



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](#)

[NINDS Fibroblast Repository](#)

[VABBB Tissue Request Information Site](#)

Funding Opportunities:

[Frick Foundation ALS Research Grants](#). Applications due September 30, 2014.

[Blueprint Neurotherapeutics Network \(BPN\): SBIR Small Molecule Drug Discovery and Development for Disorders of the Nervous System \(U44\)](#). Applications due October 21, 2014.

[NCATS Small Business Contract Funding](#). Applications due November 5, 2014.

Upcoming Meetings:

October 2014

October 7-11, 2014: Berlin, Germany: [19th International World Muscle](#)

Research News

[After a Summer of Icy Showers, What Will Happen with Buckets of Cash for ALS?](#)

Over the summer, the Ice Bucket Challenge raised over \$100 million for ALS charities across of the U.S. alone. The social media campaign that went viral globally required challenging three people to donate \$100 to an ALS charity of choice, or to dump a bucket of ice water on one's head and post the video of it on social media (or even better, dunk and donate!). What is the best use for this surge in funding for ALS? ALSTDI has disclosed its plans for using the funds (see [Aug 2014 news story](#)), but what about the remaining cash? Click [here](#) to read Dr. Amber Dance's full story on the Ice Bucket Challenge and experts' opinions on how to best use these funds.

[Ataxin-2 Variants Tip the Balance Toward ALS](#)

Nucleotide repeat expansions in the ataxin-2 gene can either cause spinocerebellar ataxia type 2 or increase the risk of ALS, depending on the number of repeats (see [Aug 2010 news story](#)). A new study published September 9 in *Neurology* by researchers from The French Research Network on frontotemporal dementia (FTD) and FTD-ALS reveals another layer of complexity in how ataxin-2 variants are linked to disease. The investigators examined the ataxin-2 gene in a large cohort of patients with ALS and/or FTD, and found that ataxin-2 repeat expansions are associated with increased risk of developing ALS or ALS-FTD, but not pure FTD. Do ataxin-2 expansions also tip the balance toward ALS symptoms in carriers of C9ORF72 repeat expansions, the most common genetic cause of ALS and FTD? Click [here](#) to find out.

[Method to Identify Modified Protein Structures has Diagnostic Potential for Neurodegenerative Diseases](#)

Researchers from the ETH in Zurich, Switzerland and University of Padua, Italy, have developed a new technique to identify conformational changes in proteins in complex biological samples, such as blood or cerebrospinal fluid. This method could eventually be developed into a diagnostic tool for ALS and other diseases associated with misfolded proteins (see [Dec 2012 news story](#), [Nov 2013 news story](#)). The technique, published September 14 online in *Nature Biotechnology*, integrates proteolysis with proteomic methods co-developed by study

[Society Congress](#)

October 12-14, 2014:
Baltimore, MD: [American Neurological Association's 2014 Annual Meeting](#)

October 22-24, 2014:
Clearwater beach,
FL: [NEALS Annual Meeting](#)

October 23-25, 2014:
Vancouver, Canada: [The 9th International Conference on Frontotemporal Dementias](#)

November 2014

November 7, 2014: Boston,
MA: [10th Annual ALS TDI Leadership Summit](#)

November 13-14, 2014:
Arlington, VA: [24th Neuropharmacology Conference](#)

November 13-14, 2014:
Arlington, VA: [9th Brain Research Conference Neuroprotection: Basic mechanisms and translational potential](#)

November 14, 2014:
Washington, DC: [Advances in ALS and FTD Genetics Workshop](#)

November 15-19, 2014:
Washington, DC: [The Annual Society for Neuroscience Meeting](#)

November 16-18, 2014:
New York, NY: [Partnering for Cures](#)

December 2014

December 3-5, 2014: San
Antonio, TX: [World Stem Cell Summit](#)

December 3-6, 2014: Cold
Spring Harbor,
NY: [Neurodegenerative Diseases: Biology & Therapeutics](#)

senior author Paula Picotti. In the future, this method could be used to develop new biomarkers that measure changes in the structure of critical proteins and the ratio between normal and pathogenic forms as disease progresses. Click [here](#) to read more about this novel technique and its potential applications for neurodegenerative disease.

