



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:

The ALSGene tool:
www.ALSGene.org

The PRO-ACT Database:
www.ALSDatabase.org

[NEALS Biofluid Repository Available to Researchers](#)

[VABBB Tissue Request Information Site](#)

Funding Opportunities:

[ALSA Call for Research Proposals: TREAT-ALS Drug Development Contract.](#) Letter of Intent due February 27, 2015.

[EU Joint Programme- Neurodegenerative Disease Funding.](#) Pre-proposals due March 10, 2015.

[CDC Grant Opportunity: Analyze and Evaluate Potential Risk Factors for ALS.](#) Applications due March 23, 2015

[California Stem Cell Agency \(CIRM\) 2.0 Awards. Funding for late stage preclinical and clinical stage projects.](#) Due

Research News

[Motor Neuron Excitability Rises then Drops in ALS](#)

Motor neurons in ALS exhibit abnormal physiological properties, with both hyperexcitability and loss of activity reported in the past (see [April 2014 news story](#); [Oct 2013 news story](#)). A new report in the January 12 *Nature Communications* online offers a potential explanation for the conflicting observations. The research team led by Gareth Miles at the University of St. Andrews in Scotland monitored motor neurons generated from skins cells of controls and ALS patients with C9ORF72 or TDP-43 mutations. Over time, the neurons transitioned from a phase of increased firing rate to a slower firing rate, all in advance of signs of cellular distress. The fact that both mutations led to the same phenomenon suggests that they may affect a shared pathway and this may be a more general hallmark of ALS. What are potential implications of these findings for ALS therapies? Click [here](#) to read more.

[Early Defects in ALS Mice Occur in Both Upper and Lower Motor Neurons](#)

Where does ALS first begin? Many researchers support the "dying back" hypothesis, in which the earliest stages of ALS is denervation at the neuromuscular junction (NMJ). Others have found evidence of a "dying forward" mechanism, wherein upper motor neurons in the neocortex are first to become affected (see [Nov 2014 news story](#), [May 2013 news story](#)). Two new studies in the January 14 *Journal of Neuroscience* identify presymptomatic defects in both upper and lower motor neurons (at the NMJ), suggesting that there might be multiple zones of disease initiation. Mark Bellingham, Peter Noakes and colleagues from the University of Queensland in St. Lucia, Australia observed loss of dendritic spines in upper motor neurons as early as 3 weeks of age in an aggressive mutant SOD1 mouse model. In a second paper, Richard Robitaille and colleagues from the Université de Montréal in Quebec, Canada identified early defects in the perisynaptic Schwann cells that impair synaptic function at the NMJ. These exciting

last business day of each month.

Upcoming Meetings:

February 2015

February 9-10, 2015: New York, NY: [17th BIO CEO & Investor Conference](#)

February 17-19, 2015: Boston, MA: [World CNS Summit 2015](#)

February 15-20, 2015: San Francisco, CA: [Molecular Med Tri-Con 2015](#).

February 23-24, 2015: Manchester, UK: [10th Annual Biomarkers Congress](#)

February 25-26, 2015: La Jolla, CA: [Biocom Global Life Sciences Partnering Conference](#)

March 2015

March 1-3, 2015: San Diego, CA: [9th Annual Drug Discovery for Neurodegeneration Conference](#)

March 1-6, 2015: Ventura, CA: [Glial Biology, Functional Interactions Among Glia and Neurons: Glial Cells in Health and Disease](#).

March 1-6, 2015: Ventura, CA: [Oxidative Stress and Disease: The Redox Biology of Age-Related Diseases](#).

March 11-14, 2015: Washington, DC: [MDA Scientific Conference](#).

March 18-20, 2015: Newcastle upon Tyne, UK: [8th Annual MRC Neuromuscular Translational Research Conference](#).

findings about the earliest defects in ALS may ultimately help in earlier diagnosis and treatment of the disease. Click [here](#) to read the full report.

[Criminal Behaviors by People with Frontotemporal Dementia - How to Respond?](#)

Approximately 15% of patients with ALS have frontotemporal dementia (FTD) and as many as 50% exhibit behavioral or cognitive impairments. The discovery of the C9ORF72 mutations, which cause ALS, FTD or a combination of both (ALS-FTD), has further strengthened the link between ALS and FTD as existing along a spectrum of a single disease (see [Dec 2014 conference news](#); [Nov 2014 conference news](#)). A new research study, led by Bruce Miller at the University of California, San Francisco and published in January 5 *JAMA Neurology*, retrospectively examined over 2000 records of neurodegenerative disease patients at the UCSF Memory and Aging Center, including FTD, Alzheimer's disease (AD) and Huntington's disease patients. The researchers found that more than a third of patients with the behavioral variant FTD exhibit criminal behaviors (as compared to 8% of AD patients), and these are often described as the first overt symptoms of the disease. What is the best way for the criminal justice system to address these cases? Click [here](#) to read more.

[New Study Reveals Mechanism of Action of Protein Clump-Dissolving Chaperone](#)

Toxicity associated with misfolded proteins is characteristic of many neurodegenerative diseases, including ALS (See [Nov 2011 news story](#)), and avenues to selectively dissolve protein aggregates or preemptively inhibit their formation are being explored as therapeutic avenues (see [June 2013 conference news](#)). A new study from the laboratory of James Shorter at the University of Pennsylvania in Philadelphia reveals the structural basis for the diverse functions of heat shock protein 104 (Hsp104), a yeast chaperone that dissolves protein aggregates. Shorter and colleagues have been engineering variants of Hsp104 that could potentially be applied toward therapeutic purposes in humans (see [Aug 2014 news story](#)), and the new findings, published January 22 in *Molecular Cell* online, could dramatically expedite progress in that direction. Click [here](#) to read more about the mechanism through which Hsp104 dissolves both disorganized protein aggregates and prions.

