



## ALS Research Forum e-Newsletter Vol. 201

July 25, 2018

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### Research News

#### [CRISPR Gene Editing May Not Be So Precise After All](#)

CRISPR Cas9-mediated gene editing is emerging as a potential strategy to correct mutations that cause ALS. But according to a new study published on July 16 in *Nature Biotechnology*, this approach can result in structural damage to the genome. Could using a Cas9 nickase reduce this collateral damage (see [December 2017](#) news)? Experts weigh in.

Check out [Scientists Unleash CRISPR on RNA](#) and [A New CRISPR Technique Fries C9orf72 RNAs](#) to learn how scientists aim to overcome these obstacles.

#### [More Potent Next-Gen Antisense Oligonucleotides Benefit SOD1 ALS Models](#)

Emerging antisense oligonucleotide (ASO) therapies aim to protect motor neurons in SOD1 ALS by reducing levels of misfolded SOD1. Now, a research team led by Washington University School of Medicine's **Timothy Miller** reports the first preclinical data supporting their "next-generation" approach as a treatment for the disease. The SOD1-targeted ASO, developed by Biogen in Cambridge, MA in collaboration with Ionis Pharmaceuticals in Carlsbad, California, is at the phase 1 stage. The findings appeared on July 16 in the *Journal of Clinical Investigation*.

To learn more about antisense oligonucleotide therapies being developed for ALS, check out [ALS-Targeted ASOs Make Waves at AAN 2018](#).

#### [A New Study Raises Questions About How Axons Degenerate in ALS](#)

The inhibition of the enzyme SARM1 is emerging as a potential strategy to protect the axons of neurons from being destroyed due to injury or chronic neurodegenerative disease (see [February 2015](#), [March 2017](#) news). But according to a new study led by Oregon Health and Science University's **Marc Freeman** and University of Massachusetts Medical Center's **Robert Brown**, this strategy may not reduce axon loss in ALS. The study found that SOD1 G93A ALS model mice that are unable to produce this enzyme did not live longer. And, no improvement in motor function could be detected. The findings appeared on July 14 in *Human Molecular Genetics*.

To find out about other strategies that aim to preserve axons in ALS, check out [For Motor Neurons To Survive, NEK1 May Be On the Line](#).

## [Small Molecule Protein-Protein Disruptors Benefit SOD1 ALS Models](#)

A new approach aims to protect motor neurons against SOD1 ALS by reducing ER stress. The approach, developed by University of Tokyo's **Hidenori Ichijo** and colleagues in Japan, is based on previous work, which suggests that the misfolding of SOD1 leads to constitutive activation of the ER stress response via an ASK1-mediated mechanism ([Nishitoh et al., 2008](#); [Homma et al., 2013](#); see also [June 2016](#) news). The strategy is one of a growing number of small molecule-based therapies that aims to treat SOD1 ALS (see [May 2018](#) news). The study is published on July 10 in *Nature Communications*.

To learn about other small molecule approaches being developed for ALS, check out [Small Molecule Keeps Mutant SOD1 Out Of Trouble](#).

### Conference News

**Keystone 2018: [TREM2: Diehard Microglial Supporter, Consequences Be DAMed](#)**

Microglia contribute to ALS. But according to a recent study led by Brigham and Womens Hospital's **Oleg Butovsky** and colleagues in Boston, these cells may also protect neurons depending on the stage of the disease (see [September 2017](#) news). Now, researchers may think they may know at least one way microglia may be of benefit: recruit peripheral macrophages. Butovsky presented these preliminary findings on June 21 at the 2018 Keystone Symposium *New Frontiers in Neuroinflammation*. Efforts to develop TREM2-activating antibodies are currently underway ([Cheng et al., 2018](#)).

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

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### Funding Opportunities:

Last chance to get your application in for Sanofi Genzyme's [Rare Innovation Challenge!](#)  
Application due by July 27, 2018.

#### August 2018

[Beckman Young Investigator Award. Arnold and Mabel Beckman Foundation](#). Application due by **August 6, 2018**.

[Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases](#). NINDS. Funding focus: clinical outcome measures and biomarkers. Application due by **August 17, 2018**.

[Postdoctoral Fellowships](#). AFM-Téléthon. Researchers across the globe are invited to apply. Applications due: August 28, 2018.

