

## 2018 Brainstorm Symposium January 17, 18 and 19

### DAY 1 – January 17, 2018

#### Wednesday

- 6:00-6:45 pm      **Reception and Registration (pick up name badges and agenda)**  
6:50-6:55      Welcome and Symposium Overview – Laura Ranum  
6:55-7:00      Introduction to Keynote speaker – Maury Swanson
- 7:00-8:00 pm      **Keynote Speaker:**  
**Roy Parker**  
*RNP Granules in Health and Disease*
- 8:00-9:00pm      Poster talks by 10-12 trainees? (5 min each)

### Day 2 January 18, 2018

#### Thursday

#### Session 1      ***RNA Transitions and Disease Therapies***

- 8:30-8:55 am      **Ron Vale**  
*RNA aggregation and repeat expansion disorders*
- 8:55-9:20 am      **Paul Taylor**  
*Disturbance of biological phase transitions drives ALS, FTD and myopathy*
- 9:20-9:45 am      **Aaron Gitler**  
*(Topic will be: C9orf72 molecular mechanisms)*
- 9:45-10:10 am      **Beverly Davidson**

#### **10:10-10:30 am**      **Coffee Break— Room 133, CGRC**

#### Session 2      ***RNA and RNAs in Development and Disease***

- 10:30-10:55 am      **Sami Barmada**  
*Abnormal RNA stability in ALS and FTD*
- 10:55-11:20 am      **Leslie Thompson**  
*Systems biology in Huntington's disease and ALS*
- 11:20-11:45 am      **Maury Swanson**  
*RNA misprocessing in development and neurological disease*

11:45-12:10 pm **Gary Bassell**  
*Muscleblind-like RNA binding proteins form RNA transport granules associated with Kif1ba*

**12:10-1:00 Lunch—Room 133, CGRC**

**1:00-2:00 Poster presentations**

**Session 3 *ALS and FTD***

2:00-2:25 pm **Clotilde Lagier-Tourenne**  
*Modeling C9ORF72 disease: a crucial step for therapeutic development in ALS/FTD*

2:25-2:50 pm **Bryan Traynor**  
*Genome analyses identify KIF5A as a novel ALS Gene*

2:50-3:15 pm **Rosa Rademakers**  
*Genetic disease modifiers in frontotemporal dementia with progranulin gene mutations*

3:15-3:40 pm **Jada Lewis**  
*Matrin 3 and its role in ALS and distal myopathy*

**3:40-4:00 pm Coffee Break**

**Session 4 *Therapeutic Strategies and Screens***

4:00-4:25 pm **Tony Ho**  
*Translation of edited cell therapies (e.g. guide RNAs, vectors and vector production systems)*

4:25-4:50 pm **Andy Berglund**  
*Selective transcriptional inhibition of expanded microsatellite repeats*

4:50-5:15 pm **Stefan Pulst**  
*ASO-targeting of ATXN2 and related proteins in mouse models of SCA2*

**6:30-9:30 pm Gala Dinner- Sweet Water Inn**

## DAY 3 – January 19, 2018

### Friday

7:30-8:30 am      **Breakfast**

#### **Session 5**      **New Insights into Neurodegeneration**

8:30-8:55

**Steven McKnight**

*Protective benefits of NAD to damaged or dying nerve cells*

8:55-9:20

**Gene Yeo**

*Stressing about granules: insight into new components that target neurodegeneration*

9:20-9:45

**Paul Hagerman**

*Pathogenic Mechanism(s) of Fragile X-associated Tremor/Ataxia Syndrome*

9:45-10:10

**Todd Golde**

*Can we model neurodegeneration in a dish: massive tauopathy in brain slice cultures?*

10:10-10:30

**Coffee Break**

#### **Session 6**      **Proteinopathy and Neurological Disease**

10:30-10:55

**Yves Barral**

*Protein Aggregation and Ageing: Damage or Adaptation?*

10:55-11:20

**Magdalini Polymenidou**

*Pathological TDP-43 assemblies and disease heterogeneity*

11:20-11:45

**Laura Ranum**

*Topic - RAN translation – various diseases, maybe stress info*

11:45-12:10

**Nahum Sonenberg**

*Translational control of neurodevelopmental diseases (e.g. Fragile X) by eIF4E*

12:10-1:00

**Lunch**

**Session 7**      **Neuronal Circuits and CNS Dysfunction**

- 1:00-1:25      **Solange Brown**  
*Cortical hyperexcitability in ALS*
- 1:25-1:50      **George Mentis**  
*Distinct mechanisms cause dysfunction and death of motor neurons in spinal muscular atrophy*
- 1:50-2:15      **Eric Wang**  
*Talk will focus on efforts to better understand CNS defects in DM1.*
- 2:15-2:40      **Jeffrey Rothstein**  
*Nuclear pore and Nuclear transport dysfunction in sporadic and familial ALS*

**2:40-3:00**      **Coffee Break**

**Session 8**      **Current and Approaching Therapies to Disease**

- 3:00-3:25      **Frank Bennett**  
*Antisense oligonucleotide based therapies for neurological diseases*
- 3:25-3:50      **James Shorter**  
*Reversing aberrant phase-separation events connected to ALS/FTD*
- 3:50-4:15      **Jan Grimm submitted the abstract**  
*Human antibodies for the treatment of protein aggregation diseases*
- 4:15-4:40      **Charles Thornton**  
*Update in progress from Wellstone Center and Myotonic Dystrophy Clinical Research Network*
- 4:40              **Announcements/Brief Closing Remarks**

**January 20, 2018**

**Saturday**

Check out of hotel by 12 noon