



Visit the [ALS Forum website](http://www.alsforum.org) 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:

Visit the new ALSGene tool at [www.ALSGene.org](http://www.ALSGene.org)

Visit the PRO-ACT Database at [www.ALSDatabase.org](http://www.ALSDatabase.org)

[NEALS Biofluid Repository Available to Researchers](#)

[NINDS Fibroblast Repository](#)

[VABBB Tissue Request Information Site](#)

#### Funding Opportunities:

[Horizon 2020: Funding opportunity : New therapies for rare diseases](#)

[Massachusetts Neuroscience Consortium Round 2 Funding](#)

#### Upcoming Meetings:

April 26 - May 3, 2014:  
Philadelphia, PA: [American Academy of Neurology 2014 Annual Meeting](#)

April 28-29, 2014: London,

## Research News

### [Epilepsy Drug Reduces Hyper-excitability in ALS Patient-Derived Motor Neurons](#)

Back in 2008, Harvard Stem Cell Institute Principal Investigator Kevin Eggan suggested that ALS patient-derived induced pluripotent stem cells (iPSCs) may be leveraged to identify novel targets and screen drugs for ALS. Two new studies led by Kevin Eggan and Clifford Woolf's groups at the Harvard Stem Cell Institute and published on April 3 in the online edition of *Cell Stem Cells* and *Cell Reports*, build on these earlier findings, and provide an intriguing example of the therapeutic promise of these cells. The investigators found that motor neurons carrying independent ALS mutations exhibit an increase in random firing activity, likely due to a deficit in potassium channels (see related story from [December 2007](#)), and that these overactive neurons generate misfolded proteins, which further increase neuronal firing. They hypothesized that by opening potassium channels in these cells they could reduce the hyperexcitability, and they found exactly that - retigabine, an FDA approved medication for epilepsy, normalized the activity. The team is now collaborating with Massachusetts General Hospital to begin initial safety testing of the treatment in ALS patients. Click [here](#) to read more.

### [Optogenetics in Motor Neurons Sends a "Wake-Up Call" to Paralyzed Muscles](#)

One of the key challenges with stem cell transplantation approaches aimed at repairing damaged motor neurons in ALS has been their functional integration into the neuromuscular circuitry. Recent work from Linda Greensmith's group of University College London's Institute of Neurology, UK and Ivo Lieberam's lab of the MRC Centre for Developmental Neurology, King's College, London, published on April 4 in *Science*, has demonstrated a successful approach to overcoming this hurdle by combining stem cell therapy with optogenetics. Murine embryonic stem-cell derived motor neurons were engineered to carry the light-sensitive ion channel channelrhodopsin-2, which allows for fine-tuning of neuronal firing using different intensities, duration and frequency of light pulses. The transplanted motor neurons not only reinnervated the hindlimb muscles following sciatic nerve injury, but also restored their function. The group is now laying the groundwork for

UK: [Astrocytes in Health and Neurodegenerative Disease](#)

April 29 - May 1, 2014: Boston, MA: [BioIT World Conference and Expo](#)

April 29-30, 2014: Boston, MA: [Translational CNS summit](#)

May 7-8, 2014: San Francisco, CA: [Neurogaming Conference](#)

May 8-10, 2014: Berlin, Germany: [The 7th European Conference on Rare Diseases & Orphan Products \(ECRD\)](#)

May 8-11, 2014: Berlin, Germany: [The 8th World Congress of Controversies in Neurology](#)

May 9, 2014: New York, NY: [The Biology of Aging: Novel Drug Targets for Neurodegenerative Diseases](#)

May 12-17, 2014: Stockholm, Sweden: [Keystone Symposia: Adult Neurogenesis](#)

May 21-23, 2014: Boston, MA: [The 13th Annual World Pharma Congress: Tackling Translational Challenges](#)

May 22-24, 2014: Leuven, Belgium: [European Network for Cure of ALS \(ENCALS\) Meeting](#)

June 14-15, 2014: New London, NH: [Gordon Research Seminar: Barriers of the CNS, The Neurovascular Unit: Partners for Life](#)

June 15-20, 2014: New London, NH: Gordon Research

human trials with their technique in the next five years. Click [here](#) to read more.

### [A Two-in-One Deal for Stem Cell Growth](#)

A new hydrogel material may revolutionize the process of large scale stem cell production. Kevin Shakesheff's group and colleagues from the University of Nottingham, UK have created a sophisticated microenvironment for stem cells that enables them to undergo both proliferation and differentiation in the same material. These findings, published online on March 27 in the *Proceedings of the National Academy of Sciences*, are based on an elegant chemical switch, ionic de-cross-linkage that shifts the alginate-rich environment needed for stem cell self-renewal to a collagen-rich environment needed for differentiation. This technology holds promise for large scale manufacturing of human pluripotent stem cells for therapeutic purposes in regenerative medicine. Click [here](#) to read more about this exciting new development.

