21st Annual Packard Center ALS Research Symposium

MONDAY, MARCH 8, 2021
Zoom Link: https://zoom.us/j/92313026655?pwd=WjVzVm5SbDlpSEIXY0tleU5hUG1Kdz09

10:00 AM  Jeffrey Rothstein, MD, PhD
Johns Hopkins University
Welcome and Announcements

SECTION CHAIR: Jeffrey Rothstein, MD, PhD

10:10 AM  Laura Ranum, PhD
University of Florida
Immunotherapy Approaches for C9orf72-ALS/FTD

10:30 AM  Jiou Wang, PhD
Johns Hopkins University
An eIF2α Kinase That Responds to Proteotoxic Stress

10:50 AM  Fen-Biao Gao, PhD
University of Massachucetts Medical Center
Molecular Mechanisms of Poly(GR)/Poly(PR) Toxicity in C9orf72-ALS/FTD

11:10 AM  Phil Wong, PhD
Johns Hopkins University
TDP-43 Cryptic Exon Encoded Peptides as Fluid Biomarkers for ALS-FTD

11:30 AM  Gene Yeo, PhD, MBA
University of California, San Diego
Persistent mRNA Localization Defects and Cell Death in ALS Neurons Caused by Transient Cellular Stress

11:50 AM  DISCUSSION (10 mins)

12:00 PM  BREAK (20 mins)
SECTION CHAIR: Piera Pasinelli, PhD

12:20 PM    Len Petrucelli, PhD  
Mayo Clinic  
*Mechanistic Link Between c9orf72 and TDP-43*

12:40 PM    Aaron Gitler, PhD  
Stanford University  
*New TDP-43 Targets*

1:00 PM    Jeffrey Rothstein, MD, PhD / Alyssa Coyne, PhD  
Johns Hopkins University  
*CHMP7: Therapeutic Alleviation of Nuclear Pore Injury and Downstream TDP-43 Dysfunction in Human ALS*

1:20 PM    Tom Lloyd, MD, PhD  
Johns Hopkins University  
*Nucleoporins are Degraded via Upregulation of the ESCRT-III/Vps4 Complex in Drosophila Models of C9-ALS/FTD*

1:40 PM    Don Cleveland, PhD  
University of California, San Diego  
*Multiple Phases of TDP-43*

2:00 PM    DISCUSSION (10 mins)

SECTION CHAIR: Sami Barmada, MD, PhD

2:10 PM    Peter Todd, MD, PhD  
University of Michigan  
*Repeat RNA Behavior and Interacting Proteins in RAN Translation and Neurodegenerative Disease*

2:30 PM    J. Paul Taylor, MD  
St. Jude Children’s Research Hospital  
*Recent Studies of Biomolecular Condensation and Cellular Response to Stress*

2:50 PM    James Shorter, MA, PhD  
University of Pennsylvania  
*Countering Deleterious Phase Transitions in ALS*
3:10 PM  Clotilde Lagier-Tourenne, MD, PhD  
Massachusetts General Hospital  
*Stathmin-2: An Emerging Therapeutic Target in TDP-43 Proteinopathies*

3:30 PM  DISCUSSION (10 mins)

3:40PM  DAY ONE CONCLUDES

Please join us this evening

VIRTUAL POSTER SESSION  
5:00 PM – 7:00 PM EST  
https://spatial.chat/s/PCPosterSession

Poster PDFs and Abstracts: www.packardsymposium.org
21st Annual Packard Center ALS Research Symposium

TUESDAY, MARCH 9, 2021

Zoom Link: https://zoom.us/j/92157417766?pwd=aVlVNjF2UlI5Y2NzYkJvUGlxeDZuUG10Zz09

SECTION CHAIR: Aaron Gitler, PhD

10:00 AM  Dorothee Dormann, PhD
Ludwig-Maximilians - University of Munich
ALS-Linked Phosphorylation Controls TDP-43 Phase Separation and Aggregation

10:20 AM  Magdalini Polymenidou, PhD
University of Zurich
Synaptic Accumulation of FUS Triggers Early Misregulation of Synaptic RNAs in ALS-FUS Mice

10:40 AM  Adrian Isaacs, PhD
University College London
New C9orf72 Knock-In Mouse Models

11:00 AM  Davide Trott, PhD/ Maria Cicardi, PhD
Thomas Jefferson University
Exosomal Secretion of DPRs as Mechanism to Propagate Disease in C9orf72-ALS/FTD

11:20 AM  Chris Donnelly, PhD
University of Pittsburgh
TDP-43 Dysfunction in ALS

11:40 AM  DISCUSSION (10 mins)

