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NYSCF SCIENTISTS ONE STEP CLOSER TO CELL THERAPY FOR MULTIPLE SCLEROSIS PATIENTS

New study shows efficiency at making living human cells from MS patients' skin samples

NEW YORK, NY (July 24, 2014) – Scientists at The New York Stem Cell Foundation (NYSCF) Research Institute are one step closer to creating a viable cell replacement therapy for multiple sclerosis from a patient's own cells.

For the first time, NYSCF scientists generated induced pluripotent stem (iPS) cells lines from skin samples of patients with primary progressive multiple sclerosis and further, they developed an accelerated protocol to induce these stem cells into becoming oligodendrocytes, the myelin-forming cells of the central nervous system implicated in multiple sclerosis and many other diseases.

Existing protocols for producing oligodendrocytes had taken almost half a year to produce, limiting the ability of researchers to conduct their research. This study has cut that time approximately in half, making the ability to utilize these cells in research much more feasible.

Stem cell lines and oligodendrocytes allow researchers to “turn back the clock” and observe how multiple sclerosis develops and progresses, potentially revealing the onset of the disease at a cellular level long before any symptoms are displayed. The improved protocol for deriving oligodendrocyte cells will also provide a platform for disease modeling, drug screening, and for replacing the damaged cells in the brain with healthy cells generated using this method.

“We are so close to finding new treatments and even cures for MS. The enhanced ability to derive the cells implicated in the disease will undoubtedly accelerate research for MS and many other diseases,” said Susan L. Solomon, NYSCF Chief Executive Officer.

"We believe that this protocol will help the MS field and the larger scientific community to better understand human oligodendrocyte biology and the process of myelination. This is the first step towards very exciting studies: the ability to generate human oligodendrocytes in large amounts will serve as an unprecedented tool for developing remyelinating strategies and the study of patient-specific cells may shed light on intrinsic pathogenic mechanisms

that lead to progressive MS". said Dr. Valentina Fossati, NYSCF – Helmsley Investigator and senior author on the paper.

In multiple sclerosis, the protective covering of axons, called myelin, becomes damaged and lost. In this study, the scientists not only improved the protocol for making the myelin-forming cells but they showed that the oligodendrocytes derived from the skin of primary progressive patients are functional, and therefore able to form their own myelin when put into a mouse model. This is an initial step towards developing future autologous cell transplantation therapies in multiple sclerosis patients

This important advance opens up critical new avenues of research to study multiple sclerosis and other diseases. Oligodendrocytes are implicated in many different disorders, therefore this research not only moves multiple sclerosis research forward, it allows NYSCF and other scientists the ability to study all demyelinating and central nervous system disorders.

“Oligodendrocytes are increasingly recognized as having an absolutely essential role in the function of the normal nervous system, as well as in the setting of neurodegenerative diseases, such as multiple sclerosis. The new work from the NYSCF Research Institute will help to improve our understanding of these important cells. In addition, being able to generate large numbers of patient-specific oligodendrocytes will support both cell transplantation therapeutics for demyelinating diseases and the identification of new classes of drugs to treat such disorders,” said Dr. Lee Rubin, NYSCF Scientific Advisor and Director of Translational Medicine at the Harvard Stem Cell Institute.

Multiple sclerosis is a chronic, inflammatory, demyelinating disease of the central nervous system, distinguished by recurrent episodes of demyelination and the consequent neurological symptoms. Primary progressive multiple sclerosis is the most severe form of multiple sclerosis, characterized by a steady neurological decline from the onset of the disease. Currently, there are no effective treatments or cures for primary progressive multiple sclerosis and treatments relies merely on symptom management.

NYSCF stem cell researcher Valentina Fossati, PhD, is the senior author and NYSCF researcher Panagiotis Douvaras, PhD, is the first author of this study.

Key collaborators on this research included Dr. Saud Sadiq and the Tisch Multiple Sclerosis Research Center of New York where patients were recruited, Dr. Fraser Sim of the State University of New York at Buffalo for the *in vivo* studies, and Dr. James Goldman of Columbia University Medical Center.

The New York Stem Cell Foundation research was supported by a NYSCF – Helmsley Early Career Investigator Award, The New York Stem Cell Foundation, and The Leona M. and Harry B. Helmsley Charitable Trust. The *in vivo* studies were supported by the Empire State Stem Cell Fund through New York State Department of Health, Contact C028108 to FJS.

About The New York Stem Cell Foundation

The New York Stem Cell Foundation (NYSCF) is an independent organization founded in 2005 to accelerate cures and better treatments for patients through stem cell research.

NYSCF employs over 45 researchers at the NYSCF Research Institute, located in New York, 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 seven major discoveries in the field, including: the first diploid stem cell line from a patient with type 1 diabetes using somatic cell nuclear transfer in April 2014; the first stem cell-derived beta cell model that accurately reflects the features of a genetic form of diabetes in June 2013; the generation of functional, immune-matched bone substitutes from patients' skin cells (featured in *The Wall Street Journal* in May 2013); the discovery of a clinical cure to prevent transmission of maternally inherited 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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