Lysosomal Therapeutics Inc. Raises $20 Million in Series A Financing

- LTI received funding for preclinical advancement of its Parkinon's disease therapeutic candidate and development of a biomarker package to guide clinical development.

- LTI appointed Darren Braccia as vice president of business development to lead external collaboration efforts and to aid in the expansion of LTI's platform by exploring known targets with genetic links between lysosomal storage disorders and neurodegenerative diseases.

CAMBRIDGE, Mass. — Feb. 3, 2015 — Lysosomal Therapeutics Inc. (LTI), a company leveraging its expertise in lysosomal biology to develop novel small molecules for use in the treatment of neurodegenerative diseases, announced today it has raised $20 million in Series A financing. This internal round consisted of expanded funding commitments from its existing investors: Atlas Venture, Hatteras Venture Partners, Lilly Ventures, Sanofi-Genzyme BioVentures, Roche Venture Fund, Partners Innovation Fund and several original angel investors, including Orion Equity Partners, LLC, and LTI co-founders Henri Termeer and Bob Carpenter.

The proceeds will fund ongoing compound optimization and preclinical development of a glucocerebrosidase (GCase) lysosomal enzyme activator candidate for the treatment of Parkinson's disease. In addition, the funding will support a biomarker initiative, for which LTI has previously received grant funding from The Michael J. Fox Foundation for Parkinson's Research. This biomarker effort will help LTI select patients for future clinical trials and predict drug response. LTI will also initiate new research programs based on the genetic links that exist between other lysosomal storage disorders and neurodegenerative diseases.

"LTI succeeded in meeting its initial, seed-stage goal – to generate a compelling lead-stage molecule within its GCase program," said Bruce Booth, Ph.D., partner at Atlas Venture, the lead investor of LTI's Series A financing. "With the syndicate's participation in this Series A round, we are not only supporting further development of LTI's breakthrough therapeutic mechanism for the treatment of Parkinson's disease, but we are also enabling LTI to expand beyond Parkinson's disease and build out a platform around additional novel targets implicated in underserved orphan and neurodegenerative disorders."

To help guide LTI's platform expansion and partnering strategy, the company has hired Darren Braccia as vice president of business development. Braccia brings to LTI's team more than 16 years of business and corporate development experience: He has worked in progressive business development roles for private and public companies, including Biogen Idec, Magellan Biosciences and most recently, at Vertex Pharmaceuticals as senior director of business development. Braccia is a graduate of the Kellogg

Graduate School of Management at Northwestern University and holds a bachelor's degree from Washington and Lee University.

"Our work to date has affirmed our theory that rare diseases, like Gaucher disease, can be used as model systems for developing therapeutics for common neurodegenerative disorders, such as Parkinson's disease," said Kees Been, LTI's founding president and chief executive officer. "With the continued support of our investors and through the expansion of our team, we will advance our Parkinson's disease therapeutic candidate to a clinical start and also grow our target-rich biology platform by exploring the known relationships between other lysosomal storage and neurodegenerative disorders."

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. The company is currently developing small molecules that cross the blood-brain barrier and increase GCase activity to potentially treat the root cause – not only the symptoms – of PD. 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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