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Small molecule lysosome activator for the treatment of neurodegeneration

PI

- Ph.D., Harvard University, MA
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- President and CEO of Qinotto, Inc.
- Past Leadership positions at Balance Therapeutics, Elan Pharmaceuticals, Johnson and Johnson and McKinsey & Co.

This Phase I trial will examine whether a novel chemical compound, which could enhance the clearance of waste from nerve cells in brain diseases, is safe and tolerable in humans.

Background

Lysosomes are compartments inside brain cells that help degrade and dispose of a cell’s waste. Studies have shown that lysosomes may often be damaged in brain diseases. When lysosome activity becomes damaged, nerve cells in the brain may lose their ability to clear waste effectively and this inability could be associated with the accumulation of beta-amyloid, alpha synuclein and other proteins. These proteins accumulate to form plaques and Lewy bodies, which are hallmark brain changes observed in Alzheimer’s and Parkinson’s disease, respectively.

STUDY

- CADRO category: Translational Research & Clinical Interventions

Researchers at Stanford University in the laboratory of Dr. Tony Wyss-Coray developed a novel chemical compound QIN-100. The research team administered QIN-100 to nerve cells in a laboratory dish and found that the compound may enhance the activity of lysosomes and protect them from damage. Follow up studies in a genetically engineered Parkinson’s-like mouse model showed that QIN-100 could reduce inflammation, improve nerve cell survival and enhance cognition and motor function (use of muscles to perform tasks like walking, balancing etc. which is impacted in Parkinson’s).

Research Plan

Building on these preliminary research results, Dr. Lien and colleagues will conduct a Phase 1 clinical trial in cognitively unimpaired individuals between 18-55 years old to evaluate the safety and tolerability of this compound in humans. Participants in this study will receive a placebo (not the actual drug but an inactive substance that has no risk for the participant) or varying doses of QIN-100. Dr. Lien’s team will use results from this study to guide next steps in this compound’s development, including selection of appropriate

QIN-100 doses for future clinical studies in individuals with Alzheimer's and Parkinson's disease.

Impact

If successful, the study results may give rise to larger clinical trials and pave the way for a potential therapeutic approach that rescues and stimulates lysosome activity to improve the survival of nerve cells in brain diseases including Alzheimer's.

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