

*First evening of events will be held at the Harn Museum  
SW 34th St and Hull Road, Gainesville, FL 32611*

## Welcome Reception and Symposium Check-In – Harn Museum

6:00-6:45 Drinks and light hors d'oeuvres

Harn Museum. Wine, beer and hors d'oeuvres.  
Pick up your symposium registration packet.

6:50-6:55 **Welcome, Symposium Overview and Acknowledgments – Laura Ranum**

6:55-7:00 **Introduction to Keynote Speaker – Chair Maurice Swanson**

## *Keynote Speaker*

7:00-8:00 **Roy Parker, PhD**  
*University of Colorado Boulder, USA*

***RNP Granules in Health and Disease***

8:00-9:00 **Selected Poster talks**

*6 poster talks (8 +2 minutes)*

8:00-8:10 **Kaitlin Weskamp, MSc**  
*University of Michigan, USA*

*Single-Cell TDP43 synthesis and metabolism in human iPSC derived neurons*

8:10-8:20 **Argel Aguilar Valles, PhD**  
*McGill University, Canada*

*Translational control of depression via phosphorylation of eukaryotic translation initiation factor 4E*

8:20-8:30 **Zachary McEachin, MSc**  
*Emory University/Georgia Tech, USA*

*RAN translation in SCA36*

8:30-8:40 **Lin Guo, PhD**  
*University of Pennsylvania, USA*

*Nuclear-import receptors reverse aberrant phase transitions of RNA binding proteins with prion-like domains*

8:40-8:50 **Shizuka Yamada**  
*Stanford University, USA*

*RPS25 regulates C9orf72 RAN Translation*

8:50-9:00 **David Borchelt, PhD**  
*University of Florida, USA*

*Characterization of gene regulation and protein interaction networks for Matrin 3 encoding mutations linked to amyotrophic lateral sclerosis and myopathy*

*All talks will be held in the CGRC Auditorium Room 101  
Poster Session will be held in Room 451*

7:30-8:20 ***Check in, Symposium packet pickup and Continental Breakfast***  
*Room 133, CGRC*

8:25 ***Welcome by*** **David S. Guzick, MD, PhD** *Senior Vice President, Health Affairs and  
President, UF Health*

***Session 1 RNA Transitions and Disease Therapies — Chair Laura Ranum***

8:30-8:55 **Ron Vale, PhD** *University of California San Francisco, USA* *RNA aggregation and repeat expansion disorders*

8:55-9:20 **Paul Taylor, MD, PhD** *St. Jude Children's Research Hospital, USA* *Disturbance of biological phase transitions drives ALS,  
FTD and myopathy*

9:20-9:30 **Peipei Zhang, PhD** *St. Jude Children's Research Hospital, USA* ***Selected Poster Talk: Reconstitute stress granules  
formation in vivo with a light inducible system***

9:30-9:55 **Aaron Gitler, PhD** *Stanford School of Medicine, USA* *Expanding insights into mechanisms of C9orf72  
mutations in ALS and FTD*

9:55-10:20 **Beverly Davidson, PhD** *University of Pennsylvania, USA* *Huntington's disease beyond the brain*

10:20-10:40 ***Coffee Break — Room 133, CGRC***

***Session 2 RNA and RBPs in Development and Disease — Chair Mingyi Xie***

10:40-11:05 **Sami Barmada, MD, PhD** *University of Michigan, USA* *Abnormal RNA stability in ALS and FTD*

11:05-11:30 **Leslie Thompson, PhD** *University of California, USA* *Systems biology in Huntington's disease and ALS*

11:30-11:55 **Maury Swanson, PhD** *University of Florida, USA* *RNA misprocessing in development and neurological  
disease*

11:55-12:20 **Gary Bassell, PhD** *Emory University* *Muscleblind-like RNA binding proteins form RNA  
transport granules associated with Kif1ba*

12:20-1:10 ***Lunch — Room 133, CGRC***

**1:00-2:00** *Poster session*  
Room 451 CGRC

Presenters will be available for discussion and judging.

### Session 3 ALS and FTD – Chair Jeehye Park

**2:10-2:35** **Clotilde Lagier-Tourenne, MD, PhD**  
Massachusetts General Hospital, USA

*Modeling C9ORF72 disease: a crucial step for therapeutic development in ALS/FTD*

**2:35-3:00** **Bryan Traynor, MD, PhD**  
National Institutes of Health, USA

*Genome analyses identify KIF5A as a novel ALS Gene*

**3:00-3:25** **Rosa Rademaker, PhD**  
Mayo Clinic, USA

*Genetic disease modifiers in frontotemporal dementia with progranulin gene mutations*

**3:25-3:50** **Jada Lewis, PhD**  
University of Florida, USA

*Matrin 3 and its role in ALS and distal myopathy*

**3:50-4:10** *Coffee Break – Room 133, CGRC*

### Session 4 Therapeutic Strategies I – Chair Matthew Disney

**4:10-4:35** **Tony Ho, MD**  
CrisprTX, Cambridge, USA

*Bringing CRISPR to the Clinic*

**4:35-5:00** **Jan Grimm, PhD**  
Neurimmune

*Human antibodies for the treatment of protein aggregation diseases*

**5:00-5:25** **Stefan Pulst, MD**  
University of Utah, USA

*ASO-targeting of ATXN2 and related proteins in mouse models of SCA2*

**5:30** **Announcements**

*Arrangements for Transportation to Gala Dinner*

Gala Dinner 6:30pm-9:30pm

See information of Page #7 for details of Gala Dinner event

7:30-8:20 *Check in, Symposium packet pickup and Continental Breakfast*  
*Room 133*

### Session 5 New Insights into Neurodegeneration – Chair Leonard Petrucelli

- 8:30-8:55 **Steven McKnight, PhD**  
*University of Texas Southwestern, USA* *Protective benefits of NAD to damaged or dying nerve cells*
- 8:55-9:20 **Gene Yeo, PhD**  
*UCSD, USA* *Stressing about granules: insight into new components that target neurodegeneration*
- 9:20-9:30 **Yi Lin, PhD**  
*UT Southwestern Medical Center, USA* ***Selected Poster Talk:** Mechanism of action studies of the toxic PR<sub>n</sub> and GR<sub>n</sub> poly-dipeptides encoded by the C9orf72 repeat expansion*
- 9:30-9:55 **Paul Hagerman, MD, PhD**  
*University of California Davis, USA* *Pathogenic mechanism(s) of Fragile X-associated tremor/ataxia syndrome*
- 9:55-10:20 **Todd Golde, MD, PhD**  
*University of Florida, USA* *Can we model neurodegeneration in a dish: massive tauopathy in brain slice cultures?*

10:20-10:40 *Coffee Break – Room 133, CGRC*

### Session 6 Proteinopathy and Neurological Disease – Chair David Borchelt

- 10:40-11:05 **Yves Barral, PhD**  
*Zurich, Switzerland* *Protein aggregation and ageing: damage or adaptation?*
- 11:05-11:30 **Magdalini Polymenidou, PhD**  
*University of Zurich, Switzerland* *Pathological TDP-43 assemblies and disease heterogeneity*
- 11:30-11:40 **Joshua Wheeler, PhD**  
*University of Colorado, Boulder, USA* ***Selected Poster Talk:** Amyloid-like TDP-43 myo-granules associate with sarcomeric RNAs during skeletal muscle formation*
- 11:40-12:05 **Laura Ranum, PhD**  
*University of Florida, USA* *Inhibition of RAN through PKR pathway improves phenotypes in C9ORF72 BAC ALS/FTD mice*
- 12:05-12:30 **Nahum Sonenberg, PhD**  
*McGill University, Canada* *Selective reduction of eIF2 $\alpha$  phosphorylation in excitatory neurons enhances learning and memory*

12:30-1:30 *Lunch – Room 133, CGRC*

**Session 7 Neuronal Circuits and CNS Dysfunction – Chair Gordon Mitchell**

- 1:30-1:55 Solange Brown, MD, PhD**  
*Johns Hopkins Medicine, USA* *Cortical hyperexcitability in ALS*
- 1:55-2:20 George Mentis, PhD**  
*Columbia University, USA* *Distinct mechanisms cause dysfunction and death of motor neurons in spinal muscular atrophy*
- 2:20-2:30 Juhyun Kim, PhD**  
*Johns Hopkins University School of Medicine, USA* *Selected Poster Talk: Stage-dependent changes in cortical neuron excitability in an hSOD1<sup>G93A</sup> mouse model of amyotrophic lateral sclerosis*
- 2:30-2:55 Eric Wang, PhD**  
*University of Florida, USA* *Molecular, cellular, and behavioral studies of the Myotonic Dystrophy Central Nervous System*
- 2:55-3:20 Jeffery Rothstein, MD, PhD**  
*Johns Hopkins Medicine, USA* *Nuclear pore and nuclear transport dysfunction in sporadic and familial ALS*

**3:20-3:40 Coffee Break – Room 133, CGRC**

**Session 8 Therapeutic Strategies II – Chair James Wymer**

- 3:40-4:05 Frank Bennett, PhD**  
*Ionis Pharmaceuticals, USA* *Antisense oligonucleotide based therapies for neurological diseases*
- 4:05-4:30 James Shorter, PhD**  
*University of Pennsylvania, USA* *Reversing aberrant phase-separation events connected to ALS/FTD*
- 4:30-4:40 Lien Nyugen, PhD**  
*University of Florida, USA* *Selected Poster Talk: Immunotherapy development for C9ORF72 ALS/FTD using a BAC transgenic mouse model and human antibodies targeting RAN proteins*
- 4:40-5:05 Andy Berglund, PhD**  
*University of Florida, USA* *Selective transcriptional inhibition of expanded microsatellite repeats*
- 5:05-5:30 Charles Thornton, MD, PhD**  
*University of Rochester, USA* *Update in progress from Wellstone Center and Myotonic Dystrophy Clinical Research Network*

**5:30 Patrick Concannon, PhD**  
*University of Florida, USA* *Poster Awards*

**5:45 Laura Ranum, PhD**  
*University of Florida, USA* *Closing Remarks*