DISCOVERING

THE ALS FORUM

A CURE

ALS Forum e-Newsletter Volume 84 **April 19, 2013**

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:

Visit the PRO-ACT Database at www.ALSDatabase.org

NEALS Biofluid Repository Available to Researchers

NINDS Fibroblast Repository

Funding News:

The MNDA just opened their Online Summary Application Form for PhD studentship grants. The deadline is Friday 3 May 2013.

Abstract Registration Deadline:

Don't Miss It! The Society for Neuroscience 2013 Annual Meeting Abstract submission opened yesterday, April 18. Don't delay, the deadline is May 9, 2013!

The RNA Metabolism in Neurological Disease Meeting Abstract deadline is May 31, 2013.

Upcoming Meetings:

April 21-24, 2013: Washington, DC: MDA Scientific Conference: Therapy Development for Neuromuscular Diseases: Translating Hope Into Promise

May 1-2, 2013: Bethesda, MD: NIH workshop on Alzheimer's Disease-

Research News

Exciting Breaking News! Prize4Life and the Neurological Clinical Research Institute at Massachusetts General Hospital were just awarded the 2013 Best Practices Award in the Clinical & Health IT category from Bio-IT World for the PRO-ACT Database (http://www.alsdatabase.org/). The Bio-IT World Best Practices Awards program "recognizes organizations for their outstanding innovations and excellence in the use of technologies and novel business strategies that will advance biomedical and translational research, drug development, and/or clinical trials." Prize4Life is thrilled that the PRO-ACT database has been recognized as an exemplar of the kind of open source collaborative data sharing initiatives that are necessary to make serious advances in treating complex diseases. We hope that this award will attract even greater attention to PRO-ACT and the possibilities the database represents to advance ALS clinical research. Read more about this exciting award here and help us spread the word about PRO-ACT to your quantitative colleagues!

Oligodendrocyte Support System Fails Early in ALS

In a recent study published in *Nature Neuroscience* and led by Dr. Dwight E. Bergles, professor of neuroscience at Johns Hopkins University School of Medicine, researchers found that oligodendrocytes may contribute to ALS onset and progression. The researchers found that gray matter oligodendrocytes die before disease symptoms even appear in SOD1 (G93A) mice. Although it was previously shown that oligodendrocyte progenitors proliferate at a higher rate in these ALS mice, in the new study the authors report that these progenitors did not go on to mature into functional oligodendrocytes. Selectively removing the mutant SOD1 gene from oligodendrocytes in these mice delayed disease onset by an astonishing four months and even prolonged survival, despite the fact that the motor neurons in these mice still harbored the mutant SOD1 gene. Click here to find out how these findings translate to people with ALS.

Blocking Axon Destruction Delays Death in ALS Mice

Last summer we covered intriguing results out of the laboratory of Dr. Marc Freeman, professor of neurobiology at the University of Massachusetts Medical School, which showed that Wallerian degeneration is an active axonal death process driven by dSarm in

Related Dementias: Research Challenges and Opportunities

May 3-5, 2013: San Francisco, CA: Targeted Drug Delivery for Pain and Neurologic Disease

May 6, 2013: New York, NY: The New York Academy of Sciences, Translating Natural Products into Drugs for Alzheimer's and Neurodegenerative Disease

May 6-8, 2013: Philadelphia, PA: 9th Annual Biomarker World Congress

May 18-24, 2013: Les Diablerets, Switzerland: Gordon Research Conference: Dendrites: Molecules, Structure & Function

May 19-22, 2013: Boston, MA: Society for Clinical Trials 34th Annual Meeting

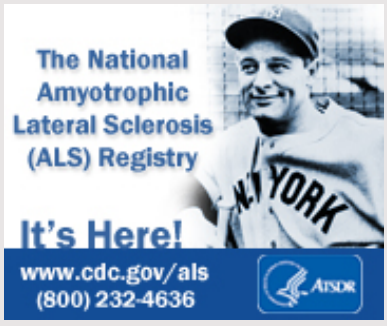
May 21-23, 2013: Boston, MA: Translational CNS Summit

May 23-24, 2013: San Francisco, CA: 8th Annual The Neurotech Investing & Partnering Conference, Advances in Drugs, Devices & Diagnostics for the Brain and Nervous System

May 23-24, 2013: Uppsala, Sweden: Neurodegenerative Disorders: Immunotherapy and Biomarkers

May 31, 2013 - June 2, 2012: Sheffield, United Kingdom: 2013 ENCALS (European Networkd for the Cure of ALS) Meeting

June 10-11, 2013: Cambridge, MA: New Avenues for Brain Repair: Programming and Reprogramming the Central Nervous System



Drosophila and Sarm1 in mice. Now, Dr. Freeman and his team have made another exciting advance that may one day impact people with ALS. The researchers discovered that ALS mice harboring a mutation in the Sarm1 gene survived 10 days longer than ALS mice with a functional Sarm1 and showed a significant increase in motor neuron survival. Click here to read the full story.

