



ALS Research Forum e-Newsletter Vol. 173

May 23, 2017

Visit the [ALS Research Forum](#) to read the complete stories featured in this e-newsletter. Friends and colleagues can sign up for the newsletter [here](#). Follow us on [Facebook](#) and [Twitter](#) for the latest updates.

Conference News

In this issue, we feature the latest advances in ALS presented at the 69th Annual Meeting of the American Academy of Neurology (AAN 2017).

[A New Potential Gene Therapy Delivers a Key Milestone](#)

Researchers are developing gene therapy strategies that aim to reduce toxic levels of mutant SOD1 in the CNS in hopes to treat the most common form of inherited ALS. But existing strategies developed to date extend the survival of adult pre-onset SOD1 G93A mice by only 20%. Now, Institute of Myology's **Martine Barkats** and **Maria-Grazia Biferi** in Paris, France introduce a new gene therapy strategy that increases the lifespan of these mice by more than 50%. The researchers received the \$1.0 M USD [Avi Kremer ALS Treatment Prize](#) at the AAN 2017 ALS Association Drug Company Working Group in recognition of their achievement. The strategy is now being optimized and is at the preclinical stage.

Check out [our feature](#) to learn more about gene therapy, including its potential for sporadic disease.

[Mexiletine Alleviates Key Source of Muscle Pain in ALS](#)

More than 90% of people with ALS experience muscle cramps, a key source of pain early in the disease. But no medicines are indicated to treat them. Now, Mayo Clinic's **Bjorn Oskarsson** reports at AAN 2017 that mexiletine reduces muscle cramps in people with ALS by more than 30%. 23 people with ALS participated in the 6-week phase IV study. The drug is now increasingly being used to treat muscle cramps in clinical practice.

Check out [our feature](#) to learn more about mexiletine, including its potential to treat ALS.

Research News

[A New Treatment Approach May PERK Up the ALS Pipeline](#)

Persistent stress granules may contribute to ALS. Reducing the formation of these granules is therefore emerging as a potential treatment for the disease. But existing

therapies that target this process are unsuitable for therapeutic use. Now, **Giovanna Mallucci** and colleagues at the University of Cambridge in the UK introduce a new strategy to identify drugs that restore general translation, which shuts down due to stress granule formation, without the unwanted toxicity. One of these FDA-approved medicines, trazodone, is soon to be tested in the clinic as a treatment for Alzheimer's disease. The study is published on April 19 in *Brain*.

Check out [our feature](#) to learn more about this therapeutic approach and its potential as a treatment for ALS.

[Clinicians Work Up a Biomarker-Based Approach to Confirm ALS Diagnosis](#)

The diagnosis of ALS can take up to 12 months. To expedite this process, researchers are developing biomarkers that can help identify people with ALS. One of these emerging biomarkers, phosphorylated neurofilament H (pNFH), aims to do just that. But neurologists remain unsure about whether this approach can help differentially diagnose the disease. Now, a research team led by VIB's **Philip Van Damme** in Belgium, report that increased levels of pNfH in the CSF can differentiate people with ALS from disease mimics (DMs) at a sensitivity of 90.7% (CI 84.9%-94.8%) and a specificity of 88.0% (CI 75.7%-95.5%). 220 people with ALS and 50 people with DMs participated in the study. The study is published on May 12 in *Neurology*.

Drug News

[FDA Approves Edaravone as a Treatment for ALS](#)

Neurologists weigh in on edaravone and its potential for people with ALS going forward.

In Brief: [Masitinib Phase III Clinical Trial Results In For ALS](#)

AB Science's masitinib may benefit some people with ALS according to phase 3 clinical trial results presented at ENCALS 2017 in Slovenia last week. But according to some experts, another clinical trial may be needed to determine whether the drug slows progression of ALS and in which people with the disease.

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

Funding Opportunities:

The MDA issued [a temporary pause](#) in funding of new research grants for Fall 2017. Applications will continue to be accepted for all other 2017 funding mechanisms.

