



U of U Cell Therapy Lab Collaborates with Q Therapeutics On Stem Cell Therapy to Fight Lou Gehrig's Disease

\$5 million NIH grant will help bring therapy to human clinical trials

SALT LAKE CITY— April 17, 2009— A team of Utahns is collaborating on a stem cell therapy to fight amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease. With \$5 million dollars in funding from the National Institutes of Health (NIH), Linda Kelley, Ph.D., director of the University of Utah's Cell Therapy Facility, James Campanelli, Ph.D., of University of Utah spin-out Q Therapeutics, Inc., and Utah native Nicholas Maragakis, M.D., of The Johns Hopkins University School of Medicine, have teamed up to bring the cell-based therapy to the point of human clinical trials to treat this deadly disease. The four-year NIH grant will enable critical manufacturing and testing requirements necessary to gain U.S. Food and Drug Administration approval for human clinical trials.

Kelley, principal investigator on the grant and professor of internal medicine at the University of Utah School of Medicine, said the project is a collaboration in the truest sense. "While the University will be home to the grant, the stem-cell technology that Q Therapeutics brings to the table and the clinical expertise of Dr. Maragakis are essential to the project. We are pleased to help bring this groundbreaking therapy toward human use," Kelley said. "Our collaboration is a terrific example of how public-private partnerships can make innovative therapeutic products a reality."

According to Jack Brittain, University vice president for technology venture development, "The translational research that this funding supports—beyond basic research, but not yet in clinical trials—has been traditionally very difficult to fund. This award validates the approach being taken here at the University of Utah toward emerging technologies, such as regenerative medicine. This kind of collaboration between the University and its commercial spin-out companies is something we strive for and enthusiastically support."

ALS is a progressive neurodegenerative disease that kills certain nerve cells in the brain and spinal cord. As these cells degenerate, they lose the ability to send impulses that control muscle movement for speech, breathing, limb movement, and other functions, with death from respiratory failure typically occurring from two to five years after diagnosis. ALS affects roughly 30,000 people in this country.

The cell-based ALS therapeutic originates from research at the University of Utah by Mahendra Rao, M.D., Ph.D., a co-founder of Salt Lake City-based Q Therapeutics, Inc.

"Q Therapeutics is delighted to be working with the University of Utah Cell Therapy Facility and Dr. Maragakis on this groundbreaking project," said Campanelli, senior director of research and development for Q Therapeutics. "The Cell Therapy Facility is one-of-a-kind in the Intermountain

West. We are fortunate to be able to work so closely with Dr. Kelley and her team. The close proximity of our two groups has allowed us to readily address manufacturing and processing issues that would have been a challenge to overcome had we needed to go outside Utah.”

In bringing together cell therapy and neurology, the collaboration focuses on two of seven life science industry sectors identified by the State of Utah for long-term development. “Given the current economic climate, this type of grassroots effort is critical to both near-term job preservation and long-term development of Utah’s life sciences industry,” said Jason Perry, executive director of the Governor’s Office of Economic Development. “This project is perfectly aligned with the state’s targeted economic cluster for the Life Sciences and is a model for public and private collaboration.”

Maragakis, a Salt Lake City native and graduate of the University of Utah School of Medicine, added, “This is an important milestone in the development of therapeutics to treat those who suffer with ALS. Given the lack of good treatment alternatives for this fatal disease, this project could lead to a first-in-class therapy that significantly alters the course of disease for many ALS patients.” Maragakis and his team of researchers at Johns Hopkins recently published results of their work in ALS in Nature Neuroscience, showing that a specific type of brain stem cell therapy can be effective in an animal model of ALS.

About the University of Utah Cell Therapy Facility: Established in 1990, the University of Utah’s Cell Therapy Facility (CTF) has grown from a two-person laboratory at the University Hospital to a 18,000-square-foot cell processing and manufacturing facility that employs 40 scientists and staff. CTF provides cell processing and manufacturing services for University of Utah researchers as well as commercial entities in the cell therapy field. To date, CTF has supported two successful Investigational New Drug (IND) filings with the FDA for cell-based therapeutics. It currently supports three pre-IND cell therapeutics and has 15 contracts with commercial entities for a variety of cell processing and manufacturing services. For more information, visit www.medicine.utah.edu/cell

About Q Therapeutics, Inc.: Q Therapeutics, Inc. is an emerging biopharmaceutical company, venture-backed and privately held, developing products to treat debilitating diseases of the central nervous system. The Company has exclusive rights to 17 patents arising out of work done by Mahendra Rao, M.D., Ph.D., at the University of Utah and NIH, as well as rights to pending patents from Steven Goldman, M.D., Ph.D. and the Cornell Medical Foundation. The company’s first product, Q-Cells®, is a cell-based therapeutic intended to restore or preserve normal function of neurons by providing essential support functions that occur in healthy central nervous system tissues. QCells® may be applicable to a wide range of demyelinating diseases, including multiple sclerosis, transverse myelitis, cerebral palsy, and white matter stroke, as well as other neurodegenerative diseases such as ALS (Lou Gehrig’s Disease), traumatic spinal cord injury, Parkinson’s and Alzheimer’s Disease. Initial clinical targets are transverse myelitis, a rapidly paralyzing, inflammatory demyelinating spinal cord injury related to MS; and ALS, with a first IND filing targeted in 2010. Q’s pipeline includes other neural cell products for treating diseases including peripheral neuropathies, as well as use of its proprietary cells for new drug discovery. For more information, visit www.qthera.com