Conference News

Drug Data Dash at the 2013 AAN Annual Meeting
The presenters in the "Emerging Science Platform Session" at the 65th Annual Meeting of the American Academy of Neurology (AAN) held March 16-23, 2013 made sure to jam-pack as much data as they could into their three minute time slots. In fact, one conference attendee likened the three-minute talks to "the neurological equivalent of speed-dating." If you happened to miss the updates, either because you weren't attending the conference or because the presenters were sprinting through their talks, Prize4Life funded science writer Dr. Amber Dance has you covered! Click here to read the latest updates from Cytokinetics Inc., BrainStorm Cell Therapeutics Inc., and Neuralstem Inc., as well as learn about a potential new biomarker that could be used to classify two different FLTD subtypes.

Drug News

Neuralstem Receives FDA Approval for Phase II Study
Neuralstem Inc. just announced that the FDA has approved their Phase II study to inject human spinal cord-derived stem cells (NSI-566) directly into the spinal cord for the treatment of ALS. The goal of the Phase II study is to determine the maximum tolerated dose of NSI-566 in people with ALS. Neuralstem Chairman and Chief Scientific Officer Dr. Karl Johe said "As a result of the excellent safety and tolerability demonstrated in Phase I, we will be able to proceed more aggressively in Phase II. In Phase I, we started with just five injection sites per patient, and advanced to a maximum of 15 injections of 100,000 cells each. In Phase II, we will advance up to a maximum of 40 injections, and 400,000 cells per injection based on safety." Pending Institutional Review Board approval, the Phase II trial will be conducted at two locations: Emory University Hospital in Atlanta, Georgia, the site of the Phase I trial, and the ALS Clinic at the University of Michigan Health System in Ann Arbor, Michigan. To learn more about the details of this Phase II dose escalation study click here.

Knopp Wants Biogen Idec to Return Data From Failed Dex Trial
Knopp Neurosciences Inc. licensed Dexpramipexole (Dex) to Biogen Idec in 2010 after the compound (formerly known as KNS-760704) showed promising Phase II clinical results in people with ALS. In 2011, Biogen and Knopp began enrolling ALS patients in the Phase III Dex trial, which was intended to evaluate the safety, efficacy, and pharmacokinetics of Dex. This past January, Biogen Idec announced that their Phase III clinical trial of Dex in over 900 people with ALS failed to meet its primary and secondary endpoints for function and survival, and Biogen decided to discontinue the development of the drug. However, Knopp isn't ready to shelve Dex. Knopp cofounder Tom Petzinger said "We want the opportunity to continue developing this drug for ALS." In order to effectively move forward with developing Dex, Knopp wants the blood samples from the patients that took part in the trial as well as the regulatory data from the trial. Although Biogen argues

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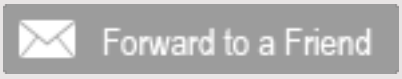

that they "don't have an obligation under the licensing deal to transfer that data," Knopp is leaving this decision up to the US District Court in Boston. Stay tuned to the ALS e-Newsletter to find out what the courts decide.

BrainStorm Cell Therapeutics Announces Dana-Farber Will Produce Stem Cells for US-based Phase II Trial

BrainStorm Cell Therapeutics Inc. is preparing to conduct a Phase II trial to test the safety and tolerability of, as well as collect preliminary efficacy data on, their NurOwn™ stem cell technology for the treatment of ALS in the United States (US) later in 2013. Although BrainStorm is still awaiting Food and Drug Administration (FDA) approval for the trial, they have already secured three clinical trial sites in the US including the University of Massachusetts (UMass), Massachusetts General Hospital (MGH), and the Mayo Clinic. On April 8, BrainStorm announced that they are working with The Connell and O'Reilly Cell Manipulation Core Facility at Dana-Farber Cancer Institute to provide "cGMP-compliant clean room facilities for production of BrainStorm's NurOwn™ stem cell candidate." Under this agreement, Dana-Farber will provide NurOwn to the UMass and MGH Phase II clinical trial sites.

Rilutek Sold To Switzerland-based Covis Pharma Sarl

Sanofi-aventis recently sold Rilutek® (riluzole) to the Switzerland-based pharmaceutical company Covis Pharma Sarl. Riluzole was one of five drugs that Sanofi sold to Covis Pharma for the bargain price tag of $114.6 million. Riluzole is the only FDA-approved drug available for people with ALS. The CEO of Covis Pharma, Jack Davis, ensures that the sale will not compromise the quality of these drugs. Davis said "Covis will continue to supply consistent, top quality, branded pharmaceuticals, ensuring that patients receive the therapeutic care they need." Riluzole will be distributed through Covis Pharma's US-based distribution site which is located in Cary, North Carolina.

Correction:

In response to questions from our readers, we would like to clarify any misconceptions around the previously published ALS Forum e-Newsletter story "New Therapeutic Opportunities for Central Nervous System (CNS) Diseases." Isis Pharmaceuticals remains fully invested in working with Dr. Timothy Miller of the Washington University School of Medicine and Dr. Merit Cudkowicz of Massachusetts General Hospital to develop an SOD1-based antisense therapeutic, including improving the antisense oligonucleotide used for future studies.

The ALS Forum was developed by Prize4Life, Inc.
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