

# THE ALS FORUM

ALS Forum e-Newsletter Volume 76

December 13, 2012

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.

In observance of the holiday season and upcoming New Year, the ALS Forum e-Newsletter will not be published on December 27. We will return to our normal bi-weekly publication schedule on Thursday, January 10.

## 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](#)

[Rare Disease Challenge Be HEARD. Proposal Deadline: December 15, 2012](#)

## Upcoming Meetings:

December 17, 2012:  
London, UK: [Mitochondria and the Central Nervous System](#)

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

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

## Research News

### [Amyotrophic Lateral Sclerosis Database Opens for Business](#)

The full PRO-ACT database ([www.ALSDatabase.org](http://www.ALSDatabase.org)) is now live and is freely available to the global scientific community! PRO-ACT houses the largest ALS clinical trials dataset ever created. PRO-ACT consists of over 8,500 ALS patient records from 18 completed Phase II and Phase III clinical trials conducted by companies including Sanofi, Novartis and Regeneron. Researchers will be able to use this data for a wide variety of purposes, including identifying factors that might either influence or be predictive of disease progression. For example [Prize4Life's DREAM-Phil Bowen ALS Prediction Prize4Life Challenge](#) recently challenged solvers to develop algorithms that could predict the future rate of ALS disease progression using just a subset of the data that is available in the PRO-ACT database. Over 1,000 solvers participated in the ALS Prediction Prize, 39 unique solutions were submitted, and the top three solutions were awarded. Read more about the ALS Prediction Prize Challenge winning solutions [here](#). PRO-ACT is a collaboration between Prize4Life, the Northeast ALS Consortium (NEALS), and the Neurological Clinical Research Institute at Massachusetts General Hospital, and is supported by funding from the ALS Therapy Alliance.

### [Two Genes May One Day Help People with ALS Breathe Better](#)

There are specialized motor neurons in the spinal cord that control breathing. These neurons control and regulate the expansion and contraction of the lungs through connections they have made with the diaphragm. However, how these motor neurons form connections with the diaphragm was previously unknown. Now, in an article published in December's issue of *Nature Neuroscience*, researchers at the NYU Langone Medical Center have discovered two genes, *Hoxa5* and *Hoxc5*, which regulate the formation of these connections. The researchers found that if these two genes are turned off during development, the motor neurons fail to form connections with the diaphragm, which prevents breathing. Learning more about how these two genes work may give us therapeutically relevant insights into ALS. Read more about

### [Annual Scientific Meeting](#)

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](#)

### Resources:

[NEALS Biofluid Repository Available to Researchers](#)

[NINDS Fibroblast Repository](#)

this story [here](#).

### [ALS-Linked Protein Critical For Protein Transport in Neurons](#)

Dr. James Clemens, an assistant professor of biochemistry at Purdue University, recently determined the function of the ALS-linked protein VAPB. In a study that was published in the November 28th issue of *The Journal of Neuroscience*, Dr. Clemens investigated VAPB loss-of-function in *Drosophila* using a mutant version of the fly ortholog of VAPB, Vap-33. He found that Vap-33 is essential for the trafficking of the cell surface receptor Down syndrome cell adhesion molecule (Dscam) to axons, and hypothesized that Vap-33 may also be responsible for trafficking other specific proteins to axons. Extrapolating from these findings, VAPB loss-of-function mutations may therefore significantly disrupt the trafficking of important proteins to axons, contributing to neuronal death in ALS. Read more about these research findings [here](#).

### [A Special Fish May Provide Insights About Neurodegenerative Diseases](#)

Researchers have developed a fish that will help them understand how the power-houses of the cell, the mitochondria, contribute to neuronal death in neurodegenerative diseases, including ALS. This genetically modified zebrafish, called the "MitoFish," has a special fluorescent marker associated with the mitochondria. Scientists can use this marker to monitor and track changes in mitochondria overtime in the neurons of this fish without invasive surgery. The researchers hope that this new model will help scientists learn more about how defects in mitochondria contribute to neurodegenerative diseases. Read more about this story [here](#).

