THE ALS FORUM

Discovering a Common Goal, Discovering a Cure

ALS Forum e-Newsletter Volume 93 **September 27, 2013**

Notice anything different? Your eyes aren't playing tricks on you! The ALS Forum and The ALS Forum e-Newsletter have a brand new look! Now that you have checked out the redesigned e-Newsletter, be sure to check out the redesigned ALS Forum website. We hope you like what you see and we would love to know what you think. Please email jgoodman@prize4life.org with any feedback you may have.

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:

National Institute of Environmental Health Sciences (NIEHS) released a new RFA: Research Linking Environmental Exposure to Neurodegenerative Disease (R21). Letter of Intent due September 30, 2013.

NIH's National Center for Advancing Translational Sciences (NCATS) released a new RFA: Rare Diseases Clinical Research Consortia (RDCRC) for Rare Diseases Clinical Research Network (U54). Letters of intent are due by October 7, 2013

MNDA Calls for Project Grant Applications. The

Research News

Paper Alert: FUS a Fixer of Damaged DNA

In the September 15th issue of *Nature Neuroscience* Dr. Li-Huei Tsai, director of MIT's Picower Institute for Learning and Memory, and her laboratory showed that fused in sarcoma (FUS) binds to histone deacetylase 1 (HDAC1) in order to facilitate the repair of double-stranded breaks in DNA. Dr. Tsai's group showed that ALS-associated mutations in FUS influence the ability of FUS to bind HDAC1, which negatively impacts the DNA repair process resulting in the accumulation of DNA damage. Dr. Tsai suggests that motor neurons are "more sensitive [to the DNA damage] because they are among the most active cells in the body." Click here to read Dr. Amber Dance's full report of this interesting story.

Tau Tracer May Light Up All Tauopathies

Researchers from the National Institute of Radiological Sciences in Japan recently developed a new tau tracer called PBB3, which can be used to detect tau aggregates in Alzheimer's disease tauopathies as well as in non-Alzheimer's disease tauopathies. PBB3 joins two other recently developed tau tracers including THK and the T807 and T808 series. Why so many tau tracers? Although the data is still being collected, THK and the T807 and T808 series appear to recognize only a subset of tauopathies. However, the researchers from Japan have evidence to suggest that PBB3 will recognize all tauopathies - including non-Alzheimer's disease tauopathies such as corticobasal degeneration (CBD). Although PBB3 still needs to be tested in people with frontotemporal dementia and chronic traumatic encephalopathy, the researchers are fairly confident that PBB3 can serve as a universal tau tracer to help diagnose all tauopathies. Click here to read the full story and find out which of the three new tracers has been used to "light up" tau-positive cases of FTD, enabling differentiation of FTD-tau from FTD-TDP-43.

Is One Protein Standing in the Way of Skin Cell-to-Stem Cell Conversion?

summary application deadline is November 1, 2013.

Upcoming Meetings:

October 2-4, 2013: Clearwater Beach, FL:2013 Annual NEALS Meeting

October 3, 2013: Boston, MA: 9th Annual ALS TDI Leadership Summit

October 14-15, 2013: Tel Aviv, Israel: BrainTech Israel 2013

October 29-30, 2013: Boston, MA: SciBX Summit on Innovation in Drug Discovery and Development 2013

November 3-5, 2013: New York, NY: FasterCures: Partnering For Cures

November 7-8, 2013: San Diego, CA: 8th Annual Brain Research Conference: RNA Metabolism in Neurological Disease

November 7-8, 2013: San Diego, CA: Workshop, Introduction to Stereology for Neuroscientists

November 9-13, 2013: San Diego, CA: Society for Neuroscience Annual Meeting: Neuroscience 2013

December 4-5, 2013: Milan, Italy: 21st Annual Meeting of the International Alliance of ALS/MND Associations

December 6-8, 2013: Milan, Italy: 24th International Symposium on ALS/MND

The discovery that mature cells can be reprogrammed into a pluripotent stem cell state has revolutionized science. In fact, one of the recipients of the 2012 Nobel Prize in Physiology or Medicine, Dr. Shinya Yamanaka of the University of Kyoto, Japan, was honored for his discovery that differentiated cells can be reprogrammed into induced pluripotent stem cells (iPS cells). Since this discovery, patient-derived iPS cells have become a promising new approach for modeling human diseases, including ALS, in a culture dish (click here to read a related ALS Forum story and check out the Harvard/Evotec story below). While iPS cells hold great potential for furthering disease-based research, getting differentiated cells to reprogram into an undifferentiated state isn't as easy as it sounds. Normally, the conversion rate is under 10% (and in most cases closer to 1%) and takes multiple weeks. Now, Dr. Jacob Hanna's group from the Weizmann Institute of Science in Rehovot, Israel has shown that they can increase the efficiency of iPS cell conversion to nearly 100% and reduce the conversion time to under two weeks, just by knocking out a single protein, Mbd3. The work was published in the September 18th issue of*Nature*. This finding could revolutionize the use of iPS cells in research and even potentially as therapies. Click here to read more about the function of Mbd3 and this exciting finding.