### [Astrocytes in Huntington's disease](#)

Increasing evidence is pointing to a central role for astrocytes in ALS disease pathology (see stories from [October 2011](#) and [February 2014](#)). A new study led by Michael Sofroniew and Baljit Khakh at the University of California, Los Angeles and published on March 30<sup>th</sup> in *Nature Neuroscience*, implicates astrocytes yet again, this time in the context of Huntington's disease (HD). The investigators examined the role of striatal astrocytes in two distinct mouse models of HD and found that in both, astrocytes expressing mutant huntingtin lack a specific potassium channel, Kir4.1, which is crucial for cleaning up extracellular potassium. The resulting increase in extracellular potassium leads to hyperexcitability of striatal neurons, and increasing Kir4.1 expression in a mouse model of HD partially alleviates the motor deficits characteristic of the disease. Interestingly, a role for impaired potassium channel function in ALS has also been recently described (see this newsletter). Click [here](#) to read more.

## Drug News

### [Genentech and PatientsLikeMe enter Broad Partnership Surrounding Patient Database](#)

PatientsLikeMe is an online patients-centered online network with a database of approximately 250,000 members, who share information about disease progression, treatment options and outcomes, as well as quality of care. ALS is one of the largest patient groups in the database, but over 2000 diseases are represented. This extensive patient data is a potential goldmine for drug companies seeking to collect medical evidence from patients and to learn about their unmet needs.

Genentech, which was acquired in 2009 by the Swiss drug company Roche, has entered a five-year partnership with PatientsLikeMe that allows them broad access to the full database of de-identified data for the next five years. Click [here](#) to read more about the partnership between Genentech and PatientsLikeMe.

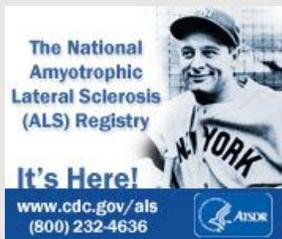
### [Daiichi Sankyo and UCSF to Collaborate over Neurodegenerative Drug Discovery and Development](#)

Conference: [Barriers of the CNS. Expanding the Understanding of CNS Barriers in Health and Disease](#)

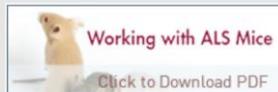
June 22-27, 2014: Waterville Valley, NH: [Cell Biology of the Neuron: Mechanistic Insight into Neuronal Development, Plasticity, Disease and Regeneration](#)

June 28-29, 2014: Hong Kong, China: [Gordon Research Seminar: Molecular & Cellular Neurobiology, Exploring the Frontiers of Foundational and Translational Neuroscience](#)

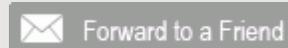
June 29 - July 4, 2014: Hong Kong, China: [Gordon Research Conference: Molecular & Cellular Neurobiology, Mechanisms of Neural Development, Circuit Assembly, Synaptic Plasticity and Neuropsychiatric Disorders](#)



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Tokyo, Japan, based Daiichi Sankyo has entered into a drug-discovery partnership with University of California, San Francisco (UCSF) to develop new therapies for neurodegenerative diseases. The partnership aims to leverage Daiichi's drug development expertise and UCSF's world-renown neurobiologists and focuses on drug discovery and development of diagnostics for neurodegenerative diseases, including Parkinson's disease, Creutzfeldt-Jakob disease and Alzheimer's disease. Daiichi Sankyo brings their compound library to the table, allowing for high-throughput drug screening using technology developed at UCSF. Daiichi will provide research funding as well as royalties and milestone payments for commercially successful products developed under the collaboration. Click [here](#) to read more.

#### [UK Speeds Up Access to Medicine](#)

The UK Government's Department of Health has recently announced an Early Access to Medicine scheme with the goal of accelerating drug accessibility to patients with limited therapeutic options. The Medicines and Healthcare Products Regulatory Agency (MHRA) will oversee the scheme, which will require that the drugs be demonstrated as safe and beneficial to patients but not that the drug be fully approved. The Early Access to Medicine scheme is a two-step process, in which, if the drug is shown to be safe and effective in humans, the MHRA can issue a "promising innovation medicine" designation. This will then be followed by a full scientific review of the existing clinical data to assess the risks and benefits of the drug. Hopefully, this program will also be beneficial to ALS patients in the UK seeking access to novel drugs. Click [here](#) to read more.

#### [Cedars-Sinai Lands \\$2.5M Grant for a Gene-Therapy-Based Preclinical Trial](#)

A multi-party ALS-research collaboration between Cedars-Sinai Regenerative Medicine Institute, the University of Wisconsin, Madison and Netherlands-based biotechnology company uniQure is now backed by a \$2.5M grant from the Department of Defense (DOD). The funding will support preclinical studies that could be the basis for a gene-therapy based clinical trial in ALS. Previous work from Masatoshi Suzuki's laboratory at University of Wisconsin, Madison (see story from [June 2013](#)) has shown that intramuscular injection of stem cells engineered to produced glial cell-line derived neurotrophic factor (GDNF) into a rat model of ALS prolongs survival and attenuates loss of motor function. These studies, to be conducted in Clive Svensen's laboratory at Cedars-Sinai and Masatoshi Suzuki's laboratory will use AAV5 viral vectors to deliver GDNF into muscles of the leg and diaphragm in order to target two major muscle groups affected in ALS. The DOD funding will support the preclinical studies from animal studies through the filing of an Investigational New Drug application with the Food and Drug Administration. Click [here](#) to read more.

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