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Mount Sinai Leads Global Program Using Stem Cells, Collaborates with The New York Stem Cell Foundation to Accelerate Cures for Alzheimer's Disease

Researchers Using Skin Samples and Brain Imaging to Identify Causes and Cures

(New York—March 26, 2013) —Sam Gandy, MD, PhD, of the Icahn School of Medicine at Mount Sinai is leading an international team of researchers working to reprogram skin cells into brain cells to gain a better understanding of Alzheimer's disease (AD). As part of the Consortium, Dr. Gandy is collaborating with Scott Noggle, PhD, the NYSCF – Charles Evans Senior Research Fellow for Alzheimer's Disease and Director of the New York Stem Cell Foundation (NYSCF)'s laboratory in Manhattan.

Dr. Gandy heads the Stem Cell Research Consortium funded by the Cure Alzheimer's Fund (CAF). The Consortium consists of six institutions that plan to directly investigate, for the first time, brain cells in petri dishes from individual patients who have the common form of AD.

Dr. Gandy is working with Dr. Noggle's team to reprogram skin cells from AD patients into brain cells using stem-cell technology. The research team will obtain and monitor adult AD brain cells, providing not only a way to study the causes of the disease but also a system for discovering potentially effective drugs. The strategy has been nicknamed "the patient-specific disease in a dish" and enables studies on a time scale of minutes or hours, compared with mouse model testing, which routinely requires nine months to one year.

"This approach is one of our best shots at understanding common forms of Alzheimer's. Once defects are identified, we can use these same brain cells to screen for new drugs," said Dr. Gandy, Professor of Neurology and Psychiatry and Director of the Center for Cognitive Health at Mount Sinai. "This breakthrough technology will enable us to identify genetic and biochemical differences underlying the most common form of Alzheimer's disease."

In collaboration with Mary Sano, PhD, Professor of Psychiatry and Director of the Mount Sinai Alzheimer's Disease Research Center (ADRC), Dr. Gandy plans to select carefully characterized patients and healthy participants from the ADRC who will have skin biopsies and will also undergo brain scans to detect the amount of amyloid plaque, the hallmark of AD, present in the brain. Samples will also be collected from a skin cell bank at the National

Institutes of Health. The scans will be used to confirm AD, the risk for developing AD, and whether a brain is amyloid-free.

Dr. Noggle will reprogram these skin cells into the various cell types that make up the brain, employing the NYSCF Global Stem Cell Array, a breakthrough automated robotic technology that produces standardized stem cell lines. Results are specific to the patient's genetic makeup, allowing researchers to uncover Alzheimer's-related changes at an individual level and to track changes that might otherwise go undiscovered.

"Having all the cell types together in the same dish enables us to mimic as closely as possible the normal and the diseased adult human brain," said Dr. Gandy. "In these mixed cultures, we will study the roughly three-dozen genes that have been linked to AD to see if any are dysfunctional in such a way as to cause one or more known features of the disease."

To encourage international collaboration in Alzheimer's treatment, consortium researchers will create a stem cell bank that can be accessed globally to accelerate drug screening worldwide. This collaboration is an example of NYSCF's commitment to work with global collaborators to advance research.

"We can, for the first time, test drugs across a large, diverse population of Alzheimer's patients, using only their cells. This stem cell resource will embolden scientific investigations and accelerate bench to beside delivery of new treatments," said Dr. Noggle. "We're incredibly excited to be working with Dr. Gandy and fellow collaborators to find a cure for Alzheimer's disease."

Other organizations involved in the Consortium are Hadassah University Medical Center, Harvard Medical School and Massachusetts General Hospital, Harvard University Stem Cell Institute, and The Rockefeller University, who is pursuing related research separately funded by CAF.

About The Mount Sinai Medical Center
The Mount Sinai Medical Center encompasses both The Mount Sinai Hospital and Icahn School of Medicine at Mount Sinai. Established in 1968, the Icahn School of Medicine is one of the leading medical schools in the United States, and is noted for innovation in education, biomedical research, clinical care delivery, and local and global community service. It has more than 3,400 faculty in 32 departments and 14 research institutes, and ranks among the top 20 medical schools both in National Institutes of Health (NIH) funding and by U.S. News & World Report.

The Mount Sinai Hospital, founded in 1852, is a 1,171-bed tertiary- and quaternary-care teaching facility and one of the nation's oldest, largest and most-respected voluntary hospitals. In 2012, U.S. News & World Report ranked The Mount Sinai Hospital 14th on its elite Honor Roll of the nation's top hospitals based on reputation, safety, and other patient-care factors. Mount Sinai is one of 12 integrated academic medical centers whose medical school ranks among the top 20 in NIH funding and by U.S. News & World Report and whose hospital is on the U.S. News & World Report Honor Roll. Nearly 60,000 people were

treated at Mount Sinai as inpatients last year, and approximately 560,000 outpatient visits took place.

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About The New York Stem Cell Foundation
The New York Stem Cell Foundation (NYSCF) is an independent research institute founded in 2005 to accelerate cures and better treatments for patients through stem cell research. NYSCF has over 40 researchers in its New York laboratory and is an acknowledged world leader in stem cell research and in developing pioneering stem cell technologies, including the NYSCF Global Stem Cell Array. Additionally, NYSCF supports another 60 researchers at other leading institutions worldwide through its Innovator Programs, including the NYSCF – Druckenmiller Fellowships and the NYSCF-Robertson Investigator Awards. NYSCF focuses on translational research in a model designed to overcome the barriers that slow discovery and replaces silos with collaboration.

NYSCF researchers have achieved four major discoveries in the field, including: the discovery of a clinical cure to prevent transmission of maternal mitochondrial diseases in December 2012; the derivation of the first-ever patient specific embryonic stem cell line (#1 Medical Breakthrough of 2011 by *Time* magazine); the discovery of a new way to reprogram stem cells; and the creation of the first disease model from induced pluripotent stem cells (also named the #1 Medical Breakthrough by *Time* magazine in 2008). More information is available at www.nyscf.org.

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