ALS Forum e-Newsletter Volume 83 **April 5, 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.

Webinars:

The ALS Association & NEALS Spring 2013 ALS Clinical Trial Pipeline Webinar has now been archived for viewing. To watch the webinar please go here.

Upcoming Journal Club:

April 9, 2013, 6:30-8 pm: Harvard NeuroDiscovery Center Student/Faculty Journal Club paper discussion: Unconventional Translation of C9ORF72 GGGGCC Expansion Generates Insoluble Polypeptides Specific to c9FTD/ALS, Neuron, 2013

Research News

No More Mouse Models of Disease? Not So Fast!

In February, the *New York Times* featured a story covering the findings of a recent and somewhat controversial *Proceedings of the National Academy of Sciences* (*PNAS*) study that suggested that some mouse models of disease may not recapitulate the actual human disease. Three physician scientists, all from top notch academic institutions and organizations, were disappointed by the *New York Times* article, and composed a riveting response. Their take home message? That "experimental research using mice is critical to building knowledge and to translating this knowledge into medical practice in a manner that minimizes risk to human beings." The scientists' response echoes and supports Prize4Life's position on using mouse models for ALS research -- there is extreme value in well-designed preclinical studies (if you are wondering what are some of the factors to consider when designing ALS preclinical studies, check out our "Working with ALS Mice" manual). This response is a must read for anyone interested in the controversy perennially swirling around mouse models of ALS. Click here to read it now.

C9ORF72 May Cause Toxicity By Sequestering Important RNA Binding Proteins

Just weeks ago we brought you two exciting new stories about C9ORF72 (if you missed them, you should check out Dr. Amber Dance's summaries of the *Science* and *Neuron* stories), both of which suggested that surprisingly the hexanucleotide repeat expansion in C9ORF72 is translated into dipeptide repeat (DPR) proteins. Although these studies provided some exciting food for thought about how the hexanucleotide repeats in C9ORF72 could be causing toxicity, the jury is still undecided about C9ORF72's exact mechanism of toxicity. Now, in yet another twist, new research out of the Emory University School of Medicine suggests that that the RNA transcribed from the hexanucleotide repeat in C9ORF72 gene might itself be toxic. How can RNA be toxic? The researchers found that the hexanucleotide repeat RNA acts as an "RNA sponge" to sequester important RNA binding proteins, resulting in neurodegeneration. Interested in finding out which RNA binding protein the researchers believe is the toxic culprit? Click here.

Cellular Chaperones Potentially Important for Preventing Neuronal

Vol 77 Issue 4

Upcoming Meetings:

April 8-9: Washington, DC: Accelerating Therapeutic Development for Nervous System Disorders towards First-in-Human Trials: A Workshop

April 9-11, 2013: Washington, DC: 3rd Annual World Orphan Drug Congress

April 14-19, 2013: Les Diablerets, Switzerland: Oxidative Stress & Disease, Program: The Metabolic-Inflammatory Axis in Brain Aging and Alzheimer's Disease

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-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:

Toxicity in ALS

In a recent study published in *PNAS*, researchers from the University of Illinois at Chicago, Yale University, and Harvard University showed that chaperones can prevent the neuronal death associated with dysregulated axonal transport in ALS. Using nerves isolated from giant squid, the researchers were able to show that to some extent Hsc70 - and to an even greater extent Hsp110 (in substoichiometric amounts) - could rescue axonal transport defects caused by aggregates of mutant SOD1. Click here to read more.

Conference News

Prize4Life's Coverage of the 7th Annual Drug Discovery for Neurodegeneration Conference

Prize4Life's Program Officer, Dr. Jessica Goodman, attended the Alzheimer's Drug Discovery Foundation's 7th Annual Drug Discovery for Neurodegeneration Conference held in San Francisco, California from February 10-12, 2013. To read Jessica's blog coverage of this thought-provoking conference exploring many of the challenges currently facing ALS drug discovery, including: 1) the evolving (and controversial) use of animal disease models in translational research, 2) the rising importance of biomarkers, and 3) the inherent difficulties in targeting protein-protein interactions (particularly in the brain), click here.

ALS Research Awards Presented at the 2013 American Academy of Neurology Conference

A few weeks ago at the 65th Annual Meeting of the American Academy of Neurology held in San Diego, California, the ALS Association and the American Academy of Neurology announced the recipients of two ALS research awards. James Berry, M.D., M.P.H., from the Department of Neurology at Massachusetts General Hospital in Boston, Massachusetts was presented with the Richard Olney AAN/ALS Association Clinician Scientist Development Award. Dr. Berry will use this award to study ALS-associated changes in the immune system that could one day be used as an ALS biomarker. The award is named in honor of ALS researcher and neurologist Richard K. Olney, M.D., who passed away after a battle with ALS in 2012. Additionally, the Sheila Essey Award for ALS Research was presented to Rosa Rademakers, Ph.D., Associate Professor of Neuroscience at the Mayo Clinic College of Medicine in Jacksonville, Florida, and Bryan Traynor, M.D., Ph.D., Chief of the Neuromuscular Diseases Research Unit at the National Institute on Aging Laboratory of Neurogenetics, for their independent work that led to the identification of C9ORF72. The Sheila Essey Award, provided by the Essey Family Fund, is in memory of Sheila Essey who passed away after a battle with ALS in 2004.

Drug News

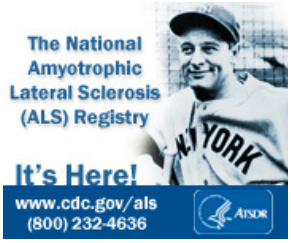
Novartis' Good News on Gilenya Raises Hopes in ALS

New results from an analysis of Phase III clinical trial data collected from more than 3,600 people showed that Novartis' new multiple sclerosis (MS) drug, Gilenya (also known as fingolimod) "significantly and consistently reduced the rate of brain volume loss" in people with relapsing and remitting MS. These findings should be encouraging for

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



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people with ALS given that this past February ALS TDI gained FDA approval for a Phase IIa clinical trial to test the safety and tolerability of Gilenya in ALS patients. To learn more about the forthcoming Phase IIa clinical trial in ALS patients, including inclusion and exclusion criteria, click here.

Phytopharm Throws in the Towel

Earlier this year, Phytopharm announced that their Phase II clinical trial of Cogane for the treatment of Parkinson's disease failed to show efficacy. In light of these negative results, Phytopharm has announced that they are cutting staff and "looking to sell the business." This announcement is disappointing not only for the Parkinson's disease community, but also for the ALS community. In December 2012, Phytopharm announced their plans to develop Cogane for the treatment of ALS. Unfortunately, considering Phytopharm's recent announcement, it is unlikely that Cogane will undergo further development for ALS.

Potential New Therapeutic Opportunities for CNS Diseases

The results of a recent Phase I trial testing the safety and tolerability of injecting an antisense oligonucleotide into the cerebrospinal fluid of people with ALS was recently published online in *Lancet Neurology*. The study was led by Dr. Timothy Miller of the Washington University School of Medicine and Dr. Merit Cudkowicz of Massachusetts General Hospital, with support from Isis Pharmaceuticals as well as the ALS Association and the Muscular Dystrophy Association. The trial showed that delivery of an antisense oligonucleotide targeted against SOD1 (ISIS 333611) by intrathecal infusion was safe and well tolerated. This is good news for ALS as well as other CNS diseases. Although it seems like Isis has no current plans to continue to develop ISIS 333611, at the 23rd Annual International Symposium on ALS/MND, two different groups announced that they are independently working with Isis to develop antisense oligonucleotide-based therapies targeting C9ORF72. Stay tuned to the ALS Forum e-Newsletter for updates!

The ALS Forum was developed by Prize4Life, Inc.
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