



MeiraGTx Expands ALS Gene Therapy Program in Collaboration with Weill Cornell Medicine and LSU

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— *Program seeks to treat ALS patients by activating cells' own quality control mechanisms* —

NEW YORK, October 11, 2016 – MeiraGTx, a New York and UK based gene therapy company, announces an expansion of its gene therapy program to treat amyotrophic lateral sclerosis (Lou Gehrig's Disease, ALS).

MeiraGTx, together with Prof. Gregory Petsko, D.Phil. (The Arthur J. Mahon Professor of Neuroscience in the Feil Family Brain and Mind Research Institute at Weill Cornell Medicine), and Prof. Ronald Klein, Ph.D. (Associate Professor Department of Pharmacology, Toxicology and Neuroscience, LSU Health Shreveport), are developing a gene therapy to target TDP-43 toxicity in ALS patients. The cellular protein TDP-43 is misregulated in the vast majority of all ALS patients and is emerging as a possible Achilles' heel in the treatment of this deadly neurodegenerative disease.

Research conducted in the Petsko laboratory (in collaboration with coworkers at Brandeis University, Wright State University and the University of Michigan) revealed that one of the cell's own quality control mechanisms, called the nonsense-mediated decay (NMD) pathway, can alleviate TDP-43 mediated toxicity in cell-based disease models of ALS. Increased expression of UPF1, the master regulator of NMD, significantly protects mammalian motor neurons from TDP-43 mediated toxicity. "UPF1 has shown promising results in animal models of ALS involving TDP-43 dysfunction and provides a rationale for developing gene-based therapies for ALS, and we are excited about moving this forward with a company that has the scientific insights of MeiraGTx" said Dr. Petsko. MeiraGTx recently entered into a research collaboration with Professor Klein of Louisiana State University (LSU) to conduct pre-clinical animal studies aimed at demonstrating the efficacy of a UPF1-based therapy in animal models of TDP-43 induced ALS pioneered in his laboratory.

MeiraGTx and its academic collaborators are defining the role of RNA metabolism in the pathology of ALS, with the goal of developing effective gene therapies. "We are very excited about our work with Drs. Petsko and Klein, leaders in the field of neurodegeneration, to develop a gene therapy targeting TDP-43 dysfunction in ALS," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx. "We believe that this may lead to not only an important new therapeutic approach, but further investigation of the genetic causes of ALS will provide insight for the development of additional gene therapies to treat this and other devastating neurodegenerative diseases."

About MeiraGTx

MeiraGTx is committed to the development of novel gene therapies to transform the lives of patients suffering from acquired and inherited disorders. The company is developing treatments for ocular diseases, including rare inherited blindness and age-related macular degeneration (AMD). MeiraGTx is also establishing treatments for xerostomia, a frequent and debilitating side effect of radiation treatment used in head and neck cancers, as well as certain neurodegenerative diseases. In addition, MeiraGTx is developing novel gene regulation platforms that promise to transform the way gene therapy can be applied and create new paradigms for biologic therapeutics.

About Weill Cornell Medicine

Weill Cornell Medicine is committed to excellence in patient care, scientific discovery and the education of future physicians in New York City and around the world. The doctors and scientists of Weill Cornell Medicine — faculty from Weill Cornell Medical College, Weill Cornell Graduate School of Medical Sciences, and Weill Cornell Physician Organization — are engaged in world-class clinical care and cutting-edge research that connect patients to the latest treatment innovations and prevention strategies. Located in the heart of the Upper East Side's scientific corridor, Weill Cornell Medicine's powerful network of collaborators extends to its parent university Cornell University; to Qatar, where an international campus offers a U.S. medical degree; and to programs in Tanzania, Haiti, Brazil, Austria and Turkey. Weill Cornell Medicine faculty provide comprehensive patient care at NewYork-Presbyterian/Weill Cornell Medical Center, NewYork-Presbyterian/Lower Manhattan Hospital and NewYork-Presbyterian/Queens. Weill Cornell Medicine is also affiliated with Houston Methodist. For more information, visit weill.cornell.edu. Weill Cornell Medicine's Office of Biopharma Alliances and Research Collaborations' mission is to proactively generate, structure and negotiate translational research alliances with industry in order to advance promising research projects that have commercial potential.

About LSU

LSU Health Shreveport core mission is to heal, to teach and to discover. The Health Sciences Center located in Northwest Louisiana includes a School of Medicine, School of Allied Health and School of Graduate Studies. It provides care to patients throughout the state of Louisiana through its 530 faculty physicians and other healthcare providers, and educates over 1,400 students, residents and fellows. Designated Centers of Excellence include the Feist Weiller Cancer Center (FWCC), the Center for Cardiovascular Diseases and Science, and the Center for Arthritis and Rheumatology. Research is a core part of our institution's mission and the LSUHSC-S research space includes approximately 200,000 sq. ft. of laboratories and offices, as well as facilities including shared equipment in the departments and a designated Research Core Facility. Faculty, staff and students at LSU Health Shreveport are actively engaged in research in a variety of biomedical areas, with concentrations in cancer, cardiovascular sciences, virology and neuroscience.

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