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NEW YORK STEM CELL FOUNDATION SCIENTISTS FEATURED AT THE ALZHEIMER'S ASSOCIATION INTERNATIONAL CONFERENCE FOR NEW MODEL OF ALZHEIMER'S DISEASE (AD)

A Major Advance Toward Drug Discovery

NEW YORK, NY (July 16, 2012) – A team of scientists at The New York Stem Cell Foundation (NYSCF) Laboratory led by Scott Noggle, PhD, NYSCF–Charles Evans Senior Research Fellow for Alzheimer's Disease, has developed a cell-based model of Alzheimer's disease (AD) by reprogramming skin cells of Alzheimer's patients to become brain cells that are affected in Alzheimer's. This will allow researchers to work directly on living brain cells suffering from Alzheimer's, which until now had not been possible. Andrew Sproul, PhD, a postdoctoral associate in Dr. Noggle's laboratory, will present this work on Thursday, July 19 at the Alzheimer's Association International Conference (AAIC) held in Vancouver.

Dr. Noggle and his team reprogrammed skin cell samples taken from twelve patients diagnosed with early-onset Alzheimer's and from healthy, genetically related individuals into induced pluripotent stem (iPS) cells, which can differentiate into any cell type. The team of scientists used these iPS cells to create cholinergic basal forebrain neurons, the brain cells that are affected in Alzheimer's. These cells recapitulate the features and cellular-level functions of patients suffering from Alzheimer's, a devastating disease that affects millions of people globally but for which there is currently no effective treatment.

NYSCF has pioneered the creation of disease models based on the derivation of human cells. Four years ago, a NYSCF-funded team created a cell-based model for ALS, or motor neuron disease, the first patient-specific stem cells created for any disease. The cell-based model for Alzheimer's builds on this earlier work.

"Patient derived AD cells will prove invaluable for future research advances, as they already have with patient-derived ALS cells," said NYSCF CEO Susan Solomon. "They will be a critical tool in the drug discovery process, as potential drugs could be tested directly on these cells. Although research on animals has provided valuable insight into AD, we aren't mice, and animals don't properly reflect the features of the disease we are trying to cure. As we work to find new drugs and treatments our research should focus on actual human sufferers of Alzheimer's disease," emphasized Ms. Solomon This cell-based model has already led to important findings. Preliminary results of this NYSCF research, done in collaboration with Sam Gandy, MD, PhD, an international expert in the pathology of Alzheimer's at Mount Sinai School of Medicine, demonstrated differences in cellular function in Alzheimer's patients. Specifically, Alzheimer's neurons produce more of the toxic form of beta amyloid, the protein fragment that makes up amyloid plaques, than in disease-free neurons.

"iPS cell technology, along with whole genome sequencing, provide our best chance at unravelling the causes of common forms of Alzheimer's disease," noted Dr. Gandy.

"This collaboration is a great example of how NYSCF is bringing together experts in stem cell technology and clinicians to save and enhance lives by finding better treatments," Ms. Solomon explained.

The research to be reported at the AAIC by Andrew Sproul focused on stem cell models of individuals with *presenilin-1* (*PSEN1*) mutations, a genetic cause of AD. As Dr. Sproul has said, this cell-based model could "revolutionize how we discover drugs to potentially cure Alzheimer's disease."

The New York Stem Cell Foundation (NYSCF) conducts cutting-edge translational stem cell research in its laboratory in New York City and supports research by stem cell scientists at other leading institutions around the world. More information is available at www.nyscf.org.