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Lysosomal Therapeutics Inc. Receives $4.8 Million in Seed Funding

Link between lysosome-based genetic disorders and neurodegenerative diseases to be explored as foundation for new therapeutics

CAMBRIDGE, Mass. — May 12, 2014 — 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 $4.8 million in seed funding. Atlas Venture was the lead investor, with additional participation from Hatteras Venture Partners, Lilly Ventures, Sanofi-Genzyme BioVentures, Roche Venture Fund, Partners Innovation Fund and several angel investors, including Orion Equity Partners, LLC. LTI was founded by Dimitri Krainc, M.D., Ph.D., and former Genzyme executives Henri Termeer, Bob Carpenter and Peter Wirth. Kees Been is the founding president and chief executive officer of the company.

LTI's unique approach to discovering new drugs for neurodegenerative diseases is based on research performed in Krainc's lab at Massachusetts General Hospital (MGH), a founding member of Partners HealthCare and an affiliate of Harvard Medical School. The work completed at MGH by Krainc and Joseph Mazzulli, Ph.D., LTI's scientific co-founder and a former postdoctoral fellow in the lab, builds on the association between Gaucher disease (GD), a rare lysosomal storage disorder (LSD) caused by mutations in the gene for glucocerebrosidase (GCase), and a predisposition to Parkinson's disease (PD). They demonstrated that increasing GCase activity in human neurons of GD and PD patients can normalize lysosomal function and improve neuronal survival. This research continues at Northwestern University, where Krainc is now Ward Professor and chairman of the department of neurology, and Mazzulli is an assistant professor of neurology.

Compounds that enhance GCase activity represent a potential breakthrough opportunity to develop an entirely new class of agents to treat GD and PD. The risk of developing PD in the broader population has also been firmly linked to GCase mutations. This illustrates how rare diseases can be used as model systems for developing therapeutics for common neurodegenerative disorders.

"This inventive approach to treating Parkinson's disease was really facilitated by the success of current treatment strategies, such as enzyme replacement therapy, that have enabled the Gaucher disease patient population to live longer," said Been. "However, an unfortunate consequence of this longevity is the finding that Gaucher patients experience a 20-fold increased risk of developing Parkinson's disease. At LTI, our lead development program is a completely novel approach to treating Parkinson's disease by developing small molecules that can cross the blood-brain barrier to increase the activity of the GCase enzyme in the lysosome and potentially reduce Parkinson's disease risk."

“This seed funding will be instrumental in enabling LTI to progress its GCase program through the lead-generation process and in setting us up for preclinical development,” said Carpenter, who chairs LTI’s board of directors. “I look forward to working with Kees and his team to refine LTI’s GCase modulator platform over the coming months, with an eye toward advancing breakthrough therapeutics for Parkinson’s disease.”

“We are pleased to support LTI through this seed round of funding,” said Bruce Booth, Ph.D., partner at Atlas Venture. “Atlas believes that LTI’s GCase modulator platform holds great promise as a foundation for discovering therapeutics for neurodegenerative diseases like Parkinson’s, through exploring the implications of known relationships between lysosomal storage and neurodegenerative disorders.”

“Our long-term mission is to develop targeted therapies for children and adults with neurodegenerative disorders that will be based on in-depth, mechanistic understanding of these disorders,” added Krainc.

With this financing, new board members will include Booth; Clay Thorp, general partner at Hatteras Venture Partners; Steve Hall, Ph.D., venture partner at Lilly Ventures; Bernard Davitian, vice president and managing director of Sanofi-Genzyme BioVentures; and Carole Nuechterlein, head of Roche Venture Fund. CEO Been also serves as a member of the board of directors. Nessan Bermingham, Ph.D., venture partner at Atlas Venture, and Reza Halse, Ph.D., partner at Partners Innovation Fund, have been appointed as board observers.

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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