THE ALS RESEARCH FORUM
Discovering a Common Goal, Discovering a Cure
PRIZE4LIFE ALS ASSOCIATION

ALS Research Forum e-Newsletter Vol. 177 **July 19, 2017**

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Research News

Blocking SRSF-Mediated Nuclear Export May Protect Motor Neurons in C9orf72 ALS

A new treatment strategy may help protect motor neurons in C9orf72 ALS according to a new study led by University of Sheffield's **Pamela Shaw** in England. The study found that depleting the SR protein SRSF1 blocked the export of expanded repeat transcripts into the cytoplasm in a neuronal cell model of the disease, reducing the synthesis of dipeptide repeat proteins. What's more, this approach protected motor neurons against toxicity mediated by patient-derived C9orf72 ALS astrocytes (iAstrocytes) in co-culture. The findings build on previous studies led by St Jude's **J. Paul Taylor** in Tennessee and Johns Hopkins University's **Jeff Rothstein** in Maryland, which found that the loss of key components of the nuclear export machinery reduced toxicity in a *Drosophila* model of C9orf72 ALS (see August 2015 news). The study is published on July 5 in *Nature Communications*.

Scientists Get a Jump on Retrotransposons and ALS/FTD

Nearly half of the human genome consists of retrotransposable elements, kept silent through multiple mechanisms. But according to a new RNA seq analysis led by Mayo Clinic's **Leonard Petrucelli** in Florida, more than 150 of these repetitive LTR and LINE elements may become re-activated and contribute to neuronal toxicity in the frontal cortex of the brain in people with C9orf72 ALS. The findings build on previous studies led by National Institute of Health's **Avindra Nath** in Maryland and Stonybrook University's **Joshua Dubnau** in New York that suggest that the derepression of at least some of these elements may contribute to ALS including sporadic disease (see October 2015 and March 2017 news). The study is published on June 16 in *Human Molecular Genetics*.

Scientists CREATE New Gene Therapy Technologies. But Can They Deliver For ALS?

A new gene delivery vehicle may help increase the efficacy of gene therapies being developed for ALS. The adeno-associated virus AAV-PHP.eB, developed by a research team led by **Benjamin Deverman** and **Viviana Gradinaru** at the California Institute of Technology, penetrates the central nervous system, including neurons and astrocytes. What's more, the vector, derived from AAV-PHP.B, can transduce more

than 50% of these cells using at least 10-fold lower doses (about 4 to 5 x 10^{12} vector genomes/kg) according to a preclinical analysis, and is therefore more suitable for clinical applications. The study is published on June 26 in *Nature Neuroscience*. <<**Read more**.

For Better and Worse, TDP-43 Controls Microglia's Phagocytic Prowess

Microglia fuel the progression of ALS. But according to a new study led by University of Zurich's **Lawrence Rajendran** in Switzerland, these dysregulated immune cells may also contribute to the onset of the disease. The study found, in part, that reduction of microglial TDP-43 promoted the microglial-mediated engulfment of synapses - at least in mice. The results suggest that microglia may contribute to neurodegeneration in ALS with TDP-43 pathology by promoting synapse loss. The findings add to growing evidence that key changes in microglia may play a key role in the development of the disease. The study is published on June 29 in *Neuron*.

Human and Mouse Microglia Look Alike, but Age Differently

Researchers beginning in the late 1990s turned to mouse models of ALS to better understand the role of microglia in the disease. But according to a new study led by University of Groningen's **Bart Eggen** in the Netherlands, these cells may exhibit key age-related and immune system differences, compared to people, that may influence how they contribute to the disease. The study is published on July 3 in *Nature Neuroscience*.

Check out our website to read more of the latest research advances in ALS.

Funding Opportunities:

July 2017

Harrington Rare Disease Scholar Award. In collaboration with Takeda Pharmaceuticals. Focus: Neurology and Gastroenterology. LOI due by **July 19, 2017**.

Accelerating Drug Discovery for FTD. Alzheimer's Drug Discovery Foundation and the Association of Frontotemporal Dementia. LOI due by July 31, 2017.

August 2017

Judith and Jean Pape Adams ALS Research Grants. Judith and Jean Pape Adams Foundation. Application due by August 4, 2017.

NeuroNEXT Clinical Trials. NIH, NINDS. Application due by August 3, 2017.

NIH Blueprint Neurotherapeutics Network: Small Molecule Drug Discovery and Development for Disorders of the Nervous System. Application due by August 9, 2017.

The Betty Laidlaw MND Research Prize. MND Australia. Applications due August 25, 2017.

MND Research Grants. MND Australia. Applications due August 25, 2017.

Postdoctoral Fellowships. MND Australia. Applications due August 25, 2017.

September 2017

MDA Venture Philanthropy Program. Muscular Dystrophy Association. LOI due by September 1, 2017.

NEW! Clinical Research Pilot Grant. Association for Frontotemporal Degeneration. Application due by

September 8, 2017.

NEW! Basic Research Pilot Grant. Association for Frontotemporal Degeneration. Application due by September 8, 2017.

Research Grants. Frick Foundation for ALS Research. Application due by September 30, 2017.

Check out our updated list of grants and awards.

Job Opportunities:

Professor, Dementia Research Institute. Cardiff University. Cardiff, Wales.
Professor, Environmental Health. Exposome Analysis. Florida International University. Miami, FL.
Physician-Scientist, Temple University. Philadelphia, PA.
Translational Scientist. Tsinghua University School of Medicine. Beijing, China.
Assistant Professor, Neurobiology. Virginia Tech. Blacksburg, VA
Neurologist, Rehabilitation Service. University of Oklahoma. Oklahoma City, OK.
Computational Neuroscientist, Krembil Research Institute. Toronto, Canada.
Research Study Coordinator. University of Miami. Miami, Florida,
Research Technician, Massachusetts General Hospital. Charlestown, MA.

Director of Clinical Operations, Alector. San Francisco, CA.
Scientist I, ALS. Biogen. Cambridge, MA.

Hiring someone onto your team? Contact us to add your listing to our updated job board: ALSjobs@prize4life.org.

Full List of Job Opportunities >>

Upcoming Meetings:

July 2017

July 23-28, 2017. Stowe, Vermont. Gordon Research Conference on Amyotrophic Lateral Sclerosis (ALS) and Related Motor Neuron Diseases.

September 2017

September 4-5, 2017. Symposium Latsis: Degeneration of Neural Circuits. Lausanne, Switzerland. Abstracts due: July 31, 2017.

September 7-9, 2017. Ottawa, Canada. Ottawa International Conference on Neuromuscular Disease and Biology.

September 16-21, 2017. Kyoto, Japan. World Congress on Neurology.

September 17-19, 2017. Sitges, Spain. Neuro-Immune Axis: Reciprocal Regulation in Development, Health and Disease.

October 2017

October 3-5, 2017. Clearwater Beach, Florida. The 16th Northeast ALS Consortium Meeting. Abstracts due: August 4.

Organizing an ALS meeting? Contact us to add your conference to our updated calendar: ALSmeetings@prize4life.org.

Full List of Upcoming Meetings>>

Download the Working with ALS Mice Manual Here

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