



## **MeiraGTx Expands Neurodegenerative Gene Therapy Pipeline in Alzheimer's and Parkinson's Disease in Collaboration with Weill Cornell Medicine**

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NEW YORK, Dec. 12, 2016 /PRNewswire/ — MeiraGTx, a New York and UK based gene therapy company, announces the expansion of its Neurodegenerative Disease gene therapy pipeline. Research into the causes of neurodegenerative disease has converged upon the central idea that misfolded proteins play a critical role in damaging neuronal function and the ultimate progression to neuronal cell death. The new program, a collaboration with Dr. Greg Petsko of Weill Cornell Medicine, targets neurodegenerative disease by altering neuronal protein processing.

Leveraging seminal work from Dr. Petsko, the Arthur J. Mahon Professor of Neuroscience and Director of its Helen and Robert Appel Alzheimer's Disease Research Institute in the Feil Family Brain and Mind Research Institute at Weill Cornell Medicine, and his colleagues, the new program targets one of the master regulators of protein trafficking, known as retromer. Dr. Petsko and colleagues have developed genetic strategies to reprogram retromer, impacting protein trafficking, processing and aggregation. Using a gene therapy approach, the team aims to modulate retromer function to treat several inherited and age-related neurodegenerative diseases including Alzheimer's and Parkinson's disease.

"We are excited to initiate this important collaboration," said Dr. Petsko. "I believe that using gene therapy approaches to correct trafficking defects in the brains of Alzheimer's patients by increasing retromer pathway function addresses what may be the seminal cellular problem in this – and probably other – neurodegenerative diseases. My colleagues and I think this is an innovative and important approach that holds great promise for the treatment of Alzheimer's disease. While we realize that there is still a long way to go before our ideas can be tested in patients, partnering with MeiraGTx, one of the world's leading companies in the exciting field of gene therapy, puts the translation of this science to the clinic in the best possible hands."

"We are very excited to begin this collaboration with Dr. Petsko and his colleagues. We believe that retromer is one of the most important new targets in the field of Alzheimer's and neurodegenerative disease. Our approaches may lead to effective new therapies for patients with these diseases," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx.

### 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

The doctors and scientists of Weill Cornell Medicine are engaged in world-class clinical care and cutting-edge research that connect patients to the latest treatment innovations and prevention strategies. The mission of the Office of Biopharma Alliances and Research Collaborations at Weill Cornell Medicine is to proactively generate, structure and negotiate translational research alliances with industry to advance promising research projects that have commercial potential. For more information, contact Larry Schlossman at [las2041@med.cornell.edu](mailto:las2041@med.cornell.edu) or at 212-746-6909.

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### Contacts

Investors:

MeiraGTx

[investors@meiragtx.com](mailto:investors@meiragtx.com)

or

Media:

W2O pure

Kelly Boothe, 206-349-3010

[kboothe@w2ogroup.com](mailto:kboothe@w2ogroup.com)