Drug News

[Treeway and uniQure Partner to Develop Gene Therapy for ALS](#)

Entrepreneurs and ALS patients Bernard Mueller and Robert Jan Stuit are not only founders of Project MinE, the largest genetic study in ALS (see [Oct 2014 news story](#)), but are also cofounders of an innovative ALS-focused biotechnology company called [Treeway](#). The company now announced a partnership with gene therapy company [uniQure](#) to develop a gene therapy for ALS based on AAV5 viral vectors and delivery of Glial cell derived neurotrophic factor (GDNF, see also [April 2014 news story](#)). Treeway will be responsible for preclinical and clinical development of the therapy, and uniQure will provide manufacturing capabilities and expertise in gene therapy. Keep a close watch for progress updates! Click [here](#) to read more.

March 18-22, 2015: Nice, France: [12th international Conference on Alzheimer's and Parkinson's Diseases.](#)

April 2015

April 7-11, 2015: Soelden, Austria: [The 17th International Neuroscience Winter Conference.](#)

April 7-8, 2015: San Francisco, CA: [The 10th Annual Neurotech Investing and Partnering Conference.](#)

April 18-25, 2015: Washington, DC: [The American Academy of Neurology \(AAN\) Annual Meeting.](#)

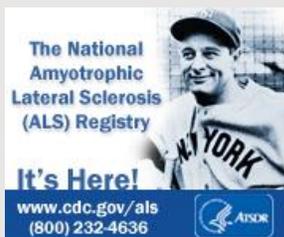
April 21-23, 2015: Boston, MA: [BioIT World Conference & Expo.](#)

April 27-29, 2015: Boston, MA: [Stem Cell Summit '15.](#)

May 2015

May 5-7, 2015: Philadelphia, PA: [Biomarker & Diagnostics World Congress 2015](#)

May 21-23, 2015: Verbania, Italy: [European Network for the Cure for ALS \(ENCALS\) Annual Meeting.](#)



Download your free copy:

[ALS Canada to Fund New Clinical Trial in ALS with Ice Bucket Challenge Donations](#)

The ALS community has been eagerly awaiting updates on the use of Ice Bucket Challenge dollars (see [Sept 2014 news story](#)). The ALS Society of Canada, with matching funds from Brain Canada and the Government of Canada, has now announced its first research grant using the Canadian 'Ice Bucket' funds, which totaled approximately \$16M. The grant will fund a new clinical trial of pimozone in ALS as well as development of a companion biomarker test. Pimozone is already approved in Canada for treating schizophrenia and Tourette's syndrome, and is thought to act by stabilizing the neuromuscular junction. A small clinical trial in Poland has previously reported benefits with pimozone (see [July 2014 news story](#)), and positive effects on disease progression have also been demonstrated in ALS mouse models. More information about the study will be released when patient recruitment begins. Click [here](#) to read more.

[Newron Pharmaceuticals Embarking on Phase II Clinical Trial for VEGF therapy in ALS](#)

Italian clinical-stage biotechnology company [Newron Pharmaceuticals](#) has announced the initiation of a Phase II clinical trial of sNN0029 in ALS. The drug candidate is a recombinant human vascular endothelial growth factor (rhVEGF165) that is administered intracerebroventricularly, and acts by inhibiting cell death in motor neurons (see [June 2013 news story](#)). In mutant SOD1 mouse models of ALS, sNN0029 increased lifespan and slowed disease progression. The drug has already been tested in humans in a Phase I/II safety study and showed preliminary evidence of efficacy. The trial will recruit 18 patients and monitor patients for 3 months. Click [here](#) to find out more about the new ALS clinical trial.

[Genentech and 23andMe Forge Partnership to Explore Parkinson's Disease Genetics](#)

Mountainview, California-based personal genomics company [23andMe](#) and [Genentech](#) have announced a new partnership around Parkinson's disease (PD) genetics. Under the new deal, 23andMe will grant Genentech access to its genomic and phenotypic data from 12,000 PD patients - a rare dataset with potential to shed light on disease mechanisms and risk genes for PD. The companies are also aiming to fully sequence the genome of 3,000 PD patients in addition to collecting clinical data to enable detailed analysis of genetic risk factors for PD. This ambitious project aims to ultimately reveal new therapeutic targets for PD and accelerate drug development. A similar initiative in ALS is also underway under the leadership of [Project MinE](#) (see [Oct 2014 news story](#)) to sequence genomes of more than 15,000 ALS patients! Click [here](#) to read more.

The ALS Forum was developed by [Prize4Life, Inc.](#)

Identified content provided through a partnership with [Alzforum.](#)

 Working with ALS Mice
Click to Download PDF

**Send the ALS Forum e-
Newsletters to your
colleagues!**

 Forward to a Friend

[Forward email](#)



This email was sent to contact@prize4life.org by contact@prize4life.org | [Update Profile/Email Address](#) | Rapid removal with [SafeUnsubscribe™](#) | [Privacy Policy](#).



Prize4Life, Inc. | PO Box 5755 | Berkeley | CA | 94705