

Visit the ALS Forum website to read the complete stories featured in this e-newsletter. Please forward this e-newsletter to friends and colleagues who may be interested in learning more about ALS.

Resources:

Visit the PRO-ACT Database at www.ALSDatabase.org

[NEALS Biofluid Repository Available to Researchers](#)

[NINDS Fibroblast Repository](#)

Funding News:

National Institute of Environmental Health Sciences (NIEHS) released a new RFA: [Research Linking Environmental Exposure to Neurodegenerative Disease \(R21\)](#). Letter of Intent due September 30, 2013.

Request for Proposals: [Accelerating Drug Discovery for Frontotemporal Dementias](#). Letter of Intent due before August 22, 2013.

Upcoming Webinar:

The ALS Association / NEALS PALS Webinar: ALSUntangled: Opening the X-Files of ALS on July

Research News

[Kinase Mediates Mouse Motor Neuron Disease](#)

In a recent study out of Dr. Eui-Ju Choi's laboratory at Korea University in Seoul, Korea, researchers provided some intriguing evidence to suggest that toxicity resulting from mutations in SOD1 (mSOD1) might be manifested through activation of mammalian sterile 20 (STE20)-like kinase 1 (MST1). The researchers found that MST1 was activated in the spinal cords of mSOD1 mice, but not their wild type counterparts. To investigate if mSOD1 toxicity is mediated through MST1, they created SOD1 G93A mice harboring an MST1 knockout. The mSOD1 mice with the MST1 knockout survived 19 days longer than the mSOD1 mice. Is MST1 activated in ALS patients? And what is the potential mechanism? Click [here](#) to read Dr. Amber Dance's full coverage of this new finding and find out the answers to these and other questions.

[Highlighting the Immune System in ALS](#)

ALS is a complex and heterogeneous disease that involves a number of different cellular players, including but not limited to motor neurons, oligodendrocytes, astrocytes, inflammatory monocytes, and microglia (just to name a few!). A new study recently published online in *Cell Reports* highlights the importance of one of these players: microglia. Dr. Isaac Chiu from Harvard Medical School, along with Prize4Life Scientific Advisory Board member Dr. Tom Maniatis from Columbia University, investigated the role of microglia in ALS. They found that microglia co-express both neurotoxic and neuroprotective factors in SOD1 G93A mice. Although more research is needed to understand the relationship between the expression of these neurotoxic and neuroprotective factors, these [findings](#) suggests that therapeutically targeting the immune response in ALS could be more complicated than originally thought.

[Using Bone Marrow to Hunt Down ALS Biomarkers](#)

Dr. Miguel Weil, a professor at Tel Aviv University's Laboratory for Neurodegenerative Diseases and Personalized Medicine in the Department of Cell Research and Immunology, along with his research group, has used stem cells derived from bone marrow to [identify](#) four genetic biomarkers that are specific to sporadic ALS. The researchers compared stem cells from both healthy individuals and people with ALS

31, 2013 at 2pm EST.
Click [here](#) to register.

Upcoming Short Meeting:

[What's Happening with Big Pharma and Neuroscience Development?](#)

McGovern Institute at MIT, Thursday, August 15, 2013 from 5:30pm to 9:00pm EST.

Upcoming Meetings:

August 10-16, 2013:
Andover, NH: [Gordon Research Conference: Cellular & Molecular Mechanisms of Toxicity](#)

September 4-5, 2013:
Helsinki, Finland: [2nd Biannual European Neurotech Investing and Partnering Conference](#)

September 18-20, 2013:
Aspen, CO: [Accelerating Translational Neurotechnology: Fourth Annual Aspen Brain Forum](#)

September 21-26, 2013:
Vienna, Austria: [XXIth World Congress of Neurology: Neurology in the Age of Globalization](#)

October 2-4, 2013:
Clearwater Beach, FL: [2013 Annual NEALS Meeting](#)

October 3, 2013: Boston, MA: [9th Annual ALS TDI Leadership Summit](#)

November 3-5, 2013: New York, NY: [FasterCures: Partnering For Cures](#)

November 7-8, 2013: San Diego, CA: [8th Annual Brain Research Conference: RNA Metabolism in Neurological Disease](#)

to determine if they could identify any genetic markers specific to ALS. From this comparison, they identified four genes: Cytoplasmic FMR-Interacting Protein 2 (CyFIP2), Retinoblastoma (Rb) Binding Protein 9 (RbBP9), human secretory leukocyte protease inhibitor (SLPI) and the TAR DNA binding protein 43 (TDP-43) that showed differential expression patterns in ALS patients as compared to normal controls. The CyFIP2 and RbBP9 findings were described in the group's recent *Disease Markers* article, and the SLPI and TDP-43 findings were described in their recent *Human Molecular Genetics* publication. The group is currently interested in screening for potential drugs that could affect the expression of these four genes.

