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THE NEW YORK STEM CELL FOUNDATION AND THE CHARCOT-MARIE-TOOTH ASSOCIATION ENTER COLLABORATION TO ADVANCE NEUROPATHIES RESEARCH

New York, NY (December 10, 2014) – The New York Stem Cell Foundation (NYSCF) Research Institute, a non-profit organization dedicated to accelerating cures through stem cell research, announced a collaboration today with the Charcot-Marie-Tooth Association (CMTA), a patient-led disease foundation with the mission to advance research on genetic neuropathies that leads to the development of new therapies. The immediate aim of the collaboration is to develop a bank of induced pluripotent stem cell (iPSC) lines for a variety of neuropathy disorders of known genetic causation and to eventually develop personalized drug therapies.

NYSCF will make stem cells lines from Charcot-Marie-Tooth patient materials that have been curated in a biobank assembled by Dr. Michael Shy at the University of Iowa, a member of the CMTA STAR consortium of sponsored investigators. Utilizing its automated technology, the NYSCF Global Stem Cell Array™, NYSCF will systematically generate iPSC lines from tissue samples obtained from patients representing a number of disease states. These cell lines will then be used to develop methods for creating differentiated cells that mimic the myelin-producing Schwann cells that are defective in Type 1 Charcot-Marie-Tooth (CMT) disorders of peripheral nerve, as well as the motor and sensory neurons that are defective in Type 2 disorders. Members of the STAR consortium currently engaged in this CMTA-sponsored effort to differentiate iPSC lines include Dr. Robert Baloh, Cedar-Sinai Medical Center, and Dr. Gabsang Lee, Johns Hopkins University. The ultimate aim of this research is to create a personalized medicine approach to rapid testing of human drug responsiveness in a dish. The iPSC lines will also be expanded and banked by NYSCF and made available to the global scientific community to be used for research and the development of therapies.

Patrick Livney, CEO of the CMTA notes: "The Foundation has assembled the scientific and clinical key opinion leaders in CMT disorders, and the research tools necessary to validate therapeutic opportunities for their clinical potential. We have set out to engage drug makers to work together with the CMTA to advance new therapeutic approaches to our patients, and our STAR network that combines this world class research expertise with an operational capability has been highly enabling to the formation of collaborative alliances for this

purpose. Currently, there are no therapies for the different CMT disorders to halt either the onset or progression of the disease. This NYSCF collaboration represents an exciting opportunity for the CMTA to place research on therapies for Charcot-Marie-Tooth disorders in a personalized, patient context at a very early stage.

"We are very exctied to partner with the Charcot-Marie-Tooth Association to develop resources that will enable the pursuit of new treatments and eventually cures for neruropathy disorders," said Susan L. Solomon, Co-Founder and CEO of NYSCF. "Partnering with CMTA provides us with the necessary community of scientists, patients, disease experts, as well as resources that allows us to move research forward. We believe that this type of interdisciplinary collaboration between various stakeholders is essential to to move research forward in the pursuit of cures."

About Charcot-Marie-Tooth Association

The Charcot-Marie-Tooth Association (CMTA) is a registered 501c3 dedicated to serving an international patient community that suffers from rare and disabling neuropathies of genetic origin (http://www.cmtausa.org). The Foundation directly engages its STAR scientific and clinical research network in the identification, validation and clinical development of therapies for the different Charcot-Marie-Tooth disorders.

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. For more information, visit www.nyscf.org