DISCOVERING A CURE

THE ALS FORUM

ALS Forum e-Newsletter Volume 86 **May 17, 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 atwww.ALSDatabase.org

NEALS Biofluid Repository Available to Researchers

NINDS Fibroblast Repository

Funding News:

The ALS Association and the MGH Neurological Clinical Research Institute announce a request for proposals: Phase II Clinical development of novel, high-potential treatments for people with ALS. Letter of intent due May 20, 2013.

ALS Research Program Announcements Released: including Therapeutic Development Award and Therapeutic Idea Award.Deadline for pre-application is June 5.

Abstract Registration Deadline:

The RNA Metabolism in Neurological Disease Meeting Abstract deadline

Research News

New Mouse Line for Studying Upper Motor Neurons Leads to Two Unexpected Discoveries in SOD1 Mice

As reported online on May 1, 2013 in the *Journal of Neuroscience*, researchers at the Northwestern University Feinberg School of Medicine in Chicago, Illinois showed that they could selectively fluorescently label upper motor neurons located in the cortex of both wild type and SOD1 G93A mice. The researchers engineered these mice to express green fluorescent protein under the control of the ubiquitin carboxy-terminal hydrolase L1 (UCHL1) promoter, as the UCHL1 de-ubiquitinating enzyme is stably expressed in corticospinal motor neurons (CSMNs). These mice are an important new tool that will allow researchers to selectively study CSMNs as well as a small population of neurons in the spinal cord. Clickhere to learn about how the authors used these mice to make two unexpected and important discoveries, one of which revealed surprising new insights about the progression of ALS.

ALS in Han Chinese: Different Race, Different Genetics?

A recent study out of First Affiliated Hospital of Anhui Medical University in Hefei, China and the Peking University Third Hospital in Beijing, China published online in *Nature Genetics* on April 28 suggests that the molecular underpinnings of ALS could differ based on race. The researchers identified 90 single nucleotide polymorphisms (SNPs) from a genome-wide association study (GWAS) in over 2,365 individuals of Han Chinese ancestry, 506 of whom were diagnosed with sporadic ALS. In a second cohort of 2,483 people of Han Chinese ancestry, where 706 were diagnosed with ALS, the researchers found that two of these SNPs showed strong association with genetic susceptibility for ALS. Surprisingly, neither of these two SNPs overlapped with the six ALS-related SNPs that had been previously identified in European GWAS, suggesting that there may be different ALS risk factors for those of European or Han Chinese ancestry. Click here to learn more about these two newly identified SNPs, and how these SNPs could be working at the molecular level to increase ALS disease risk.

is May 31, 2013.

Upcoming Webinar:

The ALS Association and NEALS PALS Webinar:Selection Trial of High Dosage Creatine and Two Dosages of Tamoxifen in ALS. The webinar is scheduled for May 20 at 3pm EST.

Upcoming Meetings:

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 Network for the Cure of ALS) Meeting

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

June 12-15, 2013: Boston, MA: International Society for Stem Cell Research

June 16-21, 2013: Les Diablerets, Switzerland:Inhibition in the CNS

Criminal Minds: Could Lack of Empathy in Prison Psychopaths Inform Studies of FTD?

A recent finding published online in *JAMA Psychiatry* on April 24 suggests that researchers working to understand frontotemporal dementia (FTD) may have an untapped and surprising resource, psychopaths. Psychopaths show little evidence of empathy, and now researchers led by Kent Kiehl from the University of New Mexico in Albuquerque have an idea why. The group used functional magnetic resonance imaging (MRI) to assess blood flow in different areas of the brain in prison inmates. They found that those who scored high in measures of psychopathy had reduced activation in the orbital frontal circuit. Given our increasing appreciation of the overlap between FTD and ALS, particularly given anexciting new finding suggesting a role for the FTD gene progranulin in ALS, these findings implicating the orbital frontal circuit may provide ALS researchers with another important brain region on which to focus attention.

