



ALS Research Forum e-Newsletter Vol. 179

August 16, 2017

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## Clinical Trial News

### [Cytokinetics CK-107 Muscles In at Phase 2](#)

Researchers are gearing up to evaluate Cytokinetics' potential muscle booster CK-2127107 in the ALS clinic. The drug will be tested at the phase 2 stage. The double-blind randomized placebo-controlled clinical trial aims to determine whether the drug is safe and tolerable for people with ALS and helps them breathe better (SVC). Muscle strength and function (ALS-FRS-R) will also be evaluated. A total of 445 people with ALS are expected to participate. The clinical trial is expected to be completed in July 2018.

Check out [our report](#) to learn more about CK-107, including how it stacks up to tirasemtiv.

## Research News

### [Phosphorylation of FUS Does Away with Droplets](#)

RNA-binding proteins including FUS aggregate in many forms of ALS, potentially leading to motor neuron toxicity. How to target this process remains unclear. Now, a research team led by Brown University's **Nicholas Fawzi** in Rhode Island report that DNA-PK-mediated phosphorylation of the low complexity (LC) domain of FUS blocked its aggregation and cytotoxicity - at least in cultured human cells. The results suggest that stimulating phosphorylation of FUS may help protect motor neurons in ALS. The study builds on previous work led by University of Texas Southwestern's **Steve McKnight**, which found that the LC domain is critical for the liquid liquid phase transition of FUS (see [March 2017](#) news; [Kato et al., 2012](#)). The study is published on August 8 in the *EMBO Journal*.

### [An Emerging ALS Test Is Going to the Dogs](#)

A test may help veterinarians confirm the diagnosis of degenerative myelopathy (DM) in dogs, an emerging large animal model of SOD1 ALS. The test, developed by the University of Missouri's **Joan Coates**, involves the measurement of phosphorylated neurofilament protein H (pNfH) in the cerebrospinal fluid. The strategy builds on previous studies led by VIB's **Philip Van Damme** in Belgium, which found that this approach may enable clinicians to confirm diagnosis of ALS by

facilitating the exclusion of key disease mimics (see [January 2017](#), [May 2017](#) news). The test is now being further validated.

**Check out [our report](#) to learn how dogs with DM are helping to develop therapies for ALS.**

### [New Technology Catalogs Immune Cells In the Brain](#)

Macrophages and T cells infiltrate the central nervous system in people with ALS, promoting inflammation (see [May 2015](#), [April 2017](#) news). But according to a new study led by Israel Institute of Technology's **Asya Rolls** in Haifa that cataloged the immune cells of the mouse brain, many of these cells normally cross the blood-brain barrier and therefore, may help the brain heal and/or fight disease. The resource, created using highly-multiplexed single-cell CyTOF mass cytometry, may enable scientists to understand how these leukocytes help patrol and protect the CNS, and form the groundwork in future studies that investigate their role in ALS. The study is published on July 24 in *Nature Neuroscience*.

### [CRISPR-Cas9 Edits Human Embryos](#)

Scientists are increasingly turning to the CRISPR-Cas9 system to develop potential ALS therapies (see [May 2017](#) news). One of these strategies, reported last week by University of California San Diego's **Gene Yeo** on August 8 in *Cell*, aims to reduce motor neuron toxicity in people with C9orf72 ALS by targeting and eliminating expanded repeat RNAs ([Batra et al., 2017](#)). However, according to a study led by Oregon Health & Sciences' **Shoukhrat Mitalipov** published on August 2 in *Nature*, at least some of these strategies might be able to be implemented earlier, well before the first signs of the disease. In this feature, experts weigh in CRISPR-Cas9 and its potential for people at risk of developing neurodegenerative diseases including ALS going forward.

Stay tuned as we continue to cover this breaking story!

## **Assistive Technology News**

### [Computing by Eye: A Perfect 10?](#)

Using a computer may get a lot easier for people with ALS. Laptops and PCs powered by Windows 10 will soon feature eye-tracking control according to Microsoft. The technology, which works in conjunction with the Tobii 4C eye tracker, enables people with ALS to move a mouse, launch an app or use a virtual keyboard with their eyes. The software, known as "Eye Control", could become available as early as Fall 2017.

**Check out [our report](#) to learn more about this feature, including how to sign up for beta testing.**

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

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## **Funding Opportunities:**

August 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.

[Clinical Research Pilot Grant](#). Association for Frontotemporal Degeneration. Application due by September 8, 2017.

[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.

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## Job Opportunities:

[Director, Waisman Center](#). University of Wisconsin. Madison, WI.

[Chair, Department of Neuroscience](#). University of Connecticut. Farmington, CT.

[Group Leader, Neurogenomics](#). EMBL Australia. Sydney, Australia.

[Asst. Assoc. or Full Professor](#). University of Illinois. Chicago, IL.

[Research Associate, Gao Lab](#). University of Massachusetts Medical Center. Worcester, MA.

[Research Technician, ALS Epidemiology](#). University of Michigan. Ann Arbor, MI.

[Director/Senior Director](#), Neurodegeneration and Repair. Biogen, MA.

[Sr. Clinical Scientist](#), Neurodegeneration and Repair. Biogen, MA.

[Scientist I](#), ALS. Biogen, MA.

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:

Registration is [now open](#) for the 2018 Australasian Motor Neurone Disease Symposium in Melbourne, Australia. Abstracts due: December 18, 2017.

### September 2017

September 4-5, 2017. Symposium Latsis: [Degeneration of Neural Circuits](#). Lausanne, Switzerland.

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](#).

### November 2017

November 11-15, 2017. Washington, D.C. [Society for Neuroscience Annual Meeting.](#)

**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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