[New Database Helps Navigate GWAS Data](#)

Genome-wide association studies (GWAS) examine the association between genetic variants, usually in the form of single nucleotide polymorphisms (SNPs), and traits such as disease risk. However, it is often difficult to pinpoint the important disease-linked genes from these data. In the Aug 31 *Nature Neuroscience* online, senior authors Mina Ryten and Michael Weale of King's College London describe a new [database](#) of expression quantitative trait loci (eQTLs) co-developed with collaborators at the U.K. Brain Expression Consortium and North American Brain Expression Consortium. This Brain eQTL Almanac reports eQTLs in 10 different brain regions from healthy brain donors. Although spinal cords data is not yet included, the database has already lead to new insights on genetic risk factors for ALS. Click [here](#) to read about the database and the ALS-associated eQTLs.

Drug and Device News

[Stephen Paul Joins as CEO of Voyager Therapeutics](#)

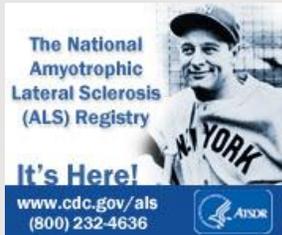
[Voyager Therapeutics](#), the Cambridge, MA-based gene therapy company launched by Third Rock Ventures earlier this year (see [Feb 2014 news story](#)), has appointed Stephen Paul as its new CEO. Interim CEO Mark Levin, a Partner at Third Rock Ventures (TRV), will become Chairman of the Board of Directors. Dr. Paul has a track record of over 35 years in neuroscience research, including CNS drug discovery and development. Prior to his role as Venture Partner at TRV, he spent 17 years at Eli Lilly and Company in key leadership roles. Voyager Therapeutics is developing adeno-associated viral (AAV) approaches to treat disorders of the central nervous system, including familial ALS caused by SOD1 mutations. Click [here](#) to read more.

[Calico and 2M Companies Partner to Tackle Neurodegenerative Diseases](#)

[Calico Life Sciences, LLC](#), the Google-backed biotechnology company launched last year, has entered into a worldwide, exclusive license agreement with 2M Companies to develop P7C3 compounds, originally licensed from University of Texas (UT) Southwestern Medical Center, for the treatment of neurodegenerative diseases. These compounds have previously shown efficacy in mouse models of ALS, Parkinson's disease and depression (see [Oct 2012 news story](#)), and a paper published September 11 by researchers from UT Southwestern in the journal *Cell* reveals a new molecular target of these compounds, a key enzyme for energy metabolism and cell survival called NAMPT. Under the new license agreement between 2M and Calico, Calico will develop and commercialize compounds from the P7C3 program as well as fund research in several laboratories in the Dallas area. This announcement came one week after [Calico and AbbVie announced](#) establishment of a Bay Area research and development center in a deal potentially worth \$1.5B. Click [here](#) to read more.

December 5-6, 2014:
Brussels,
Belgium: [International
Conference on ALS/MND](#)

December 17-20, 2014:
Hotel Vila Galé Coimbra,
Portugal: [Mitochondria,
Metabolism and Disease](#)



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[Kinemed Receives U.S. Patent Allowance for Noscapine for ALS](#)

[Kinemed, Inc.](#) has received a notice of allowance from the U.S. Patent and Trademark Office for their patent covering noscapine for treatment of ALS, PD and diabetic neuropathy. This important step in securing patent protection for a new use of this approved drug is essential for Kinemed in order to de-risk the drug candidate and advance its development for ALS. Research from the company has shown that noscapine normalizes microtubule breakdown and impaired transport of cargo proteins, processes which are linked to several neurodegenerative diseases, including ALS (see [Oct 2012 news story](#); [Aug 2013 news story](#)). In addition, the company has developed a proprietary *in-vivo* technique to track protein transport in both humans and animal models, which holds promise as a diagnostic tool that could also advance the identification and development of other ALS drugs. Click [here](#) to read more.

[Sanofi Co-funding the Canadian Consortium for Research on Neurodegeneration](#)

Sanofi is contributing \$2.5M dollars to the [Canadian Consortium on Neurodegeneration in Aging](#) (CCNA), a new partnership focused on neurodegenerative disease prevention and treatment. The CCNA, which was launched September 10th in Montreal, will bring together 20 research teams to bring new ideas and discoveries to the neurodegenerative space, with a focus on diseases that affect cognition in aging. Sanofi's funding is directed to two key themes of the CCNA - prevention of cognitive impairment and delaying clinical manifestations of dementia. Hopefully this new effort will lead to new insights on the links between frontotemporal dementia and ALS, and the causes of mild to severe cognitive impairments in up to 50% of people with ALS. Click [here](#) to read more.

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