Paper Alert: Antisense Oligonucleotide Therapy Safe for ALS

Click here to read Dr. Amber Dance's in depth coverage of a story that was published online in *Lancet Neurology* in April showing that the Phase I trial, testing the safety and tolerability of injecting an antisense oligonucleotide targeted against SOD1 into the cerebrospinal fluid of people with ALS, was safe. Read more about the results of the trial and find out antisense developer Isis Pharmaceuticals' future plans for drug development and clinical trials here.

Drug News

PRO-ACT Wants You!

This past December, Prize4Life and the Neurological Clinical Research Institute (NCRI) at Massachusetts General Hospital launched the PRO-ACT database (www.alsdatabase.org), which consists of over 8,500 ALS patient records from 17 completed Phase II and Phase III clinical trials conducted by companies including Sanofi, Novartis, Teva, and Regeneron. In April, Prize4Life and NCRI were awarded the 2013 Best Practices Award in the Clinical & Health IT category from Bio-IT World for PRO-ACT. Click here to read about how merging multiple datasets from different clinical trials into PRO-ACT can be used to identify ways to "streamline clinical trials." Also read about Prize4Life's and NCRI's plans to expand PRO-ACT, which includes potentially adding the data from recent Phase III ALS clinical trials. With PRO-ACT continually expanding, there are many outstanding research questions that can now be addressed. Click here to learn about how you can use PRO-ACT to help advance ALS clinical research, and if you have an ALS clinical trial dataset that has not yet been included, PRO-ACT needs your data!

ALS-ETF Partners with Denovo Biomarkers to Identify Patients Who Respond to ALS Drugs

The ALS Emergency Treatment Fund (ALS-ETF) helps provide post-Phase II ALS therapies to ALS patients, who might not qualify for clinical trials, through the government's expanded access programs (EAPs). ALS-ETF just announced that they are teaming up with Denovo Biomarkers to see if Denovo can use their proprietary

June 23-27, 2013: Boston, MA: DIA 49th Annual Meeting

June 29-30, 2013:Philadelphia, PA: The ALS Clinical Research Learning Institute

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genomic biomarker discovery technology to identify ALS patient subgroups that respond to particular ALS drugs.Through the partnership, ALS-ETF will provide biological samples collected from individuals participating in the EAPs to Denovo for the analysis. Being able to identify unique patient-responder groups could transform the current clinical trial process, which is one reason why Prize4Life and NCRI built the PRO-ACT database, to enable a retrospective analysis of potential responders from previous trials. Maybe the data in PRO-ACT may also be a useful resource to Denovo!

Critique of Precision StemCell's Stem Cell Therapy for ALS

Precision StemCell is an "outpatient imaging and image-guided treatment facility" established in 2012 by radiologist Dr. Jason Williams in Gulf Shores, Alabama. Since opening his doors, Dr. Williams has treated 24 patients with his reprogrammed stem cells, including 18 people with ALS. However, Dr. Williams' stem cell therapy and medical approach isn't approved by the FDA, nor has it ever been tested in any preclinical animal models, including any animal model of ALS. In February 2013, two individuals from ALS Worldwide visited Dr. Williams' clinic and subsequently published an astonishing report providing detailed information about the unsanitary facility conditions and the highly questionable therapy and procedure, which could significantly harm patients. Click here to read ALS Worldwide's report and click here to read this informative blog post which urges the FDA to take action against Dr. Williams.

NeuroPhage Raises $6.4 Million to Support Pre-IND Development Efforts

NeuroPhage Pharmaceuticals just secured $6.4 million to support the pre-IND development of their lead drug candidate, NPT002, as well as to support the continued development of their "second-generation fusion protein" drug candidates. The funds were primarily contributed by Mérieux Développement, with additional contributions from Shire LLC and other undisclosed investors. Dr. Valérie Calenda, Partner at Mérieux Développement, said "We believe the company's transformative approach to addressing protein misfolding could make a major contribution to achieving greatly improved treatments for these disabling conditions that affect millions of people worldwide." NeuroPhage has previously reported positive preclinical results in animal models of Alzheimer's disease and Parksinson's disease and their drug's mechanism of action seems to hold great promise for ALS as well.

The ALS Forum was developed by Prize4Life, Inc.
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Identified content provided through a partnership with the Alzheimer Research Forum.