BioSpectrum
the business of Bio & Health Sciences
ASIA EDITION

Gain Therapeutics SA announces funding support from a leading Parkinson's Research Foundation

February 26, 2019 | News

Parkinson's disease is a chronic, degenerative neurological disorder with limited treatment options that affects one in 100 people over age 60.



Gain Therapeutics SA, a biotechnology company discovering and developing novel therapeutics to target lysosomal enzymes involved in inborn errors of metabolism and in CNS diseases has announced that it has received funding support from The Michael J. Fox Foundation for Parkinson’s Research (MJFF) and The Silverstein Foundation for Parkinson’s with GBA to advance its proprietary non-competitive molecular chaperones for Parkinson’s disease. This grant has been awarded together with Dr. Marta Martínez Vicente from the Vall d’Hebron Research Institute in Barcelona (Spain).

MJFF and The Silverstein Foundation’s grant support recognizes Gain Therapeutics’ novel therapeutic approach for Parkinson’s with mutations in the glucocerebrosidase (GBA) gene. The program emerges from Gain’s technology platform, which is able to identify and design brain-penetrant allosteric, non-inhibitory pharmacological chaperones for diseases where lysosomal enzyme functions, folding and intracellular trafficking is affected.

“We are honoured to have received this grant from The Michael J. Fox Foundation and The Silverstein Foundation, which recognises the importance of our efforts to develop novel treatment approaches for Parkinson’s disease, which remains an area of significant need for patients, their families and caregivers,” said Dr. Lorenzo Leoni, board representative of Gain Therapeutics SA.

Parkinson's disease is a chronic, degenerative neurological disorder with limited treatment options that affects one in 100 people over age 60. More than 6 million people around the world live with Parkinson's disease, a disorder of the central nervous system that results from the loss of cells in various parts of the brain. Mutations in the glucocerebrosidase (GBA) gene are one of the most common risk factors for Parkinson's disease. GBA encodes a lysosomal enzyme, beta-glucocerebrosidase (GCase). Reduced GCase activity is associated with GBA mutations and has been reported in idiopathic PD, suggesting a more general role for GCase pathway dysfunction in Parkinson's.

“Gain Therapeutics is proud to be part of the joint effort of these foundations to accelerate and develop novel therapeutic interventions to prevent pathogenic mechanisms triggered by GCase-pathway dysfunction,” said Prof. Xavier Barril, CSO of Gain Therapeutics SA.

Said Dr. Liliana Menalled, MJFF senior associate director of Research Programs, “The GBA pathway is a leading target for therapies that aim to slow or stop Parkinson’s progression, and The Michael J. Fox Foundation is committed to supporting innovative treatment approaches in this area. We hope our grant supports successful work that builds evidence for the advancement of these molecular chaperones closer to patient hands.”