[Clinical Research Fellowships](#). AFM-Téléthon. Researchers across the globe are invited to apply. Applications due: August 28, 2018.

[John Templeton Foundation](#). Funding priorities include basic and translational epigenetics/genetics research. Online inquiries must be submitted by August 31, 2018.

[Betty Laidlaw MND Research Prize](#). MND Australia. Application due by August 31, 2018.

[Research Grants](#). MND Australia. Application due by August 31, 2018.

[Postdoctoral Research Fellowships](#). MND Australia. Application due by August 31, 2018.

#### September 2018

[MDA Venture Philanthropy Program](#). MDA. LOI due by September 1, 2018.

**NEW!** [Tools to Target, Identify and Characterize Non-Neuronal Cells in the Brain](#). NIH Brain Initiative. LOI due by September 4, 2018.

**NEW!** [Basic Science Pilot Grant](#). AFTD. Application due by September 7, 2018.

**NEW!** [Clinical Research Pilot Grant](#). AFTD. Application due by September 7, 2018.

**NEW!** [Research Grants](#). Cure SMA. Funding priorities include SMN regulatory mechanisms. Proposal due by September 7, 2018.

**NEW!** [Biomarkers Across Neurodegenerative Diseases](#). Alzheimer's Association, ARUK, Michael J. Fox Foundation, Weston Brain Institute. LOI due by September 10, 2018.

[Rare Diseases Clinical Research Consortia](#). NIH. Projects funded: biomarkers, outcome assessment measures and studies that inform clinical trial design. LOI due by September 9, 2018.

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

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

## October 2018

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

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

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

**NEW!** [Research Grants](#). Frick Foundation for ALS Research. Application due by October 8, 2018.

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

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## Job Opportunities:

[Assistant or Associate Professor](#), Neuroscience. University of Connecticut. Farmington, CT.  
[Assistant or Associate Professor](#), Neuroscience. Stony Brook University. Stony Brook, NY.  
[Assistant Professor](#), Neuroimmunology. Washington University School of Medicine. St Louis, MO.  
[Professor, Experimental Genetics](#). University of Luxembourg. Belvaux, Luxembourg.  
[Postdoctoral Fellow](#), Baker Lab. Newcastle University. Newcastle Upon Tyne, England.  
[Postdoctoral Fellow](#), Belzil Lab. Mayo Clinic. Jacksonville, FL.  
[Postdoctoral Fellow](#), Bhattacharya Lab. University of Arizona. Tucson, AZ.  
[Postdoctoral Fellow](#), Landers Lab. University of Massachusetts Medical Center. Worcester, MA.  
[Postdoctoral Fellow](#), Whitworth Lab. University of Cambridge. Cambridge, England.  
[Research Technician](#), Suzuki Lab. University of Wisconsin. Madison, WI.

[Director of Biological Discovery](#). Denali Therapeutics. San Francisco, CA.  
[Research Scientist](#), Molecular and Cellular Immunology. Amgen. Cambridge, MA.  
[Research Scientist](#), Medicinal Chemistry. Cytokinetics. Cambridge, MA.  
[Research Scientist](#), Pharmacology. Cytokinetics. Cambridge, MA.  
[Research Associate](#). Regeneron. Tarrytown, NY.

Hiring someone onto your team? Contact us to add your listing to [our updated job board](#): [ALSjobs@prize4life.org](mailto:ALSjobs@prize4life.org).

[Full List of Job Opportunities >>](#)

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## Upcoming Meetings:

Are you attending the 2018 meeting of the Northeast ALS (NEALS) Consortium in Florida? Abstracts are due August 3, 2018. Check the [NEALS website](#) for details.

### August 2018

August 5-10, 2018. Castelldefels, Spain. GRC: [The Role of Innate Immunity, Glia, Neurons, and the Blood-Brain Barrier in the Pathogenesis.](#)

### September 2018

September 16-19, 2018. Heidelberg, Germany. EMBL. [The Human Microbiome.](#)

### October 2018

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

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

**Organizing an ALS meeting? Contact us to add your conference to [our updated calendar](#): [ALSmeetings@prize4life.org](mailto:ALSmeetings@prize4life.org).**

[Full List of Upcoming Meetings>>](#)

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[Download the Working with ALS Mice Manual Here](#)

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