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

ALS Research Forum e-Newsletter Vol. 184 **November 1, 2017**

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Research News

Scientists Unleash CRISPR on RNA

A new CRISPR-Cas13 technique may enable ALS-linked mutations to be corrected more safely. The RNA editing approach, developed by a research team led by Broad Institute's **Feng Zhang** in Cambridge, Massachusetts, seeks out and replaces a targeted adenosine with an inosine using an ADAR2-based mechanism. The strategy, known as REPAIR, is one of a growing number of techniques that may reduce the risks of emerging CRISPR-Cas therapies by targeting the mRNA encoded by the disease-linked gene (see October 2017 news). The study is published on October 25 in *Science*.

To learn more about how scientists aim to leverage the CRISPR editing system to treat ALS, check out *A New CRISPR Technique Fries C9orf72 RNAs*.

Mutant FUS Jams Axons, Blocking a Deacetylase May Help

Cellular cargo pile up in the axons of motor neurons in ALS, potentially leading, in part, to their vulnerability (see January 2012, February 2014 news). But whether this traffic tie-up is a key contributor or a consequence of the disease remains hotly debated (see March 2012 news). Now, researchers at VIB in Belgium report that patient-derived mutant FUS motor neurons exhibit tell tale signs of ALS including reduced axonal transport of ER vesicles and mitochondria. What's more, blocking the histone deacetylase HDAC6 helps clear this axonal traffic jam. The iPS-based model system may enable scientists to further evaluate the role of axonal trafficking in ALS and the potential of emerging HDAC6 inhibitors to treat the disease. The study appeared on October 11 in *Nature Communications*.

Gene Expression Map of Human Body Gives Value to Variants

Researchers are increasingly turning to genome-wide association analysis in hopes to identify genetic factors that contribute to ALS. But many of the variants identified in these studies lie in non-coding regions and therefore, provide few clues as to why these genetic changes may lead to the disease. Now, a new open access resource may help experts zero in on key mechanisms underlying neurodegenerative diseases including ALS. The database, developed by the **Genotype-Tissue Expression (GTEx) Consortium**, includes maps of the expression quantitative trait

loci (eQTL) landscape of nearly 450 healthy people across 44 tissues including 10 regions of the brain. The study appeared on October 11 in *Nature*.

FTD Variant Alters Chromatin, Boosts Expression of TMEM106b

Changes in the architecture of the genome may contribute to the onset of ALS and FTD according to a new study led by University of Pennsylvania's **Alice Chen-Plotkin** in Philadelphia. The study found that a key FTD-linked variant may increase the risk of developing the disease by increasing levels of TMEM106B by facilitating long-range chromatin interactions. This chromatin remodeling mechanism, mediated by CTCF, may also contribute to other neurodegenerative diseases including ALS according to subsequent analysis. The study is published on October 19 in the *American Journal of Human Genetics*.

ALS Kin Have More Neuropsychiatric Disease

ALS may share some of the same genetic roots as neuropsychiatric diseases including autism and obsessive-compulsive disorder (OCD) according to a new study led by Beaumont Hospital's **Orla Hardiman** in Dublin. The population-based case-control study found that these diseases occur more frequently in families of people with ALS - at least in Ireland. The results build on a previous study from Hardiman's team, which found that ALS and schizophrenia may be genetically associated and thereby, may share disease mechanisms (McLaughlin et al., 2017). Together, the findings open up the possibility that network disruptions in the brain may contribute to ALS. The study is published on October 16 in *JAMA Neurology*.

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

Funding Opportunities:

The Weston Brain Institute is now accepting applications for funding early phase clinical trials in Canada for diseases including FTD. Collaborators can be based worldwide.

November 2017

Biomedical Research Project Grants. MND Association. Applications due by **November 3, 2017**.

Dynamic Neuroimmune Interactions in the Transition from Normal CNS Function to Disorders. NIH Blueprint for Neuroscience Research. LOI due by **November 7, 2017**.

ALS ACT (Accelerated Therapeutics). Academic-Industry Partnerships. Phase I/II Clinical Trials. ALS Finding a Cure, ALS Association and NEALS. LOI due by **November 10, 2017**.

December 2017

MDA Venture Philanthropy Program. LOI due by December 1, 2017.

NEW! Clinical Scientist Development Awards. Doris Duke Charitable Foundation. Pre-Proposal due by December 1, 2017.

NEW! Technological Innovations in Neuroscience Awards. McKnight Foundation. LOI due by December 4, 2017.

NeuroNEXT Clinical Trials. NINDS. Applications due by December 6, 2017.

Transformational Research: Canada 2018. Weston Brain Institute. LOI due by December 7, 2017.

January 2018

NEW! Altman Award in Developmental Neuroscience. Japan Neuroscience Society. Application due by January 31, 2017. Scientists worldwide are invited to apply.

Check out our updated list of grants and awards.

Job Opportunities:

Director, Division of Neuroscience. NINDS. Washington, DC.
Director, Center for Neurodegenerative Disorders. University of Maryland. Baltimore, MD.
Assistant, Associate or Full Professor, Neurobiology. Barrow Neurological Institute. Phoenix, AZ.
Assistant, Associate Professor, Neuroscience. Albert Einstein School of Medicine. New York, NY.
Assistant, Associate Professor, Neuroscience. Iowa Neuroscience Institute. Iowa City, IA.
Assistant Professor, Neuroscience. Yale University School of Medicine. New Haven, CT.
Assistant or Associate Professor, Neuropathology. Brigham and Women's Hospital. Boston, MA.
Assistant Professor. University of Massachusetts. Amherst, MA.
Junior Group Leader, Psychiatry & Neuroscience. Paris-Descartes University. Paris, France.
Postdoctoral Fellow, Sareen Lab. Cedar-Sinai Medical Center. Los Angeles, CA.
Postdoctoral Fellow, Strittmatter Lab. Yale University. New Haven, CT.
Postdoctoral Fellow, Verfaillie Lab. KU Leuven. Leuven, Belgium.
Research Assistant, Spillantini Lab. University of Cambridge. Cambridge, MA.
Research Technician, Morrison and Rothstein Labs. Johns Hopkins University. Baltimore, MD.
Clinical Coordinator, Division of Neuromuscular Disorders. University of Miami. Miami, FL.

Director, Sr. Director, Neurodegeneration and Repair. Biogen. Cambridge, MA.
Sr. Clinical Scientist. Biogen. Cambridge, MA.
Sr. Associate Scientist. Biogen. Cambridge, MA.
Senior Scientist. LifeArc (MRC Technology). Stevenage, England.

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:

November 2017

November 10, 2017. Sydney, Australia. 13th MND Australia Research Conference.

November 11-15, 2017. Washington, D.C. Society for Neuroscience Annual Meeting.

November 21-23, 2017. Trieste, Italy. Atypical Dementias: From Diagnosis to Therapies. FTD.

December 2017

December 2, 2017. Philadelphia, PA. Cell Biology of Neurodegeneration and Repair in the Nervous System.

December 7, 2017. Boston, MA. ALS/MND Allied Professionals Forum.

December 8-10, 2017. Boston, MA. International Symposium on ALS/MND.

March 2018

March 11-14, 2018. Arlington, VA. MDA Clinical Conference.

March 18-21, 2018. EMBL. Heidelberg, Germany. Microglia 2018. Abstracts due: February 15, 2018.

March 22-24, 2018. Melbourne, Australia. Australasian Motor Neurone Disease Symposium. Abstracts due: December 18, 2017.

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

STAY CONNECTED: