THE ALS FORUM

Discovering a Common Goal, Discovering a Cure

ALS Forum e-Newsletter Volume 111 **September 5, 2014**

We hope you have all had a great summer and kept cool with lots of ice buckets! Prize4Life would like to express its gratitude again to all its donors for their generosity and for supporting ALS research as part of the IceBucketChallenge. If you would like to support the ALS Forum, please donate here.

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:

The ALSGene tool: www.ALSGene.org

The PRO-ACT Database: www.ALSDatabase.org

NEALS Biofluid Repository Available to Researchers

NINDS Fibroblast Repository

VABBB Tissue Request Information Site

Funding Opportunities:

Harvard NeuroDiscovery Center, 2014 NeuroBehavior LaboratoryPilot Project Research Program. Applications due September 15, 2014.

Frick Foundation ALS Research Grants.Applications due September 30, 2014.

Blueprint Neurotherapeutics Network (BPN): SBIR Small Molecule Drug Discovery and Development for Disorders of the Nervous System (U44). Application

Research News

Defects in Mitochondrial Motility Cause Upper Motor Neuron Disease

Researchers from the laboratory of Janet Shaw at the University of Utah School of Medicine in Salt Lake City have demonstrated that restricting mitochondrial motility is sufficient to cause upper motor neuron disease in mice. According to work published August 18 online in the*Proceedings of the National Academy of Sciences*, neuron-specific deletion of the calcium-binding mitochondrial Rho 1 (Miro1), which attaches mitochondria to the kinesin and dynein motors for axonal transport, leads to progressive upper motor neuron disease in mice and death within the first month of life (see Nov 2009 news story for more about axonal transport defects in ALS). Interestingly, the mice exhibit defects in retrograde transport of mitochondria, but normal mitochondrial respiration and calcium buffering. Although further characterization is needed to establish whether these animal models reflect a specific human disease, they could potentially serve to identify drugs that specifically repair the mitochondrial distribution defects. Click here to read more about these new animal models.

MRI Abnormalities Correlate with Upper Motor Neuron Symptoms in ALS

We recently reported on early changes in the pattern of brain MRI that are detectable even prior to ALS symptom onset (see June 2014 news story). A new study published in the August edition of *PLOS One*describes MRI changes in the brain corticospinal tract, which correlate with severity of upper motor neuron (UMN) symptoms in ALS patients. The researchers, led by John Woo at the Perelman School of Medicine at the University of Pennsylvania in Philadelphia, combined computational analysis of diffusion tensor imaging (DTI) of the corticospinal tract with a detailed clinical assessment of UMN function using a new, UMN-specific scale. They found that ALS patients exhibit changes in two DTI measures, mean diffusivity (MD) and fractional anisotropy (FA), which correlate with the severity of upper motor neuron dysfunction, suggesting that the DTI changes reflect UMN pathology. The scientists are now planning follow-up studies the examine sub-regions of the corticospinal tract. Click here to read more.

due date: October 21, 2014.

NCATS Small Business Contract Funding.Application due date: November 5, 2014.

Upcoming Webinars:

Sept 17, 2014, 1-2pm EST:Exploring the Role and Impact of Crowdfunding on Medical Research.

Upcoming Meetings:

September 2014

September 8-10, 2014: Philadelphia, PA: 3rd International Conference and Exhibition on Neurology & Therapeutics

September 17-20, 2014: Minneapolis, MN: 1st ALS Research Group Meeting

September 18-20, 2014: Montreal, Quebec:Fondation André-Delambre 10th Annual ALS Symposium

October 2014

October 7-11, 2014: Berlin, Germany: 19th International World Muscle Society Congress

October 12-14, 2014: Baltimore, MD: American Neurological Association's 2014 Annual Meeting

October 22-24, 2014: Clearwater beach, FL:NEALS Annual Meeting

October 23-25, 2014: Vancouver, Canada: The 9th International Conference on Frontotemporal Dementias

November 2014

Yeast Protein Sheds Light on Potential Benefits of Caloric Restriction in ALS Patients

Abnormal repeats in the polyglutamine protein Ataxin-2 cause spinocerebellar ataxia type 2 and increase risk of developing ALS (seeAug 2010 news story and Aug 2013 news story). How exactly these mutations cause disease is not well understood. Now, researchers from University of Toronto in Ontario, Canada led by Karim Mekhail have taken a major step in this direction by studying Pbp1, the yeast ortholog of Ataxin-2. In the study published July 28 in *Developmental Cell*, the investigators demonstrate that Pbp1 inhibits the formation of non-coding DNA-RNA hybrids and prevents aberrant recombination. Caloric restriction in Pbp1-null yeast suppresses the accumulation of RNA-DNA hybrids. The scientists are now replicating these findings in tissue from ALS patients and testing whether caloric restriction is beneficial for ALS patients. Click here to read more.

