



ALS Research Forum e-Newsletter Vol. 204

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Research News

[Let 'Er Rip: ALS/FTD Variant Plus Aging Unleash Deadly Kinase](#)

A new study may explain why aging may make motor neurons more vulnerable to ALS. The study, led by Harvard Medical School's **Junying Yuan** in Cambridge, Massachusetts, found that levels of the RIPK1 inhibitor TAK1 (mRNA) dropped 80% in the prefrontal cortex in the brain in people that are older than 60. What's more, blocking RIPK1 by reducing levels of TAK1 *and* TBK1 resulted in tell-tale signs of ALS/FTD in mice including neuroinflammation and neuronal loss. The results add to growing evidence that inhibiting RIPK1 may help protect motor neurons from the disease ([September 2016](#) news, see also [February 2017](#) news). A clinical trial in healthy volunteers is ongoing (see [July 2018](#) news).

To learn more about the potential role of RIPK1 kinase in ALS, check out [For Motor Neurons to Survive, NEK1 May Be On the Line](#).

[Targeting SOD1 ALS From Within](#)

A new strategy may help reduce motor neuron toxicity in SOD1 ALS. The approach, developed by a research team led by University of Chicago's **Raymond Roos** in Illinois, uses intracellular single-chain antibody fragments, known as intrabodies, to target misfolded SOD1 for destruction. The intrabodies, which are encoded by a single gene, can be packaged into adeno-associated viruses (AAVs) including AAV9, enabling delivery into motor neurons and glia in the brain and spinal cord. The study appeared on August 31 in *Neurobiology of Disease*.

Check out [this recent article](#) to find out how scientists are using these same strategies to target TDP-43 in ALS.

[Scientists Go Long To Navigate C9orf72 Repeat Sequences](#)

Understanding how repeats expand in the C9orf72 gene is critical to determine how the most common genetic form of ALS develops *and* progresses. Sequencing these repeat-rich sequences is a key first step. But charting this repeat-rich region of the C9orf72 gene in people with ALS can be tricky due to its GC-rich nature. Now, a research team led by **Leonard Petrucelli** and **John Fryer** at the Mayo Clinic in

Jacksonville, Florida report that long-range next sequencing technologies can be used to navigate these expanded sequences in the C9orf72 gene. The study is published on August 21 in *Molecular Neurodegeneration*.

To find out about strategies to identify cases of C9orf72 ALS including on the population scale for GWAS, check out [A New Test for C9orf72 ALS?](#)

[Treating ALS in a Dish? Models Point to Drug Candidate for Sporadic Disease](#)

A new approach may help scientists identify treatments for ALS, including sporadic disease. The high-content high-throughput strategy, developed by a research team led by Keio University School of Medicine's **Hideyuki Okano** in Tokyo, Japan, identifies potential therapies for ALS by screening for suppression of at least four phenotypes including the number of stress granules, TDP-43 pathology and key signs of neurodegeneration such as neurite shrinkage. The approach is one of a growing number of strategies that aims to use ALS patient-derived motor neurons to identify existing medicines that can be repurposed as potential treatments for the disease. The study is published on August 20 in *Nature Medicine*.

To learn more, check out [A New Drug Discovery Approach May Help Tackle ALS Heterogeneity](#).

['Organ on a Chip' Models the Ins and Outs of the Blood-Brain Barrier](#)

A new microfluidic device may help scientists develop drugs for ALS. The blood-brain barrier in vitro model, developed by Harvard University's **Donald Ingber** and **Kit Parker** in Cambridge, Massachusetts, may help scientists optimize the ability of potential ALS drugs to enter the brain and spinal cord by enabling the assessment of blood-brain barrier permeability. The "organ on chip" approach is one of a growing number of strategies scientists are developing to study the blood-brain barrier and how it is impacted by neurodegenerative disease (for review, see [Aday et al., 2016](#)). The findings appeared on August 20 in *Nature Biotechnology*.

To find out how scientists aim to deliver therapies into the CNS including ASOs and antibodies, check out *Exosomes*: [Bubbling Over the Blood-Brain Barrier](#).

Check out [our website](#) to read more of the latest research advances in ALS.

Funding Opportunities:

Final weeks to apply for the [Richard Olney Clinical Scientist Development Award](#) funded by the ALS Association and the American Brain Foundation! Deadline: October 1, 2018.

September 2018

[Sloan Research Fellowships](#). Alfred P. Sloan Foundation. Nominate an early-career scientist today! Letters of support due by **September 17, 2018**.

[Innovative Technologies to Deliver Genome Editing Machinery to Disease-Relevant Cells and Tissues](#). NIH. LOI due by **September 18, 2018**.

[Genomics of Rare Diseases](#). French Foundation for Rare Diseases. Funding focus: Next-Gen Sequencing Initiatives. Applications due by **September 25, 2018**.

[Pilot Projects Investigating Understudied G Protein-Coupled Receptors, Ion Channels, and Protein Kinases](#). Illuminating the Druggable Genome. NIH. LOI due by **September 26, 2018**.

[Research Grants](#). Frick Foundation for ALS Research. Application due by **September 30, 2018**. Note the revised due date!

October 2018

[Clinical Research Training Scholarship in ALS](#). ALS Association and the American Brain Foundation. Application due by October 1, 2018.

[Richard Olney Clinical Scientist Development Award](#). ALS Association and the American Brain Foundation. Application due by October 1, 2018.

