Lysosomal Therapeutics Receives Grant from The Michael J. Fox Foundation to Fund Parkinson's Disease Research

CAMBRIDGE, Mass. — Dec. 5, 2014 — Lysosomal Therapeutics Inc. (LTI), a company leveraging its expertise in lysosomal biology to develop novel small molecules for the treatment of neurodegenerative diseases, announced today that it has received a grant of $230,000 from The Michael J. Fox Foundation for Parkinson's Research. LTI will use the grant to explore whether the glucocerebrosidase (GCase) lysosomal enzyme may not only be used as a feasible target for treatment of Parkinson's disease (PD) patients, but also as a biomarker for both PD diagnosis and to predict response to LTI's drug candidates.

The goal of this *ex vivo* treatment study is two-fold: establish GCase enzyme activity and corresponding alpha-synuclein protein profiles in isolated blood cells of PD patients, and measure response to treatment with LTI's drug compounds. This project is the first part of a large-scale study to investigate GCase activity and synuclein level profiles in different PD patient populations, and to seek to establish GCase activity as a disease marker. This marker will be used for patient selection in future LTI clinical trials. In the long term, this marker could be used to diagnose people with PD risk and potentially to justify pre-emptive treatment with an LTI GCase activator drug.

Read more about this LTI project on The Michael J. Fox Foundation website here.

About the Implications of Lysosomal Storage Disorders on Neurodegenerative Diseases

Lysosomal storage disorders (LSDs) are a group of approximately 60 known genetically inherited diseases characterized by a deficiency of various vital enzymes. All LSDs consist of neurological components, but Gaucher disease (GD) is the most common LSD, occurring when the gene that encodes the lysosomal enzyme glucocerebrosidase (GCase) is mutated and unable to effectively break down its substrate, glucosylceramide. This results in a build-up of lipids in patients' cells, causing serious health issues.

Recent genetic research suggests that GCase mutations may also cause a predisposition to Parkinson's disease (PD). The manifestation of the neurotoxic aggregation of the protein alpha-synuclein, also known as Lewy bodies, is the hallmark symptom of PD. Lysosomal Therapeutics Inc.'s (LTI) initial research shows that restoring lysosomal function in human neurons of GD and PD patients may normalize the otherwise-elevated levels of alpha-synuclein. In addition to its work with GD and PD, LTI is investigating other lysosomal enzyme deficiencies and their respective genetic links to common neurodegenerative diseases.

About Lysosomal Therapeutics Inc.

Lysosomal Therapeutics Inc. (LTI) is dedicated to innovative small-molecule research and development in the field of neurodegeneration, yielding new treatment options for patients with severe neurological diseases. Our strategy leverages the clinically-validated link between lysosome-based genetic disorders and neurodegenerative diseases to establish a unique and effective molecular platform for novel drug discovery. LTI's lead program targets Gaucher-related neurodegeneration, Parkinson's disease and other synucleinopathies. www.lysosomaltx.com

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