Contact: David McKeon
212-365-7440
dmckeon@nyscf.org

Contact: Maggie McGuire
212-509-0995 ext. 295
mmcguire@michaeljfox.org

THE NEW YORK STEM CELL FOUNDATION RESEARCH INSTITUTE ANNOUNCES PARTNERSHIP WITH PARKINSON'S PROGRESSION MARKERS INITIATIVE

Modeling and identifying predictors of Parkinson's disease with stem cells

NEW YORK, NY (October 15, 2013) – The New York Stem Cell Foundation (NYSCF) Research Institute has entered into a partnership with the Parkinson's Progression Markers Initiative (PPMI) to build resources for studying Parkinson's disease to accelerate new treatments. Sponsored by The Michael J. Fox Foundation for Parkinson's Research, PPMI is a landmark effort to identify and validate biomarkers of Parkinson's disease. The study is currently under way at 32 clinical sites worldwide with funding from 13 industry partners.

NYSCF will generate stem cell lines from PPMI participants, allowing researchers to examine how Parkinson's disease develops in and progresses in a large and genetically diverse group of patients. This research could potentially lead to the development of treatments tailored to different subtypes of PD. To accelerate discovery and drug development, these stem cell lines will be made available, with de-identified patient data, to the scientific community at large.

"To prevent or slow the progression of Parkinson's disease, we need to elucidate the pathobiology of PD," said Ken Marek, PhD, principal investigator of PPMI and senior scientist and co-founder of the Institute for Neurodegenerative Disorders. "The cell lines generated by The New York Stem Cell Foundation will be an important tool to help us understand the etiology and progression of PD in combination with the comprehensive biomarker data already coming from PPMI."

Lawrence E. Golub and Karen L. Finerman have committed to fund the first phase of this partnership. The couple previously funded the NYSCF – Golub Stem Cell Research Initiative for Parkinson's Disease Research, which provided a basis for the collaboration. Golub and Finerman are both members of the NYSCF Leadership Council and Finerman also serves on the Michael J. Fox Foundation Board of Directors.

Pursuit of Parkinson's Biomarkers

PPMI is a long-term, observational study that tracks the progression of PD to identify biomarkers: biological substances, processes or characteristics associated with the risk, presence and/or progression of disease. The study completed initial recruitment of 400 PD patients and 200 healthy, age and gender-matched controls in early 2013. Participants undergo longitudinal imaging, clinical and biological tests over five years, and, since its inception, PPMI has offered all qualified investigators real-time access to that de-identified data through an online database. In addition to the initial cohort of 600, PPMI has expanded to enroll individuals without PD but with known risk factors – smell loss, REM sleep behavior disorder and certain genetic mutations – to characterize biomarkers before the onset of motor symptoms.

"We are very excited to be a part of this joint effort with the Parkinson's Progression Markers Initiative. This collaboration will provide a comprehensive look at this devastating disease at the cellular level across a broad number of patients," said Susan L. Solomon, Chief Executive Officer of NYSCF. "We are very grateful to Lawrence Golub and Karen Finerman for their pioneering support of the NYSCF – Golub Stem Cell Research Initiative for Parkinson's Disease Research."

Automated Stem Cell Technology

At the NYSCF Research Institute, scientists will make stem cell lines using NYSCF's Global Stem Cell Array™, an automated robotic platform that makes it possible to create identical stem cell lines from a large number of patients. Through advanced stem cell techniques, isolated skin cells are "reprogrammed"—or reverted—into an embryonic-like state, then known as induced pluripotent stem (iPS) cells. These iPS cells can become any of the cell types implicated in PD, replicate indefinitely, and reflect the progression of the disease in the laboratory.

NYSCF scientists will produce disease models using iPS cell-derived, dopamine-producing cells, known as dopaminergic neurons, which degenerate in Parkinson's disease. Observation of pathological changes in these models will illuminate the pathology of PD, which can identify potential drug targets. These cell lines can also be used in the testing of drug candidate compounds.

Traditionally, iPS cells are produced, with varying success, by hand. Procured cells may not be fully reprogrammed or may carry over molecular "memories" of their adult cell pasts. The NYSCF Global Stem Cell Array™ eliminates these confounding variables; iPS cells are completely standardized, without any possibility of alteration to the donor's DNA, providing researchers with comparable cells to pinpoint cellular-level changes.

Parkinson's disease is a degenerative disorder of the central nervous system that affects five million people worldwide. There is no cure for PD, and current therapies do not alleviate all symptoms such as tremor, rigidity and impaired balance, depression, and mild cognitive impairment. Most cases of Parkinson's are idiopathic, meaning that the cause of the disease is unknown, yet one to two percent carry a genetic mutation attributed to the development of PD. Contributing risk factors remain poorly understood.

###

About The New York Stem Cell Foundation

The New York Stem Cell Foundation (NYSCF) is an independent research institute founded in 2005 that accelerates cures and better treatments for patients through stem cell research. NYSCF has over 45 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 is conducting a new model of research that is overcoming the barriers that slow discovery and leverages multi-institutional collaboration.

NYSCF scientists have achieved four major research breakthroughs in the field, including: the recent 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 (named the #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).

About the Parkinson's Progression Markers Initiative

The Parkinson's Progression Markers Initiative (PPMI) is a $55-million international clinical study sponsored by The Michael J. Fox Foundation for Parkinson's Research (MJFF) and funded by a consortium of 13 industry partners in conjunction with MJFF. Launched in 2010, PPMI aims to find reliable and consistent biomarkers of Parkinson's disease (PD) progression. The study is testing today's most promising biomarker candidates through neuroimaging, the collection of blood, urine, and spinal fluid, and clinical and behavioral tests. Valid measures could allow scientists to predict, objectively diagnose and monitor diseases in both Parkinson's disease patients and populations at-risk to developing Parkinson's. In April 2013, PPMI completed the recruitment of 400 newly diagnosed PD patients and 200 control subjects. Using the same infrastructure and protocols, a pre-motor arm of PPMI is evaluating two at-risk cohorts (individuals with decreased sense of smell and people with REM sleep behavior disorder) and a genetics arm is evaluating Parkinson's patients and healthy controls with a LRRK2 or alpha-synuclein genetic mutation or a first-degree relative with such a mutation.

About The Michael J. Fox Foundation for Parkinson's Research

Our challenges don't define us. Our actions do.

The Michael J. Fox Foundation exists for one reason: to find the cure for Parkinson's disease in our lifetime. Parkinson's is the second most common brain disease, estimated to affect one in 100 individuals over age 60. Founded by Michael J. Fox in 2000, the Foundation has quickly grown to become the largest nonprofit funder of Parkinson's research globally, and was called "the most credible voice on Parkinson's research in the

world" by *The New York Times*. As Michael returns to network television full-time after more than two decades living with Parkinson's disease, the Foundation has launched think/able, a project celebrating the power of optimism and determination to overcome challenges and achieve our biggest goals. Now through the end of October, visit the Foundation's Web site to write Michael a message about how you think/able to reach for your dreams. www.michaeljfox.org/thinkable

###