



ALS Research Forum e-Newsletter Vol. 188

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Conference News

[Tirasemtiv Fails in Phase 3; Side Effects Cited](#)

Cytokinetics halted development of its potential muscle booster tirasemtiv as a treatment for ALS because the drug, according to a phase 3 clinical trial analysis, did not provide significant benefit to people with the disease. But according to principal investigator **Jeremy Shefner** of the Barrow Neurological Institute in Phoenix, Arizona, there may be a future in ALS for a muscle booster that it is better tolerated. At the International Symposium on ALS/MND in Boston, Massachusetts last month, experts weighed in on this approach and its potential for people with ALS going forward.

Research News

[Gene Editing Delays Disease Onset in ALS Model Mice](#)

CRISPR-Cas9 is emerging as a potential strategy to treat ALS by correcting the mutation underlying the disease. Now, a research team led by **David Schaffer** at the University of California, Berkeley report that the approach can improve motor function and increase survival - at least in a mouse model of SOD1 ALS. The findings appeared on December 20 in *Science Advances*. University of Massachusetts Medical Center's **Robert Brown** weighs in on this approach and the challenges going forward.

To learn how scientists are using genome editing technologies to develop strategies for ALS, check out [CRISPR: In the Nick of Time for ALS?](#)

[Missing Connections May Explain Cognitive Challenges in ALS](#)

Researchers may be one step closer to understanding why some people with ALS may have trouble planning and making complex decisions (see [January 2016](#) news). The study, led by University of Edinburgh's **Christopher Henstridge** and **Tara Spire-Jones** in Scotland, found in part, that people with ALS that are cognitively impaired had a significantly lower number of synapses in a key region of brain, known as Brodmann area 9, important for cognition. The findings build on previous studies, using MRI and PET brain imaging, which suggest that changes in this region of the brain occur in people with ALS that experience cognitive challenges. The results may help researchers zero in on why these synapses

crumble and therefore, design treatment strategies that may stabilize them. The study appeared on December 22 in *Acta Neuropathologica*.

[Scientists Weigh In On Exercise in ALS](#)

At least some forms of exercise are safe for people with ALS according to a new study led by Johns Hopkins University School of Medicine's **Nicholas Maragakis** in Baltimore, Maryland. The clinical trial compared stationary cycling and weightlifting to "range of motion" stretching, the current standard of care. No difference in the rates of disease progression were detected between the three groups. 60 people with ALS participated in the randomized, 6-month study. The results may help neurologists develop personalized exercise routines for people with ALS to help manage their disease. The study is published online on November 30 in *Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration*.

Deals and Partnerships

[Pfizer and Sangamo Point Fingers at C9orf72 ALS and FTD](#)

Researchers unveiled a new strategy this month to tackle the most common form of ALS. The potential gene therapy, to be developed by **Sangamo Therapeutics** in partnership with **Pfizer**, aims to reduce motor neuron toxicity in C9orf72 ALS. The approach, which involves the use of genetically-engineered zinc finger proteins, is one of a growing number of strategies that aims to protect motor neurons against ALS and FTD by reducing levels of potentially toxic C9orf72 repeat-rich RNAs (see [December 2017](#) news). The strategy is also being developed as a potential treatment for C9orf72-FTD.

To learn more about emerging treatments for C9orf72 ALS, check out [A New CRISPR Technique Fries C9orf72 RNAs](#).

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

Funding Opportunities:

January 2018

[Rapid Isolation and Characterization of Extracellular Vesicles of CNS Origin](#). NIH Blueprint Neurotherapeutics Network. Application due by **January 22, 2018**.

[ALS Research Grants](#). Thierry-Latran Foundation. Abstracts due by **January 29, 2018**.

[Altman Award in Developmental Neuroscience](#). Japan Neuroscience Society. Application due by **January 31, 2018**. Scientists worldwide are invited to apply.

February 2018

[Joint Call for European Research Projects on Rare Diseases](#). E-Rare. Pre-Proposal due by February 6, 2018.

[High Impact Neuroscience Research Resource Grants](#). NINDS. Application due by February 9, 2018.

[Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases](#). Focus: Clinical Outcome Measures and Biomarkers. NINDS. Application due by February 15, 2018.

[Klingenstein-Simons Fellowship in Neuroscience](#). (For Early-Career PIs.) Applications due by

February 15, 2018.

[Transformational Research: Canada 2018](#). Weston Brain Institute. Deadline extended! LOI now due by February 15, 2018.

NEW! [Neuroscience Investigator Awards](#). (For Early-Career PIs.) New York Stem Cell Foundation. Application due by February 21, 2018.

NEW! [Stem Cell Investigator Awards](#). (For Early-Career PIs.) New York Stem Cell Foundation. Application due by February 21, 2018.

April 2018

[Research Grants](#). Potential ALS Therapies: Preclinical to Phase 1. Fight MND. Application due by April 1, 2018.

NEW! [Graduate Student Fellowship](#). AFM-Téléthon. Application due by April 24, 2018.

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

Job Opportunities:

[Scientific Director, VIB](#). University of Antwerp. Antwerp, Belgium.
[Asst. Professor, Pharmacology & Chemical Biology](#). Emory U. School of Medicine. Atlanta, GA.
[Postdoctoral Fellow, Miller Lab](#). King's College London. London, England.
[Postdoctoral Fellow, Mueller Lab](#). University of Massachusetts Medical Center. Worcester, MA.
[Postdoctoral Fellow, Plowman Lab](#). University of Florida. Gainesville, FL.
[Postdoctoral Fellow, Sareen Lab](#). Cedar-Sinai Medical Center. Los Angeles, CA.
[Postdoctoral Fellow, Tompa Lab](#). VIB. Gent, Belgium.
[Postdoctoral Fellow](#). Northwestern University. Evanston, IL.

[Regional Medical Director, Medical Affairs](#), Northeast or Central. AveXis. Remote, US.
[Associate Scientist](#). ALS Therapy Development Institute. Cambridge, MA.
[Postdoctoral Fellow, Braingeneering Technologies](#). Esch-sur-Alzette, Luxembourg.
[Research Associate](#). Voyager Therapeutics. 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:

Attending [FENS 2018](#) in Berlin, Germany this July? Abstracts are due February 13, 2018.

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](#).

April 2018

April 17-21, 2018. Cold Spring Harbor Laboratory. Cold Spring Harbor, NY. [Protein Homeostasis in Health and Disease](#). Abstracts due: February 2, 2018.

April 21-27, 2018. Los Angeles, CA. [Annual Meeting of the American Academy of Neurology](#).

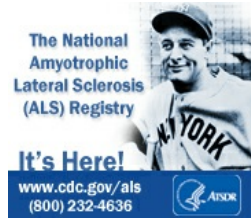
NEW! April 30-May 4, 2018. Jackson Laboratory. Bar Harbor, ME. Workshop: [Using Mouse Models to Study Neurodegenerative Disease](#).

May 2018

April 17-21, 2018. Cold Spring Harbor Laboratory. Cold Spring Harbor, NY. [Cellular Mechanisms Driven by Liquid Phase Separation](#). Abstracts due: February 14, 2018.

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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