Bypassing the Blood-Brain Barrier by a Nose

The blood-brain barrier prevents the delivery of many potential neurodegenerative disease therapeutics to the brain. However, the good news is that there is a secret way to bypass this barrier and it happens to be right under your nose. Therapeutics that can be absorbed by the olfactory bulb can be delivered directly to the brain. The challenge is actually getting the therapeutics to be absorbed by the olfactory bulb. New research led by Dr. Massimiliano di Cagno from the University of Southern Denmark and Dr. Barbara Luppi from the University of Bologna, Italy may have solved this problem. Drs. di Cagno and Luppi developed liposomes and polymers that were successfully taken up into the olfactory bulb and subsequently showed sustained release of an encapsulated tracer over a period of at least three hours. Use of these liposomes and polymers may enable direct delivery of drugs to the brain, which has the additional benefit of minimizing the side effects that can come from oral administration. On a related note, in case you didn't catch it the first time, click here to read Prize4Life's coverage of the Alzheimer's Drug Discovery Foundation's 7th Annual Drug Discovery for Neurodegeneration Conference held in San Francisco, California, which also discussed the benefits of bypassing the blood-brain barrier using nasal drug delivery.

Drug News

Harvard Scientists Team Up with Evotec to Find New Treatments for ALS

Harvard Stem Cell Institute (HSCI) scientists Dr. Kevin Eggan and Dr. Lee Rubin have joined together with the German biotech company Evotec to target ALS. Drs. Eggan and Rubin have contributed to the field of ALS research by developing patient-derived stem cell models of ALS. Now Drs. Eggan and Rubin are collaborating with Evotec to screen potential drugs in their ALS stem cell models in the hopes of quickly identifying molecules that can be developed into ALS treatments for patients. Brock Reeve, the Executive Director of HSCI, is optimistic about the collaboration, which he says will "enabl[e] us to hasten the

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transfer of stem cell-based discoveries to the clinic." More information on the new partnership, as well as on the work of Drs. Eggan and Rubin, can be found here and here.

Neuralstem Trial Treats First Phase II ALS Patient

Maryland-based Neuralstem just announced that they have treated their first patient in the Phase II trial of their spinal cord-derived neural stem cells (known as NSI-566) for the treatment of ALS. Neuralstem completed their Phase I clinical trial of NSI-566 for the treatment of ALS last year. After positive Phase I trial results, Neuralstem was granted permission to move to a Phase II dose escalation and safety trial, which includes 15 patients who can be treated with up "to 40 injections and up to 400,000 cells per injection" at two centers. Karl Johe, Neuralstem's Chairman of the Board and Chief Scientific Officer, said that "the primary goal of our Phase II trial is to identify the maximum-safe tolerated dose." More information about the trial, which is currently ongoing at the University of Michigan and Emory University, is available here.

iPierian Raises Money to Advance Tau Antibody and Form Spin-Off

iPierian, Inc. recently announced that it has secured $30 million in financing. A portion of the money will be used to continue to advance iPierian's drug development pipeline, which includes their anti-Tau antibody that could be used in the treatment of tau-based diseases, including Alzheimer's disease and frontotemporal dementia (FTD). In addition, a portion of the financing will be used to support a spin-off called True North Therapeutics. True North is developing humanized antibodies that inhibit toxicity associated with the complement pathway, with a focus on "hematologic, renal and neurological therapeutic areas." Hopefully ALS is on their list of target therapeutic areas. Stay tuned to the ALS Forum e-Newsletter for continued updates about this developing story.

Genome Center Dream Come True for Dr. Tom Maniatis

Last week, the New York Genome Center, a collaborative partnership between twelve of "New York's biggest medical research institutions" including, University/Weill Cornell Medical College, Columbia University, Cold Spring Harbor Laboratory, the Albert Einstein College of Medicine, Rockefeller University, and others, officially opened its doors inManhattan's Lower West Side. The event was a dream come true for Dr. Tom Maniatis, a pioneer in molecular biology and a member of Prize4Life's Scientific Advisory Board. Dr. Maniatis says the Genome Center will "bring together the entire New York community to tackle the problem of making biological and medical sense of large data sets." The collaborative efforts of the New York Genome Center will involve the analysis of large sets of basic and clinical data and transition these findings toward clinical studies. The hope is that this new approach will help to accelerate our understanding of diseases such as ALS.
Click here for more information on the new center.

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