Gain Therapeutics, Inc.

Gain Therapeutics is a preclinical biotechnology company focused on developing new medicines to treat rare genetic diseases and neurological diseases caused by protein misfolding, with an initial focus on lysosomal storage disorders including neuronopathic Gaucher disease.

Our supercomputer-driven target-based drug discovery platform, "SEE-Tx™," is a novel approach that uses structural and computational biology to discover previously unidentified allosteric binding sites with high potential for druggability. These sites can be targeted with small molecules, which we call STARs, for therapeutic benefit to correct enzyme misfolding, thereby restoring function, eliminating toxic substrate, and addressing the underlying cause of disease. Our STARS can transform the process of creating life changing treatments in a more efficient method, at fraction of the time, and at a fraction of the cost.

GBA1 (GCase) Enzyme-Related Disorders: Neuronopathic Gaucher Disease (nGD)

We are investigating the restoration of GCase enzymatic function as a treatment for neuronopathic Gaucher disease, a lysosomal storage disorder. Unlike other types of Gaucher disease, none of the existing therapeutics are effective in treating nGD. GCase is an enzyme encoded by the GBA1 gene and found in lysosomes that is needed to breakdown the large molecule glucocerebroside (a component of the cell membrane) into sugar and fat. The misfolding of GCase can lead to accumulation of these substrates to toxic levels in the liver, spleen, bone marrow and nervous system and can result in lysosomal storage and neurodegenerative diseases.

Currently, there is no cure for nGD and there are very limited treatment options. Current treatments such as ERT cannot address central nervous system symptoms because they cannot cross the blood-brain barrier.

We have identified novel STARs targeting GCase and are continuing characterization of our most promising compounds through preclinical studies, where we will further identify optimal lead compounds for each indication that we will advance into clinical trials. Through academic partnerships, co-development and licensing arrangements, we intend to develop a broad pipeline of therapeutics to catalyze a new approach to the treatment of diseases caused by misfolded enzymes.

For more information, please visit https://www.gaintherapeutics.com/