Potent Drugs Synthesized in Diseased Cells Hold Promise for ALS

Investigators led by Matthew Disney at the Scripps Research Institute in Jupiter, FL have successfully demonstrated an approach to synthesize potent small molecules inside diseased cells in a highly selective and sensitive manner. The research, published August 27 in *Angewandte Chemie*, adapted a method called 'click chemistry' to create modular small molecules which bind internal loops in the RNA tetranucleotide repeats that cause myotonic dystrophy type 2, a rare form of muscular dystrophy. Inside the cells, the compounds assemble to form a drug that is 1000 times more potent than the original small molecule. This approach holds potential for other diseases associated with nucleotide repeat expansions, such as C9ORF72 ALS/FTD (see Jan 2013 news story). Click here to read more.

Drug and Device News

Biogen Idec Appoints Donald Johns to Lead ALS Innovation Hub

Biogen Idec has appointed Donald Johns, M.D., as vice president and head of its ALS Innovation Hub (ALS iHub). The ALS iHub was established as a center of innovation to integrate research and clinical development with cutting-edge new technologies, in order to accelerate therapy development for ALS. Dr. Johns' career spans 30 years in translational research for neurodegenerative diseases. Most recently, he joined Biogen-Idec from Novartis Institutes for Biomedical Research, where he was vice president and global head of Neuroscience Translational Medicine. The ALS iHub, under Dr. Johns' leadership, is a clear demonstration of Biogen Idec's commitment to bringing new therapies for ALS into the clinic. Click here to read more.

MediciNova to Begin Clinical Trial ofMN-166(ibudilast) in ALS

MediciNova, a publicly-traded biopharmaceutical company based in La Jolla, California, has obtained FDA approval to initiate a clinical trial of MN-166 (ibudilast) for the treatment of ALS. MN-166 is a first-in-class, orally bioavailable,small molecule with anti-inflammatory and neuroprotective properties. The drug has been on the market since 1989 in Japan and Korea for treatment of cerebrovascular disorders, and was licensed by MediciNova for use in neurological disorders. -The clinical

November 7, 2014: Boston, MA: 10th Annual ALS TDI Leadership Summit

November 13-14, 2014: Arlington, VA: 24th Neuropharmacology Conference

November 13-14, 2014: Arlington, VA: 9th Brain Research Conference Neuroprotection: Basic mechanisms and translational potential

November 14, 2014: Washington, DC: Advances in ALS and FTD Genetics Workshop

November 15-19, 2014: Washington, DC: The Annual Society for Neuroscience Annual Meeting

November 16-18, 2014: New York, NY: Partnering for Cures

December 2014

December 3-5, 2014: San Antonio, TX: World Stem Cell Summit

December 3-6, 2014: Cold Spring Harbor, NY:Neurodegenerative Diseases: Biology & Therapeutics

December 5-6, 2014: Brussels, Belgium:International Conference on ALS/MND

December 17-20, 2014: Hotel Vila Galé Coimbra, Portugal: Mitochondria, Metabolism and Disease

trial will test safety and tolerability of MN-166 vs. placebo when taken in combination with riluzole, as well as assess several efficacy endpoints. Click here to read more.

ALSTDI to Launch New ALS Clinical Trial With IceBucketChallenge Donations

The ALS Ice Bucket Challenge, the viral social media ALS fundraising campaign, has transformed the ALS funding landscape. How exactly the funds will be used to advance ALS research is still in the works, but Steve Perrin, President and CEO of the non-profit biotechnology company the ALS Therapy Institute (ALSTDI), has announced that half of the three million dollars the company has raised will go toward funding a new clinical trial in ALS. The identity of the drug has not yet been revealed, but it will be the second drug ever to be brought into human clinical trials by the biotech company after the repurposed multiple sclerosis drug Gilenya. Another one million will go to fund ALSTDI's newly launched precision medicine program. Click here to read more.

CQDM and ODE Co-Fund Collaboration to Identify Inhibitors of Protein Misfolding and Aggregation

The CQDM, a research consortium focused on precompetitive research to accelerate drug development, has partnered with the Ontario Center for Excellence (OCE) and together they have announced new funding for an industry-academic partnership to accelerate discovery of drugs that could be candidate therapies for neurodegenerative diseases, including ALS. The support will be granted to Xiao-Yan Wen of the Keenan Research Centre for Biomedical Sciences at St. Michael's Hospital, Pierre Drapeau of the Université de Montréal and Chrisopher Barden from Treventis, to develop a high-throughput small molecule screening platform in zebrafish to accelerate screening and identification of compounds that inhibit protein misfolding and aggregation (to read more about use of the zebrafish model in ALS, see Aug 2013 news story). CQDM and OCE will each pitch in $150k and Treventis will add $600k to support this synergistic partnership. Click here to read more.

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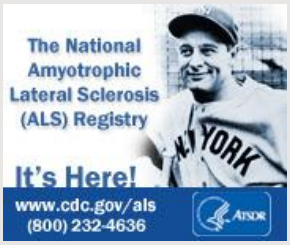

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