[Teva Celebrates Commitment to Neuroscience Research Including ALS](#)

This week Prize4Life's Scientific Director Dr. Neta Zach was invited to attend the [official launch](#) of the National Network of Excellence in Neuroscience (NNE), which is a five year \$15 million initiative supported by Teva Pharmaceutical Industries Ltd. The network was created to promote collaboration among Israeli neuroscience researchers, harnessing Israel's unique neuroscience expertise in order to "increase the potential to translate basic science into therapeutic options". Importantly, the NNE is committed to supporting and furthering ALS research. So far, the NNE has awarded two research grants and two postdoctoral fellowships to ALS researchers. Prize4Life has been working directly with Teva to support this initiative and was instrumental in encouraging the commitment of NNE resources to ALS.

Drug News

[Biogen Idec Partners with University of Edinburgh on MS and MND Research](#)

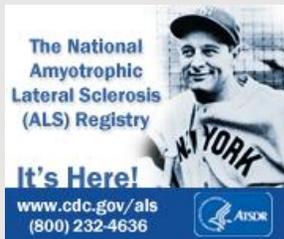
Biogen Idec just [announced](#) that they will be collaborating with the University of Edinburgh in Scotland to further understand multiple sclerosis (MS) and motor neuron disease (MND). The three year collaboration will focus on using the strengths of each partner to identify potential therapeutics for MS and MND. Biogen's VP of neurology research, Ken Rhodes, said the partnership will "provide an in-depth portrait of the pathophysiology of multiple sclerosis and motor neuron disease, while identifying important new targets for potential therapies." Biogen is committed to continuing to invest in ALS, in fact, they just announced an ALS-focused "[Manhattan Project](#)." Stay tuned to the ALS Forum e-Newsletter for additional updates about this new initiative!

[Cytokinetics Goes Ahead with Tirasemtiv Trial and Increases Enrollment](#)

Cytokinetics, Inc. is currently conducting a 500 person Phase II clinical trial of their skeletal muscle activator tirasemtiv for the treatment of ALS. Unfortunately, the Phase II trial had a recent setback due to a contractor's computer programming error that resulted in 58 patients receiving placebo instead of the study drug. To compensate for this error, Cytokinetics recently announced that they are increasing the size of their Phase II trial to 680 patients, which is good news for patients and researchers. The trial is expected to conclude in late 2013, and the results from the trial will be announced sometime in early 2014. Increasing the trial by 180 patients will cost an estimated additional \$5 million. Read more about the changes to the Phase II trial [here](#).

November 9-13, 2013: San Diego, CA: [Society for Neuroscience Annual Meeting: Neuroscience 2013](#)

December 6-8, 2013: Milan, Italy: [24th International Symposium on ALS/MND](#)



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[Results of ALS Studies Provide Support for Stem Cell Therapies](#)

Martin McGlynn the President and CEO of StemCells, a small company focused on developing neural stem cell-based therapeutics, recently [suggested](#) that this is a "pivotal time for stem cell therapeutics." This is especially the case for ALS, as there have been several promising recent clinical trials involving stem cells in ALS. For example, earlier this year BrainStorm Cell Therapeutics Inc. presented the results from their Phase I/II clinical trial to test the tolerability and safety of their NurOwn stem cell technology for the treatment of ALS at the 65th Annual Meeting of the American Academy of Neurology (AAN) Conference held in San Diego, California. The [early results](#) from the trial suggested "initial indications of clinical benefit." BrainStorm is awaiting Food and Drug Administration (FDA) approval in order to move forward with a Phase II trial in the United States. In addition, this past April Neuralstem Inc. received FDA approval for their Phase II study to inject human spinal cord-derived stem cells (NSI-566) directly into the spinal cord for the treatment of ALS. Dr. Eva Feldman, the Principal Investigator on the Neuralstem Phase I and early Phase II clinical trials, recently shared the results from the Phase I studies in a plenary address at the Canadian Neurological Sciences Federation Annual Congress. Dr. Feldman [expressed](#) that in some early stage ALS patients the therapy can "...integrate into the host tissue and nurture and protect the remaining healthy cells, and ... nurse some "sick" cells back to better health." Let's hope the follow-up Phase II studies continue to show promising results for stem cell-based treatments in ALS.

[Amorfix Promises Update on Blood Test Diagnostic for ALS at the MND/ALS 24th International Symposium](#)

In [January](#), Amorfix Life Sciences announced they were developing a blood test that could be used to diagnose ALS, building on initial findings that showed that ALS patients have misfolded SOD1 circulating in their blood. The "simple and rapid blood test" diagnostic will utilize Amorfix's "proprietary antibodies", which can detect misfolded SOD1, in hopes of identifying "patients with early-stage disease" and providing patients earlier access to treatment. Amorfix has promised an [update on the development](#) of this blood test diagnostic at the upcoming 24th Annual International Symposium on MND/ALS, which will be held in Milan, Italy, December 6-8, 2013. Planning to attend ALS/MND this year so that you can learn about the latest research findings in ALS? July 28th is the last day to get the Early Bird registration rate. Click [here](#) to register now.

The ALS Forum was developed by Prize4Life, Inc.
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