 C9ORF72 haploinsufficiency synergizes the toxicity of GGGGCC RAN translation, a double hit mechanism that can be prevented by drugs activating autophagy
Nicolas Charlet-Berguerand, *University of Strasbourg, France*

11:15-11:30 ADAR2 mislocalization and widespread RNA editing aberrations in C9orf72 mediated ALS
Stephen Moore, *Barrow Neurological Institute, USA*

11:30-11:45 The RNA binding protein Zfp106 protects against neurotoxicity caused by C9orf72 GGGGCC repeats
Barbara Celona, *University of California, USA*

11:45-14:00 Lunch & Poster Session 1 | *Sunset Pavilion South*

14:00-15:30 Session 3: RNA-binding protein metabolism and neurodegeneration Session Chair: Robert Bowser

14:00-14:30 Formation and spreading of TDP-43 pathology in animal models
Virginia Lee, *University of Pennsylvania, USA*

14:30-15:00 Working with mouse models to understand ALS and then move into translation
Elizabeth Fisher, *University College London, UK*

15:00-15:15 Activity-dependent TDP43 accumulation in iPSC-derived neuron models of ALS
Sami Barmada, *University of Michigan, USA*

15:15-15:30 Structural and functional MRI reveals cerebellar and frontal cortical deficits in the TDP-43^{Q331K} knock-in mouse model of ALS-FTD
Jemeen Sreedharan, *King's College London, UK*

15:30-16:00 Refreshment break | *Sunset Pavilion South*

16:00-17:30 Session 4: RNA-protein assemblies and neurological disease Session Chair: Hong Joo Kim

16:00-16:30 Amyloid-like TDP-43 myo-granules associate with sarcomeric RNAs during skeletal muscle formation
Roy Parker, *University of Colorado, USA*

16:30-17:00 RNA binding proteins as engineers of neuronal health
Gene Yeo, *University of California San Diego, USA*

17:00-17:15 RNA aggregation in repeat expansion disorders
Ankur Jain, *Whitehead Institute, USA*

17:15-17:30 Optogenetic enhancement of TDP-43 intermolecular interaction triggers its cytoplasmic mislocalization and inhibits axon outgrowth of spinal motor neurons
K. Asakawa, National Institute of Genetics, Japan

17:30-19:00 **Welcome Reception** | Sunset Terrace

Friday November 2, 2018

07:30-08:00 Morning Coffee available | Sunset Pavilion South

Room *Sunset Ballroom*

08:00-09:30 **Session 5: Repeat-associated non-ATG translation and disease** Session Chair: **Sandra Almeida**

08:00-08:30 Targeting RAN proteins improves phenotypes in *C9orf72* BAC ALS/FTD mice
Laura Ranum, University of Florida, USA

08:30-09:00 Ribosome heterogeneity in translating the genetic code
Maria Barna, Stanford University, USA

09:00-09:15 RAN translation, cellular stress pathways, and neurodegeneration in repeat expansion disorders
Peter Todd, University of Michigan, USA

09:15-09:30 Repeat-associated non-AUG (RAN) translation of expanded hexanucleotide repeats in *C9ORF72*-ALS/FTD
Shuying Sun, Johns Hopkins University School of Medicine, USA

09:30-10:00 **Refreshment break** | Sunset Pavilion South

10:00-11:30 **Session 6: Nucleocytoplasmic transport and disease** Session Chair: **Tom Lloyd**

10:00-10:30 Nuclear Pore complex as a contributor to neurodegeneration
Jeff Rothstein, Johns Hopkins University School of Medicine, USA

10:30-11:00 Multiple roles of nuclear import receptors in protein aggregation diseases
Dorothee Dormann, Ludwig Maximilian University of Munich, Germany

11:00-11:15 Mechanistic insights into the complex cation- π interactions, antiparallel β -sheet and molecular hydrogen bonding that contribute to physiological and pathological LLPS and gelation of FUS
Peter St George-Hyslop, University of Cambridge, UK

11:15-11:30 Defective RNA interaction drives aberrant phase separation and cellular aggregation of ALS-linked mutant FUS
Sua Myong, Johns Hopkins University, USA

11:30-13:30 **Lunch and Poster Session 2** | Sunset Pavilion South

13:30-15:30 **Session 7: Aberrant phase transitions and neurological disease** Session Chair: **Nickolas Kramer**

13:30-14:00 Dynamic RNA-protein assemblies and neurological disease
Paul Taylor, HHMI and St. Jude Children's Research Hospital, USA

14:00-14:30 The role of RNA binding proteins in tauopathies
Benjamin L. Wolozin, Boston University, USA

14:30-15:00 Driving forces of tau-RNA complex coacervation and relationship to tau aggregation
Songi Han, University of California Santa Barbara, USA

15:00-15:15 Functional and pathological RNA-binding protein phase separation with atomistic detail
Nicolas Fawzi, Brown University, USA

15:15-15:30 Photokinetic modulation of TDP-43 proteinopathy reveals mechanism of pathogenic aggregation
Christopher Donnelly, University of Pittsburgh, USA

15:30-16:00 **Refreshment break** | Sunset Pavilion South

16:00-17:15 **Session 8: The advent of therapeutics for disorders of RNA metabolism** Session Chair: **Jeff Rothstein**

16:00-16:30 Translation of gene therapeutics in neurological and neuromuscular diseases
Brian Kaspar, AveXis Inc., Illinois, USA

16:30-17:00 Premature polyadenylation and loss of the neuronal stathmin-2 inhibits axonal regeneration in TDP-43-dependent neurodegeneration
Don Cleveland, University of California San Diego, USA

17:00-17:15 **Closing Remarks**