May 2017

[TREAT ALS](#). Preclinical assessment of potential ALS therapeutics. The ALS Association. LOI due by **today, May 23, 2017**.

June 2017

[MDA Venture Philanthropy Program](#). Muscular Dystrophy Association. LOI due by June 1, 2017.

NEW! [Springboard Challenge](#). Partners HealthCare and the Wellcome Trust. Application due by June 1, 2017.

[ALS Canada Project Grant Program](#). Includes former ALS Discovery, Bridge and Clinical Management grant programs. Application due by June 2, 2017.

NEW! [ALSRP Therapeutic Development Award](#). US Department of Defense CDMRP. Application due by June 9, 2017.

NEW! [ALSRP Therapeutic Idea Award](#). US Department of Defense CDMRP. Application due by June 9, 2017.

NEW! [Next Generation Leaders](#). Allen Brain Institute. Application due by June 9, 2017.

July 2017

[Research and Trampoline Grants](#). AFM-Téléthon. Applicants outside France are encouraged to collaborate with a lab located in France. Application due by July 4, 2017.

[Rapid Response 2017](#). Weston Brain Institute. High-risk high reward translational research. FTD. Canada only. July 5, 2017.

[Accelerating Drug Discovery for FTD](#). Alzheimer's Drug Discovery Foundation and the Association of Frontotemporal Dementia. LOI due by July 31, 2017.

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

Job Opportunities:

[Assistant Professor, Neuroscience and Cell Biology](#). University of Texas. Galveston, TX.

[Assistant Professor, Neuroimaging](#). Karolinska Institutet. Huddinge, Sweden.

[Assistant Professor, Genetics](#). University of Trento. Trento, Italy.

[Clinical Senior Lecturer](#), Neuropathology. Imperial College London. England.

[Clinical Senior Lecturer](#), King's College London. England.

[Senior Lecturer](#), King's College London. England.

[Research Scientist, University of Bologna](#). Bologna, Italy.

[Postdoctoral Fellow, Neurogenetics](#). UMC Utrecht. The Netherlands.

[Postdoctoral Fellow, Respiratory Physiology](#). University of Wisconsin. Madison, WI.

[Postdoctoral Fellow, Van Damme Lab](#). VIB. Leuven, Belgium.

[Postdoctoral Fellow, Cell-Based HTS for Neurodegeneration](#). University of Oxford. England.

[Director of Neurobiology](#). Verge Genomics. Ann Arbor, MI.

[Research Associate II/III](#). Regeneron. Tarrytown, NY.

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:

Abstracts are due June 9 for [Neuro-Immune Axis: Reciprocal Regulation in Development, Health and Disease](#). The meeting will be held on September 17-19, 2017 in Sitges, Spain.

June 2017

June 14-17, 2017. EMBL Heidelberg, Germany. [EMBO-EMBL Symposium: Mechanisms of Neurodegeneration](#).

June 19-23, 2017. Keystone, Colorado. [Keystone Symposium: Neuroinflammation: Concepts, Characteristics, Consequences](#).

June 24-27, 2017. Amsterdam, Netherlands. [3rd Congress of the European Academy of Neurology](#).

June 26-27, 2017. Buckinghamshire, England. [Meeting the Challenges of Modelling Neurodegenerative Disease in Mice](#).

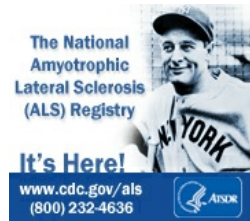
July 2017

July 8-11, 2017. Edinburgh, Scotland. [European Meeting on Glial Cells in Health and Disease.](#)

July 23-28, 2017. Stowe, Vermont. [Gordon Research Conference on Amyotrophic Lateral Sclerosis \(ALS\) and Related Motor Neuron Diseases.](#) Abstracts due: June 25, 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](#)

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