THE ALS RESEARCH FORUM
Discovering a Common Goal, Discovering a Cure
PRIZE4LIFE ALS ASSOCIATION

ALS Research Forum e-Newsletter Vol. 172 **May 9, 2017**

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Drug News

FDA Approves Edaravone as a Treatment for ALS in the US

The Federal Drug Administration (FDA) approved edaravone (MCI-186) on May 5 as a treatment for amyotrophic lateral sclerosis (ALS) in the United States. The decision is based on a six month phase 3 clinical trial, known as "study 19", in Japan which found that edaravone reduced functional decline by 2.49 ALS-FRS-R points over six months compared to placebo in a subset of people recently diagnosed with the disease (see January 2016 news). Edaravone, which is to be marketed by MT Pharma America under the name Radicava, is expected to be available in the United States by August 2017.

A Potential Cramp Reliever Muscles in the ALS Clinic at Phase 2

A potential treatment is approaching the ALS clinic for muscle cramps - a key source of pain early in the disease. The strategy, which includes in part, capsaicin, aims to reduce cramps and muscle spasticity by opening up TRP ion channels in key sensory neurons located in the gastrointestinal tract. The approach, introduced by Harvard University's **Roderick MacKinnon**, is one of two that is being evaluated in the clinic that aims to reduce muscle cramps and spasms in ALS by lowering motor neuron hyperexcitability (see March 2016 news). The strategy, known as FLX-787, is being developed by Flex Pharma in Massachusetts. The clinical trial, which is to be led by Mayo Clinic's **Bjorn Oskarsson** in Florida, is expected to launch in the United States this summer.

Conference News

AAN 2017: A New Outcome Measure Gathers Strength in ALS

A new outcome measure may expedite testing of therapies for ALS according to a retrospective analysis presented at the 69t h Annual Meeting of the American Academy of Neurology in Boston. The strategy, developed by a research team led by Biogen's **Toby Ferguson**, involves manual strength testing of key muscles in the arms and legs. Changes in this outcome measure could be detected over time in people with bulbar and spinal onset ALS, which correlated with key functional outcomes including ALS-FRS, survival and forced vital capacity. A total of 924 people with ALS participated in the study. **Read more**>>

Research News

Ataxin 2 ASOs Aim to De-stress ALS

Reducing levels of ataxin-2 may be a potential treatment strategy for most cases of ALS according to a report published on April 20 in *Nature*. The study, led by **Aaron Gitler** at the Stanford University School of Medicine, found that the injection of ataxin-2 antisense oligonucleotides (ASOs) into the brain of a TDP-43 mouse model of ALS reduced motor deficits and extended survival by 35%. The approach may lower levels of TDP-43 aggregates in the CNS by reducing recruitment of TDP-43 to stress granules. Evaluation of this potential treatment strategy in other mouse models of ALS is now underway.

Check out this feature to learn more about this antisense-based strategy, including its potential for sporadic disease.

Inside Out, or Outside In? ALS Turns on Monocytes in Blood

Monocytes may contribute to neuroinflammation in ALS according to a new study published on April 24 in *JAMA Neurology*. The study, led by Houston Methodist Hospital's **Stanley Appel** in Texas, found, using RNA-seq analysis, that monocytes isolated from patients with ALS appear to be in a proinflammatory state. 43 patients with ALS and 22 healthy volunteers participated in the study. The results suggest that monocytes may be a potential target of the disease.

Read more about the emerging role of monocyte activation in ALS, a potential target of Neuraltus' NP001.

Potential New ALS Gene Leads to Extraordinary Aggregates

A potential new ALS gene is reported on May 3 in *Science Translational Medicine*. The study, led by King's College London's **Christopher Shaw** in England, found, using exosome sequencing analysis, mutations in the vesicle transport protein annexin A11 in two families with ALS that associated with the disease. Subsequent analysis identified mutations in this gene in 2 additional cases of the disease. The results add to growing evidence that an intracellular traffic tie up may contribute to ALS, including the most common form of 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

Bioengineering Research Partnerships. NINDS. Application due by **May 18, 2017**.

TREAT ALS. Preclinical assessment of potential ALS therapeutics. The ALS Association. LOI due by **May 23, 2017**.

June 2017

MDA Venture Philanthropy Program. Muscular Dystrophy Association. LOI 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.

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, Associate, or Full Professor. University of California, San Diego, CA.
Clinical Psychologist. Salford Royal NHS Foundation Trust. Salford, England.
Graduate Research Assistant, Massachusetts General Hospital. Boston, MA.
Research Scientist, University of California. San Diego, CA.
Lab Manager. University of California, San Francisco, CA.

Scientist, Neuroscience. Genentech. San Francisco, CA.
Director/Sr. Director, Neurodegeneration and Repair Research. Biogen. Cambridge, MA.

Biocuration Scientist. Alzforum. Boston, 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:

May 2017

May 11-13, 2017. Bonn, Germany. 5th Venusberg Meeting on Neuroinflammation.

May 18-20, 2017. Ljubljana, Slovenia. ENCALS 2017.

May 22-24, 2017. Barcelona, Spain. Annual World Congress on NeuroTalk. "New Technologies, New Ideas and New Future."

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.

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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