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THE NEW YORK STEM CELL FOUNDATION PARTNERS WITH BEYOND BATTEN DISEASE FOUNDATION TO FIGHT JUVENILE BATTEN DISEASE

Batten Disease Support and Research Association collaborates to give families the opportunity to participate at its Annual Family Conference

NEW YORK, NY (July 23, 2014) – The New York Stem Cell Foundation (NYSCF) and Beyond Batten Disease Foundation (BBDF) have partnered to develop stem cell resources to investigate and explore new treatments and ultimately find a cure for juvenile Batten disease, a fatal illness affecting children.

NYSCF scientists will create induced pluripotent stem (iPS) cell lines from skin samples of young people affected by juvenile Batten disease as well as unaffected family members. IPS cell lines are produced by artificially "turning back the clock" on skin cells to a time when they were embryonic-like and capable of becoming any cell in the body. Reprogramming juvenile Batten iPS cells to become brain and heart cells will provide the infrastructure needed to investigate what is going wrong with the cells adversely affected by the disease. Thus far, efforts to study juvenile Batten disease have been done using rodent models or human skin cells, neither of which accurately mimic the disease in the brain, leaving researchers without proper tools to study the disease or a solid platform for testing drugs that prevent, halt, or reverse its progression. This will be the largest and first genetically diverse collection of human iPS cells for a pediatric brain disease.

In addition to working with BBDF to actively recruit patients and families to donate skin samples, Batten Disease Support and Research Association (BDSRA) is providing resources and technical support, spreading awareness among academic scientists, and notifying its Pharmaceutical partners. Together, BBDF and BDSRA will ensure that juvenile Batten disease and other researchers are aware of and utilize the 48 stem cell lines resulting from this collaboration to further juvenile Batten disease research worldwide.

"We know the genetic mutations associated with juvenile Batten disease. This partnership will result in stem cell models of juvenile Batten, giving researchers an unprecedented look at how the disease develops, speeding research towards a cure," said Susan L. Solomon, NYSCF Chief Executive Officer.

"Working with NYSCF to generate functional neuronal subtypes from patients and families is a stellar example of one of our key strategies in the fight against juvenile Batten disease: creating resource technology with the potential to transform juvenile Batten disease research and accelerate our timeline to a cure," said Danielle M. Kerkovich, PhD, BBDF Principal Scientist.

Juvenile Batten disease begins in early childhood between the ages of five and ten. Initial symptoms typically begin with progressive vision loss, followed by personality changes, behavioral problems, and slowed learning. These symptoms are followed by a progressive loss of motor functions, eventually resulting in wheelchair use and premature death. Seizures and psychiatric symptoms can develop at any point in the disease.

Juvenile Batten disease is one disorder in a group of rare, fatal, inherited disorders known as Batten disease. Over 40 different errors (mutations) in the *CLN3* segment of DNA (gene) have been attributed to juvenile Batten disease. The pathological hallmark of juvenile Batten is a buildup of lipopigment in the body's tissues. It is not known why lipopigment accumulates or why brain and eventually, heart cells are selectively damaged. It is, however, clear that we need disease-specific tools that reflect human disease in order to figure this out and to build therapy.

NYSCF is a world leader in stem cell research and production with a mission to find cures for the devastating diseases of our time, including juvenile Batten disease. NYSCF has developed the NYSCF Global Stem Cell Array™, an automated robotic technology that standardizes and scales stem cell production and differentiation, enabling the manufacture and analysis of large numbers of identical cells from skin samples of patients. The Array technology allows for the production of large-scale iPS cells that have the potential to become any cell type in the body.

This collaboration brings together the expertise of these two leading non-profit organizations, the support of BDSRA, and the participation of affected families, to create and make available to researchers, juvenile Batten disease iPS cell lines. Building on the NYSCF Research Institute's leading stem cell expertise and unique automated technology and analytics, while taking advantage of the tremendous resources and expertise of BBDF, BDSRA and affected families, this collaboration will move research in this field forward by providing the first large-scale human platform of affected cells to academic and industry scientists. These cell lines will be a renewable source of disease-relevant human brain and heart cell populations used for 1) fundamental discovery, 2) identifying therapeutic targets, and 3) testing therapies; including cell transplantation.

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.

About Beyond Batten Disease Foundation

Beyond Batten Disease Foundation works to cure and prevent juvenile Batten disease, a rare, inherited neurological disorder that strikes young children, first causing vision loss and seizures, then cognitive and motor impairment, and ultimately death by the late teens or 20s. The foundation raises funds for research and is leading development of an easy and inexpensive, groundbreaking blood test to detect the gene mutations that cause juvenile Batten disease as well as 750-plus, other rare but serious and often fatal childhood ailments. For more information, visit www.beyondbatten.org.

About Batten Disease Support and Research Association

The Batten Disease Support and Research Association (BDSRA) is the largest nonprofit organization in North America dedicated to funding research, advancing education, providing family support services, and raising awareness of the disease and its impact. Founded in 1987, the focus of BDSRA is to help unravel the mysteries of Batten disease by bringing the worlds of science, research, and health care together toward a common goal: the discovery of treatments and cures. For more information see: www.bdsra.org