

The logo for The New York Stem Cell Foundation (NYSCF) is a red square containing the text "NYSCF" in large, white, bold, sans-serif capital letters. Below "NYSCF", the text "The New York Stem Cell Foundation" is written in a smaller, white, sans-serif font, with "The New York" on the first line and "Stem Cell Foundation" on the second line.

The New York
Stem Cell Foundation

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SCIENTISTS AT NEW YORK STEM CELL FOUNDATION, COLUMBIA U. MAKE ADVANCE IN DEVELOPMENT OF PATIENT-SPECIFIC STEM CELLS

Major Step Toward Cell-Based Therapies for Life-Threatening Diseases

NEW YORK, NY (October 5, 2011) – A team of scientists led by Dieter Egli and Scott Noggle at The New York Stem Cell Foundation (NYSCF) Laboratory in New York City have made an important advance in the development of patient-specific stem cells that could impact the study and treatment of diseases such as diabetes, Parkinson's, and Alzheimer's.

As reported in today's *Nature*, for the first time the scientists have derived embryonic stem cells from individual patients by adding the nuclei of adult skin cells from patients with type 1 diabetes to unfertilized donor oocytes.

The achievement is significant because such patient-specific cells can potentially be transplanted to replace damaged or diseased cells in persons with diabetes and other diseases without rejection by the patient's immune system. The scientists report further work is necessary before such cells can be used in cell-replacement medicine.

The research was conducted in The NYSCF Laboratory in collaboration with clinicians and researchers at Columbia University Medical Center. DNA analysis was provided by scientists at the University of California, San Diego.

“The specialized cells of the adult human body have an insufficient ability to regenerate missing or damaged cells caused by many diseases and injuries,” said Dr. Egli, NYSCF senior scientist in the study. “But if we can reprogram cells to a pluripotent state, they can give rise to the very cell types affected by disease, providing great potential to effectively treat and even cure these diseases. In this three-year study, we successfully reprogrammed skin cells to the pluripotent state. Our hope is that we can eventually overcome the remaining hurdles and use patient-specific stem cells to treat and cure people who have diabetes and other diseases.”

“The ultimate goal of this study is to save and enhance lives by finding better treatments and eventually cures for diabetes, Alzheimer's, Parkinson's and other debilitating diseases and injuries affecting millions of people across the US and the globe,” said NYSCF CEO Susan L. Solomon. “This research brings us an important step closer to creating new healthy cells for patients to replace their cells that are damaged or lost through injury.”

The scientists demonstrate for the first time that the transfer of the nucleus from an adult skin cell of a patient into an oocyte without removing the oocyte nucleus results in reprogramming of the adult nucleus to the pluripotent state. Embryonic stem cell lines were then derived from the oocyte containing the patient's genetic material.

Since these pluripotent stem cells also have a copy of the chromosome from the oocyte, resulting in an abnormal number of chromosomes, these cells are not ready for therapeutic use. Future work will focus on understanding the role of the oocyte chromosome so that patient-specific stem cells can be made that contain only the patient's DNA.

In the study, skin cells from patients with type 1 diabetes and healthy patients (control group) were reprogrammed, allowing the derivation of pluripotent stem cells, cells that have the capacity for universal tissue production. Such cells potentially could be used to create beta cells that produce insulin.

Patients with type 1 diabetes lack insulin-producing beta cells, resulting in insulin deficiency and high blood sugar levels. Producing beta cells from stem cells for transplantation holds promise for the treatment and potential cure of type 1 diabetes.

“This is an important step toward generating stem cells for disease modeling and drug discovery, as well as for ultimately creating patient-specific cell replacement therapies for people with diabetes or other degenerative diseases or injuries,” said Rudolph L. Leibel, MD, co-director of Columbia's Naomi Berrie Diabetes Center and a collaborator in the study.

The study raises the possibility of using somatic cell reprogramming to create banks of stem cells that could be used for a wide range of patients, noted another collaborator, Robin Goland, MD, co-director of the Naomi Berrie Diabetes Center. “In theory, stem cell lines could be matched to a particular patient, much as we do now when we screen an individual for compatibility with a kidney transplant,” she said.

“This project is a great example of how enormous strides can be achieved when investigators in basic science and clinical medicine collaborate,” said Mark V. Sauer, MD, a coauthor of the paper and Vice Chairman of the Department of Obstetrics and Gynecology and chief of reproductive endocrinology at Columbia University Medical Center. Dr. Sauer is also program director of assisted reproduction at the Center for Women's Reproductive Care. “I feel fortunate to have been able to participate in this important project.”

Zach W. Hall, PhD, former Director of the NIH's National Institute of Neurological Disorders and Stroke and former President of the California Institute for Regenerative Medicine said, “This work represents a major advance toward the production of patient-specific stem cells for therapeutic use by demonstrating that the nucleated oocyte has the ability to completely reprogram the nucleus of an adult human cell.”

The study was funded solely with private funding and adhered to ethical guidelines adopted by the American Society for Reproductive Medicine and the International Society for Stem Cell Research, as well as protocols reviewed and approved by the institutional review board and stem cell committees of Columbia University.

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.

Columbia University Medical Center (CUMC) provides international leadership in basic, pre-clinical and clinical research, in medical and health sciences education, and in patient care. More information is available at www.cumc.columbia.edu.