



ALS Forum e-Newsletter Volume 145

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Research News

[CuATSM Brings Disease to a Halt in ALS Mouse Model](#)

A brain-penetrant copper chelator, called CuATSM, extends lifespan of a mouse model of ALS from two weeks to almost two years, according to a paper in the Jan 27 Neurobiology of Disease online. Researchers report that CuATSM delivery to mice co-expressing mutant human SOD1 and its copper chaperone (CCS) results in dramatic improvements in motor function and lifespan. ALS mice expressing human mutant SOD1 without CCS also benefit from the treatment (see [Jun 2014 news](#)). The drug is already slated for Phase I trials in ALS in Australia this year under the sponsorship of ProCypra Therapeutics, a subsidiary of [Collaborative Medicinal Development](#).

[Induced Motor Neurons May Mirror Cellular Aging in ALS](#)

Motor neurons created by direct lineage reprogramming of fibroblasts from FUS-ALS patients model several important aspects of the disease, including neuromuscular junction defects and mislocalization of FUS protein, according to a paper in the Jan 5 Cell Reports online. Unlike induced pluripotent stem cells, induced neurons bypass the embryonic-like state, and thus retain some of the molecular characteristics of the aging cells from which they are derived (see [Jan 2010 news](#)). This study

demonstrates the value of induced motor neurons as a cellular model of ALS in which the effects of aging and ALS-linked mutations can be explored.

[Rho Kinase Inhibitor Proves Beneficial for Tauopathies in Lab Models](#)

Scientists hypothesize that eliminating toxic forms of tau protein could prevent or attenuate neurodegeneration in tauopathies, which include some forms of frontotemporal dementia. In the Jan 27 Journal of Neuroscience, researchers demonstrate that fasudil, a Rho-associated protein kinase inhibitor that is clinically approved in Japan and China, stimulates autophagy and increases tau degradation in cellular models and in fruit flies. Read the discussion around this intriguing therapeutic [here](#).

[ALS Broadly Impacts Cognition and Behavior](#)

A multi-center study with over 250 patients has confirmed that many ALS patients also suffer from cognitive and behavioral impairments, according to a report in the Jan 27 Neurology online. Using the two-part ALS Cognitive Behavioral Screen (ALS-CBS), researchers found significant cognitive and/or behavioral deficiencies in many of patients, sometimes severe enough to merit a diagnosis of frontotemporal dementia. Some behavioral impairments correlated with other clinical features, such as pseudobulbar affect, decreased respiratory function, and lower ALS-FRS scores. The researchers hypothesize that disease 'spreading' between degenerating motor areas and adjacent brain regions could determine which cognitive or behavioral deficits accompany the motor impairments.

[Biogen Trial Aims to Improve Identification of Most Promising ALS Drug Candidates](#)

The ALS Functional Rating Scale-Revised (ALSFRS-R) is the most widely-used outcome measure in ALS clinical trials, but studies have shown that its utility for predicting disease progression in shorter, Phase II trials is limited. A new study sponsored by the biotechnology company [Biogen](#) aims to overcome this challenge, by comparing more than a dozen ALS outcome measures, including electrophysiological, neuroimaging, and respiratory measures. Ultimately, the study goal is to improve the design of Phase II drug trials to expedite identification of drugs most likely to succeed in Phase III and to improve patient outcomes.

Assistive Technology

[Add-On Technology Allows Electric Wheelchairs to be Controlled by Thoughts](#)

Researchers at California State University, Fullerton, have developed an affordable 'mind-controlled' electric wheelchair by combining a mobile application with a specialized headset that detects brain waves. The system, which is controlled by specific thoughts and facial expressions, can be easily retrofitted to existing electric wheelchairs, and will be priced under \$150, making it more affordable for those in need. Further development is ongoing to improve the system before making it more widely available.

Deals and Partnerships

[Biogen and Target ALS Launch \\$20M Corporate Funding Challenge](#)

The Target ALS Foundation has received a \$5M donation from [Biogen](#) to fund preclinical ALS research across the Foundation's network of academic laboratories. Target ALS-funded studies focus on identification and validation of new therapeutic targets with a view toward fueling drug development programs in biopharmaceutical companies. With this donation, Biogen has seeded Target ALS' *Industry Fund for ALS Research*, and launched a challenge to raise a total of \$20M, of which 50% of contributions received through 2018 will be matched by Biogen.

[Yumanity Secures \\$45M to Identify Drugs for Protein Misfolding-Diseases](#)

Startup company [Yumanity Therapeutics](#) has secured a \$45M Series A financing to identify drugs for neurodegenerative diseases caused by protein misfolding, including ALS, Alzheimer's and Parkinson's disease. [Biogen](#) and [Sanofi-Genzyme BioVentures](#) are among the new investors in the company, which was launched in 2014 by former Onyx CEO Tony Coles and protein folding leader Susan Lindquist (see [Dec 2014 news](#)). The company's discovery platform was developed in Lindquist's laboratory and uses yeast cells to identify drugs that prevent protein misfolding and aggregation.

Funding Opportunities:

[Department of Defense ALS Research Program \(ALSRP\) Funding Opportunities](#). Pre-announcement.

[California Institute of Regeneration Medicine \(CIRM\) Quest 2.0 Awards](#). Applications due March 15, 2016.

[NEW! ALSA, ALS Finding a Cure Grand Challenge: Generation of a PET Tracer for TDP43 Aggregates](#). Letter of Intent due March 22, 2016.

[NIH Therapeutics for Rare and Neglected Diseases \(TRND\) Program](#). Now accepting rolling applications.

[All funding opportunities >>](#)

Upcoming Meetings:

February 2016

Feb 25-26, 2016: Manchester, UK: [11th Annual Biomarkers Congress](#).

March 2016

March 6-8, 2016: Miami, FL: [Annual Drug Discovery for Neurodegeneration Conference](#).

March 20-23, 2016: Arlington, VA: [MDA Clinical Conference](#).

New! March 22-23, 2016: Oxford, UK: [UK Neuromuscular Translational Research Conference](#).

[Full list of upcoming meetings>>](#)

Resources:

[ALS Drugs in Development Database](#)

[ALSGene](#)

[The PRO-ACT Database](#)

[NEALS Biofluid Repository Available to Researchers](#)

[VABBB ALS CNS Tissue Request Information Site](#)

[TargetALS Cores](#)

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