NEW! [Discovering New Therapeutic Uses for Existing Molecules](#) (Pre-Application). NCATS. Letter of Intent due by October 1, 2018.

[Career Awards for Medical Scientists](#). Burroughs Wellcome Fund. Application due by October 1, 2018.

[Canada Gairdner International Award](#). Gairdner Foundation. Focus: Human Biology and Disease. [Nominations](#) due by October 1, 2018. Researchers around the world are encouraged to apply!

[Research Grants](#). Basic Neuroscience. Whitehall Foundation. LOI due by October 1, 2018.

[Excellent Paper In Neuroscience Award](#). Network of European Funding for Neuroscience Research. Early Career Scientists. Application due by October 1, 2018.

[Sartorius & Science Prize for Regenerative Medicine & Cell Therapy](#). Science Magazine and the Sartorius Group. Publish in *Science*! Application due by October 1, 2018.

NEW! [Systems Approaches for the Discovery of Combinatorial Therapies for Complex Disorders](#). EU Horizon 2020. Application due by October 2, 2018.

NEW! [MRC AstraZeneca Centre for Lead Discovery Initiative](#). UK. Access provided to AstraZeneca small molecule libraries. Application due by October 3, 2018.

NEW! [MRC UCB Antibody Discovery Initiative](#). UK. Application due by October 3, 2018.

[New Investigator Research Grant](#). Neuroscience and Mental Health. MRC. UK. Application due by October 3, 2018.

NEW! [Computational Science Fellowship](#) (Includes FTD). Canada. Funding Priorities Include: Identifying Potential Biomarkers and Therapies. Modeling Progression Rates. Michael J. Fox Foundation and Weston Brain Institute. Proposal due by October 8, 2018.

November 2018

NEW! [Biomedical Research Project Grants](#). MND Association. Summary application due by November 2, 2018.

NEW! [AP Giannini Foundation Postdoctoral Research Fellowship and Career Development Award](#). Application due by November 5, 2018.

Check out our [updated list](#) of grants and awards.

Job Opportunities:

[Chief Scientific Director, Professor](#), Innovative Genomics Institute. University of CA. Berkeley, CA.

[Managing Director](#), Kavli Institute for Neuroscience. Yale University. New Haven, CT.

[Assistant Professor](#), Biology. Whitehead Institute. MIT. Cambridge, MA.

[Assistant Professor](#), Computational Neuroscience. CalTech. Pasadena, CA.

[Assistant Professor](#), Cell Biology and Neuroscience. Rutgers University. Piscataway, NJ.

[Assistant Professor](#), Molecular Neuroscience. Stanford University. Stanford, CA.

[Assistant Professor](#), Neurobiology. University of Maryland. Baltimore, MD.

[Asst or Assoc. Professor](#), Neuroscience. University of Utah. Salt Lake City, UT.

[Asst or Assoc. Member](#), Neuronal Cell Bio. St Jude Children's Research Hospital. Memphis, TN.

[Assistant Investigator](#) (Stadtman, Tenure-Track), Neuroscience. NIH. Bethesda, MD.

[Associate Professor](#), Neuroscience. Mayo Clinic. Jacksonville, FL.

[Faculty](#) (Multiple Subject Areas). LA BioMed. Harbor - UCLA Medical Center. Los Angeles, CA.

[Faculty](#) (Multiple Subject Areas). Rockefeller University. New York, NY.

[Faculty](#), Neurological Disorders (Open Rank). Hamad Bin Khalifa University. Ar-Rayyan, Qatar.

[Faculty](#), Neuroscience (Open Rank). University of North Carolina. Chapel Hill, NC.

[Faculty](#), Neuroscience (Open Rank). Virginia Tech. Blacksburg, VA.

[Faculty](#), Pathology (Open Rank). Stanford University School of Medicine. Stanford, CA.

[Investigator](#) (Open Rank), Neurobiology of Disease. Gladstone Institutes. San Francisco, CA.

[Assistant Investigator](#). University of Arizona. Tucson, AZ.

[Postdoctoral Fellow](#), Mayr Lab. Memorial Sloan Kettering Cancer Center. New York, NY.

[Postdoctoral Fellow](#), Meyer Lab. Nationwide Children's Hospital. Columbus, OH.

[Graduate Studentship](#), ALS. University of Exeter. Exeter, England.

[Medical Director, Clinical Development \(ALS, SMA\)](#). Biogen. Cambridge, MA.

[Senior Scientist or Scientist](#), Neuroscience. Alnylam Pharmaceuticals. Cambridge, MA.

[Postdoctoral Fellow](#), ALS. Amgen. 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:

October 2018

October 2-4, 2018. Clearwater Beach, FL. [NEALS Annual Meeting](#).

October 23-25, 2018. Fort Worth, TX. [ALS Association Clinical Conference](#).

November 2018

November 1-2, 2018. San Diego, CA. [RNA Metabolism In Neurological Disease](#).

November 3-7, 2018. San Diego, CA. [Society for Neuroscience Annual Meeting](#).

November 28-December 1, 2018. Cold Spring Harbor, NY. [Neurodegenerative Diseases, Biology and Therapeutics](#). Abstracts due by **September 14, 2018**.

December 2018

December 2-4, 2018. Los Angeles, CA. [Translation of Stem Cells To The Clinic, Challenges and Opportunities](#).

December 6, 2018. Glasgow, Scotland. [ALS/MND Allied Professionals Forum](#).

December 7-9, 2018. Glasgow, Scotland. [International Symposia on ALS/MND](#).

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:

