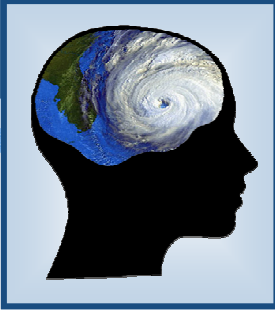


December 14, 2015



All talks will be held in the CGRC Room 101  
Poster Session will be held in the Atrium.

7:30 *Check in and Continental Breakfast—Room 133, CGRC*

8:20 **Welcoming remarks— Laura Ranum, PhD**  
University of Florida

**Session 1** Chair: Eric Wang, PhD

- 8:30-8:55 **Maurice Swanson, PhD** *HnRNP-mediated RNA mis-splicing in myotonic dystrophy*  
University of Florida, USA
- 8:55-9:20 **Thomas Cooper, MD** *Mouse models for myotonic dystrophy: yes, no, maybe*  
Baylor College of Medicine, USA
- 9:20-9:45 **Jeffrey Rothstein, MD, PhD** *Blocking and Unblocking Nuclear Traffic in Neurodegeneration*  
Johns Hopkins University, USA
- 9:45-10:10 **Fen-Biao Gao, PhD** *Lessons from Drosophila Models of C9ORF72-related FTD/ALS*  
University of Massachusetts, USA

10:10-10:30

*Coffee Break— Room 133, CGRC*

**Session 2** Chair: Timothy Ebner, PhD

- 10:30-10:55 **Philip Wong, PhD** *TDP-43 in Health and Disease*  
Johns Hopkins University, USA
- 10:55-11:20 **Christopher Ross, PhD** *Pathogenesis of HD*  
Johns Hopkins University, USA
- 11:20-11:45 **Leslie Thompson, PhD** *Targeting protein homeostasis and gene networks in HD models*  
University of California, Irvine, USA
- 11:45-12:10 **Monica Banez-Coronel, PhD** *RAN Translation in Huntington Disease*  
University of Florida, USA

12:10-1:10

*Lunch—Room 133, CGRC*

# Day One

1:10-2:10 **Poster session 1**

Posters will be displayed in the Atrium of the Cancer Research Genetics Complex building—First floor.

## **Session 3** Chair: Laura Ranum, PhD

2:10-2:35 **Suja Jagannathan, PhD**  
Fred Hutchinson Cancer Research  
Center, USA

*Disruption of RNA and protein quality control in FSHD*

2:35-3:00 **P. Hande Ozdinler, PhD**  
Northwestern University, USA

*Understanding cellular and molecular basis of corticospinal motor neuron vulnerability*

3:00-3:25 **Anne Messer, PhD**  
Neural Stem Cell Institute, USA

*Active and passive vaccination approaches to HD therapeutics.*

3:25-3:45

*Coffee Break—Room 133, CGRC*

## **Session 4** Chair: Anne Messer, PhD

3:45-4:10 **Jan Grimm PhD**  
Neurimmune, Switzerland

*Development of Human-Derived Antibodies for Neurodegenerative Proteinopathies*

4:10-4:35 **Westley Friesen, PhD**  
PTC Therapeutics, USA

*Targeting splicing to treat genetic disease*

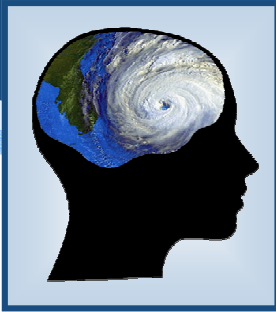
4:35-5:00 **Andy Berglund, PhD**  
University of Florida, USA

*Targeting toxic RNA at the level of transcription with small molecules*

5:00 **Announcements/Brief Closing Remarks**

6:30 **Gala Dinner**  
*Invitation only*

*Shuttle service will be available for those who need it.*



December 15, 2015

All talks will be held in the CGRC Room 101

7:30 *Check in and Continental Breakfast—Room 133, CGRC*

8:25 **Welcoming remarks— Maurice Swanson, PhD**  
University of Florida

**Session 5** Chair: Maurice Swanson, PhD

- |            |   |  |
|------------|---|--|
| 8:30-8:55  | <b>Marc Diamond, MD</b><br>University of Texas Southwestern,<br>USA | <i>Tau Prion Strains: Implications for Cause, Diagnosis and Therapy of Tauopathies</i>         |
| 8:55-9:20  | <b>David Borchelt, PhD</b><br>University of Florida USA             | <i>Transmissible proteinopathies in neurodegenerative disease</i>                              |
| 9:20-9:45  | <b>Todd Golde, PhD</b><br>University of Florida USA                 | <i>An rAAV based toolkit to accelerate preclinical therapeutic discovery for CNS disorders</i> |
| 9:45-10:10 | <b>Gordon Mitchell, PhD</b><br>University of Florida USA            | <i>The trick is to keep breathing: compensatory respiratory plasticity in ALS</i>              |

**10:10-10:30**

*Coffee Break— Room 133, CGRC*

**Session 6** Chair: Andy Berglund, PhD

- |             |   |  |
|-------------|---|--|
| 10:30-10:55 | <b>Roy Parker, PhD</b><br>University of Colorado, USA                           | <i>Analysis of mammalian stress granules and their implications for neurodegenerative diseases</i> |
| 10:55-11:20 | <b>Gary Bassell, PhD</b><br>Emory University, USA                               | <i>mRNA granule dynamics, transport and local translation in neuronal health and disease</i>       |
| 11:20-11:45 | <b>Eric Wang, PhD</b><br>University of Florida, USA                             | <i>Global Approaches for Studying RNA Regulation in Microsatellite Repeat Diseases</i>             |
| 11:45-12:10 | <b>Matthew Taliaferro, PhD</b><br>Massachusetts Institute of<br>Technology, USA | <i>Distal alternative last exons localize mRNA to neural projections</i>                           |

**12:10-1:10**

*Lunch—Room 133, CGRC*

# Day Two

## Session 7

Chair:  
Jeffrey Rothstein, MD, PhD

- 1:10-1:35 **Leonard Petrucelli, PhD** *Pathobiology of C9 RAN translated proteins*  
Mayo Clinic—Jacksonville, FL, USA
- 1:35-2:00 **Jie Jiang, PhD** *Gain of toxicity from GGGGCC repeat expansions of C9orf72 in ALS and FTD is alleviated by antisense oligonucleotides targeting repeat-containing RNAs*  
University of California San Diego, USA
- 2:00-2:25 **Laura Ranum, PhD** *BAC transgenic model of C9ORF72 ALS/FTD*  
University of Florida, USA
- 2:25-2:50 **Nicolas Sergeant, PhD** *Development and characterization of small molecules against Tauopathies*  
Inserm, France
- 2:50-3:15 **Matthew Disney, PhD** *Is Selective and Potent Targeting of RNA Repeats with Small Molecules Possible?*  
Scripps Research Institute, Florida, USA
- 3:15 **Announcements/Brief Closing Remarks**

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3:30-5:00 Florida Natural History Museum and Butterfly Rainforest—  
*~additional information in the back of the program book~*

6:00 ***Farewell Reception***  
Harn Museum of Art  
University of Florida  
*~additional information in the back of the program book~*