



ALS Forum e-Newsletter Volume 77

January 10, 2013

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:

NEW Resource Added!!
Visit the PRO-ACT
Database at
www.ALSDatabase.org

[NEALS Biofluid
Repository Available to
Researchers](#)

[NINDS Fibroblast
Repository](#)

Funding News:

[Opportunities now
available to be a contract
collection site for the
NIMH, NICHD and NINDS
Brain and Tissue
Repository. Deadline:
January 22, 2013](#)

[2013 AAN Foundation
ALS-Richard Olney, MD
Clinician Scientist
Development Three Year
Award](#)

Upcoming Webinars:

January 11, 2013:
[ALSA/NEALS PALS
Webinar: Mexiletine in
Patients with Sporadic](#)

Conference News

[Chicago-ALS Protein SOD1 Painted as Disease Template](#)

Researchers and clinicians gathered at the 23rd International Symposium on ALS/MND held in Chicago, IL from December 5-7 to discuss the latest ALS research topics, including C9ORF72, the [status of current clinical trials](#), and the latest in basic research findings. In part 1 of a four part series, the controversial question, of whether misfolded SOD1 can actively propagate disease, is explored. Dr. Neil Cashman, a professor at the University of British Columbia in Vancouver, Canada, presented some compelling data to suggest that misfolded SOD1 can in fact propagate the disease from cell-to-cell via a "template protein misfolding" mechanism. Read more about how misfolded SOD1 is thought to spread the disease [here](#).

[ALS Clinical Trials: New Hope in Wake of Big Phase 3 Setbacks](#)

In a second comprehensive report from the 23rd International Symposium on ALS/MND, Prize4Life-funded Science Writer Dr. Amber Dance covers the status of a number of ongoing and completed ALS clinical trials. Read the full report to get insights into why the olesoxime (Trofos), ceftriaxone, and now, [dexpramipexole](#) clinical trials failed, how Neuraltus plans to move NP001 into a Phase III study, ALS TDI's plans for testing Gilenya in ALS, the status of the Phase IIb Cytokinetics trial of tirasemtiv, and much more.

Research News

[Parkin Overexpression - A Potential Treatment for ALS and FTD?](#)

In a recent study published online in the *Journal of Biological Chemistry*, researchers at Georgetown University Medical Center have uncovered a surprising potential therapeutic target for both frontotemporal dementia (FTD) and amyotrophic lateral sclerosis (ALS). Often in both FTD and ALS, the normally nuclear TDP-43 protein is sequestered in cytoplasmic protein inclusions, which many researchers believe contributes to neuronal toxicity. However, new research out of Dr. Charbel E-H Moussa's group suggests that it may not be the cytoplasmic TDP-43

[ALS](#)

January 15, 2013: [ALSA Research Update Webinar: 2012 Year In Review](#)

Upcoming Meetings:

January 11-16, 2013: Big Sky, MT: [Keystone Symposium: Multiple Sclerosis](#)

January 15-19, 2013: Hokkaido, Japan: [The Society of Neuromuscular Sciences Incorporated 7th Annual Scientific Meeting](#)

January 22, 2013: Boston, MA: [Harvard NeuroDiscovery Center Student/Faculty Journal Club, Article for discussion is Multimodal Actions of Neural Stem Cells in a Mouse Model of ALS: A Meta-Analysis](#)

January 22, 2013: New York, NY: [The New York Academy of Sciences: T Cells at the Interface of Immune-CNS Cross-Talk](#)

February 3-8, 2013: Santa Fe, NM: [Keystone Symposia Joint Meeting: Neurogenesis & New Frontiers in Neurodegenerative Disease Research](#)

February 10-12, 2013: San Francisco, CA: [7th Annual Drug Discovery for Neurodegeneration Conference](#)

February 19-20, 2013: Manchester, UK: [8th Annual Biomarkers Congress](#)

March 6-7, 2013: Washington, DC: [The Traumatic Brain Injury](#)

inclusions that are toxic, instead toxicity might stem from dysregulated levels of TDP-43 in the nucleus. Dr. Moussa found that increasing the levels of the E3-ubiquitin ligase, parkin, facilitated TDP-43 degradation and slowed disease progressions in TDP-43 mouse and rat models. Read the full story [here](#).