## Conference News

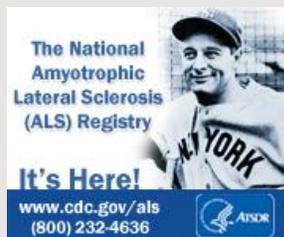
### [ALS/MND Meeting Recap](#)

[The 23rd International Symposium on MND/ALS](#) was held in Chicago, IL from December 5-7. If you weren't able to attend the meeting, you are in luck! You can catch the highlights of the meeting by reading these MND Association blog posts:

- [The clinical trials session](#)
- [Nature, nurture, genetics and / or chance - what causes MND?](#)
- [Reading the stars - why are 'astrocytes' toxic?](#)
- [A prize-winning story worth repeating](#)
- [Mastering Pac-Man](#)
- [Vive la difference!](#)

## Drug News

[BrainStorm Cell Therapeutics Inc. Presents Data From Clinical Trial](#)  
[BrainStorm Cell Therapeutics](#) is in the process of 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. BrainStorm had originally scheduled an international conference call on Tuesday, December 11 to provide updates about this ongoing clinical trial, as well as provide information about upcoming clinical trials. However, they deferred this conference call because Brainstorm was



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selected to present their latest data from their Phase I/II study at the opening presentation of the Israel Neurological Association's Annual Meeting on December 12, 2012 in Jerusalem, Israel. Dr. Dimitrios Karussis, principal investigator of the study, presented the data from the first 12 patients that have been treated in the trial. BrainStorm plans to reschedule their conference call, so stay tuned to the Weekly Digest for updates!

#### [Neuralstem Presents Data from Phase I Study and Announces Exciting News About Phase II Trial](#)

At The 23rd International Symposium on MND/ALS held in Chicago, IL from December 5-7, Dr. Jonathon Glass, Director of the Emory ALS Center, presented some exciting new findings from Neuralstem's Phase I clinical trial. Neuralstem's Phase I trial was designed to test the safety of injecting human spinal stem cells (NSI-566) directly into the spinal cord for the treatment of ALS. On October 8, [Dr. Eva Feldman, principle investigator of the Neuralstem trial, said](#) that "this has been a very successful trial so far. We have demonstrated that intraspinal transplantation is feasible and well-tolerated." Now, there is even more exciting news, Dr. Glass just announced that the transplanted stem cells have "long-term survival" in these patients - even up to 30 months after transplantation. In addition to this exciting finding, Dr. Glass announced that the NIH has agreed to fund a majority of the Phase II study of NSI-566 in people with ALS. Neuralstem plans to begin recruiting for the Phase II study once their protocol is approved by the FDA.

#### [Biogen Idec and Isis Pharmaceuticals Form Collaboration Worth \\$630 Million](#)

Isis Pharmaceuticals just [completed a Phase I study of their antisense oligonucleotides \(ASO\) drug \(ISIS SOD1Rx\)](#) in people with ALS. The results of the study were positive - the ASO drug was shown to be well tolerated and safe. [Isis currently has plans to develop additional ALS therapies.](#) Now it seems that they might be reaching out to Biogen Idec as a collaborator in this process. Biogen just signed a huge deal with Isis -- Biogen is paying Isis \$30 million now, with the potential to pay up to \$600 million more, to collaborate on three different research programs. The exact details of the collaboration were not disclosed, but Biogen did mention that the collaboration is around three programs that are targeting neuromuscular and neurological diseases. Hopefully one of these programs is focused on ALS!

#### [Cytokinetics Hosts Research and Development Day](#)

Cytokinetics, Inc. hosted a Research and Development Day on Wednesday, December 12, 2012 at the New York Grand Hyatt Hotel in New York City. If you weren't able to attend in person, you can listen to a recording of the conference by phone. Learn more about how to access the replay of the conference [here](#). Cytokinetics is currently recruiting participants for their Phase IIb study of tirasemtiv in people with ALS. Cytokinetics plans to enroll around 400 people with ALS in the clinical trial.

The ALS Forum was developed by Prize4Life, Inc.  
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