11:50 AM  BREAK (20 mins)
**SECTION CHAIR: Chris Donnelly, PhD**

12:10 PM  **Erika Holzbaur, PhD**  
University of Pennsylvania  
*Autophagy and Mitophagy in Neuronal Homeostasis and Neurodegeneration*

12:30 PM  **Michael Coleman, PhD**  
University of Cambridge  
*Molecular Mechanism of Programmed Axon Death and Approaches to Understanding its Roles in Human Disease*

12:50 PM  **Michael Ward, MD, PhD**  
National Institutes of Health  
*Unexpected Links Between RNA Splicing, DNA Damage, and TDP-43*

1:10 PM  **Rita Sattler, PhD/ Justin Ichida, PhD**  
Barrow Neurological Institute/ University of Southern California  
*Role of Microglia in C9orf72-ALS/FTD*

1:30 PM  **Deepi Lall, PhD (Balogh Lab)**  
Cedars-Sinai Medical Center  
*Role of C9orf72 in Neuroinflammation and Neurodegeneration*

1:50 PM  **DISCUSSION (10 mins)**

**SECTION CHAIR: Emily Baxi, PhD**

2:00 PM  **Leslie Thompson, PhD**  
University of California, Irvine  
*Updates from Answer ALS*

2:20 PM  **Bryan Traynor, MD, PhD**  
National Institutes of Health  
*New Genes in Adult ALS and Juvenile ALS*

2:40 PM  **Matthew Harms, MD**  
Columbia University  
*Update on the GTAC Study*

3:00 PM  **Shuying Sun, PhD**  
Johns Hopkins University  
*RNA Metabolism in C9orf72-ALS/FTD*
3:20 PM  Aaron Hauesler, PhD  
Thomas Jefferson University  
*Identifying Modulatory Mechanisms for C9orf72 NRE Locus Methylation*

3:40 PM  DISCUSSION  (10 mins)

3:50 PM  DAY TWO CONCLUDES

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Please join us this evening  

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5:00 PM – 7:00 PM EST  
https://spatial.chat/s/PCPosterSession  

Poster PDFs and Abstracts: www.packardsymposium.org
21st Annual Packard Center ALS Research Symposium

WEDNESDAY, MARCH 10, 2021

Zoom Link: https://zoom.us/j/97540091774?pwd=bjRHdlFkaVVyM2VVam41alVjMmllQT09

SECTION CHAIR: Piera Pasinelli, PhD

10:00 AM  
Timothy Miller, MD, PhD  
Washington University  
RNA-Targeted Therapy for SOD1-ALS

10:20 AM  
Sami Barmada, MD, PhD  
University of Michigan  
Physiological and Pathophysiological Turnover of MATR3 in ALS Disease Models

10:40 AM  
Yongjie Yang, PhD  
Tufts University  
Dysfunctional Astroglial Exosome to (Motor) Neuron Axon Signaling in ALS

11:00 AM  
Kevin Eggan, PhD  
Harvard University  
Connecting Alterations in RNA Metabolism to Motor Neuron Dysfunction in ALS

11:20 AM  
Jinsy Andrews, MD, MSc  
Columbia University  
Northeastern ALS Consortium (NEALS) ALS Clinical Trials Update

11:40 AM  
DISCUSSION (10 mins)

11:50 AM  
BREAK (20 mins)
SECTION CHAIR: Jeffrey Rothstein, MD, PhD

12:10 PM  Lindsey Hayes, MD, PhD  
Johns Hopkins University  
*Nuclear RNA Binding Regulates TDP-43 Availability for Passive Nuclear Export*

12:30 PM  Eran Hornstein, PhD  
Weizmann Institute of Science  
*Spatiotemporal Proteomic Analysis of Stress Granule Disassembly Using APEX Reveals Regulation by SUMOylation and Links to ALS Pathogenesis*

12:50 PM  Claire Le Pichon, PhD  
National Institutes of Health  
*Single Nucleus RNA-Sequencing Defines Unexpected Diversity of Cholinergic Neuron Types in the Adult Mouse Spinal Cord*

1:10 PM  Cathleen Lutz, PhD  
The Jackson Laboratory  
*Modeling Familial and Idiopathic ALS in Mice*

1:30 PM  Sua Myong, PhD  
Johns Hopkins University  
*Molecular Analysis of ALS/FTLD Linked FUS Mutations*

1:50 PM  Poster Presentation from Poster Session Winner

2:10 PM  DISCUSSION (10 mins)

2:20 PM  CLOSING REMARKS

SAVE THE DATE!  
The 22nd Annual Packard Center Symposium  
February 28 – March 2, 2022  
Baltimore, MD