[Columbia University Receives \\$200 Million to Establish Mind Brain Behavior Institute](#)

Columbia University has been given a \$200 million endowment from Mortimer B. Zuckerman to establish the Mind Brain Behavior Institute, which will support interdisciplinary neuroscience research and discovery. Mr. Zuckerman and Columbia University President, Lee Bollinger, along with the Institute's founding co-directors Thomas Jessell and Nobel Laureates Richard Axel and Eric Kandel, joined New York City Mayor Michael Bloomberg to make the announcement. The Mind Brain Behavior Institute will work to develop new therapies and a cure for ALS, as well as other neurodegenerative diseases, including Alzheimer's disease and Parkinson's disease. Read the full story about the Mind Brain Behavior Institute [here](#).

Drug News

[Tau-Targeting Drug Davunetide Washes Out in Phase 3 Trials](#)

ALS is not the only indication suffering a [recent clinical disappointment](#), on December 19, Allon Therapeutics Inc. announced that its potential drug candidate for progressive supranuclear palsy (PSP), davunetide, [did not show efficacy in the Phase 2/3 trial](#). Davunetide is an eight-amino acid peptide derived from the growth factor, activity-dependent neuroprotective protein (ADNP). In ADNP deficient mice, davunetide was able to block tau hyperphosphorylation, and may have contributed to microtubule stabilization. Microtubule stabilization is also thought to be a potential therapeutic opportunity in ALS. In fact, [davunetide was tested in SOD1 G93A mice](#), and showed promising initial results, although further development of davunetide for ALS has not been pursued.

[ALS TDI and to-BBB Form Collaboration To Find Drugs For ALS](#)

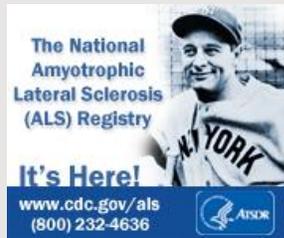
The [ALS Therapy Development Institute](#) (ALS TDI) and [to-BBB](#) have announced their formal collaboration to find treatments for ALS. to-BBB is known for their development of a liposome-based drug delivery system capable of crossing the blood-brain barrier. ALS TDI will use to-BBB's technology to facilitate transportation of potential ALS therapeutics across the blood-brain barrier in a pre-clinical mouse model of ALS. Dr. Steve Perrin, CEO and CSO of ALS TDI, said "This partnership brings a new potential tool to safely enhance the delivery of drugs that may slow or stop the progression of ALS. Being able to dose a drug systemically, but yet have it cross the blood-brain barrier, allows us to try things we weren't able to do before. It enhances the arsenal of weapons we have available in our search for effective treatments for ALS." Read more about the collaboration [here](#).

[Biogen Idec Commits \\$10M to New ALS Research Consortium](#)

Biogen Idec recently announced the creation of a new ALS focused academic research consortium with the long-term goal of identifying

[Conference](#)

March 16-23, 2013: San Diego, CA: [65th Annual American Academy of Neurology Meeting](#)



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potential drugs to treat ALS. The Chief Scientific Officer at Biogen Idec, Spyros Artavanis-Tsakonas, said "We believe that taking a holistic approach that explores the many variables involved in the development and progression of ALS will speed our ability to identify viable drug targets that can be moved into testing." The consortium will include research groups from Yale University, Harvard Medical School, Columbia University, The Rockefeller University and the Harvard Stem Cell Institute. Read more about the consortium [here](#).

[BrainStorm Cell Therapeutics Announces Several Exciting Developments](#)

[BrainStorm Cell Therapeutics Inc.](#) is currently conducting a [Phase I/II clinical trial](#) to collect preliminary efficacy data as well as test the tolerability and safety of their NurOwn technology for the treatment of ALS. On January 7, 2013, BrainStorm announced that its Phase I/II trial has been fast tracked to a Phase IIa dose-escalating trial by the Israeli Ministry of Health. BrainStorm's CEO, Dr. Adrian Harel, said "Acceleration to Phase IIa will save us critical time, enabling us to proceed much more quickly with achieving our goal of developing an effective treatment for ALS." Read more about the details of the Phase IIa study [here](#). In other exciting news, BrainStorm was recently [awarded a \\$3 million NIS grant](#) from Israel's Office of the Chief Scientist (OCS). The grant money will be used to support further development of BrainStorm's NurOwn mesenchymal stem cell technology for the treatment of ALS. Furthermore, BrainStorm announced on December 17 that they had [partnered with Octane Biotech of Kingston, Ontario, Canada](#) to develop a bioreactor that will be used for the production of stem cells. On Monday, January 14, BrainStorm will hold an international conference call to discuss these recent developments. The call will be broadcast in Hebrew at 8:00 am and in English at 8:45 am. Find out how to join the call [here](#).

The ALS Forum was developed by Prize4Life, Inc.
P.O. Box 425783 Cambridge, MA 02142

www.prize4life.org

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