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M3 Biotechnology, Inc.
Seattle, Washington

2018 The Part the Cloud to RESCUE Brain Cell Degeneration in Alzheimer's Disease-
\$1,000,000

Phase 2a Alzheimer's Trials of a Novel Neurotrophic Activator, NDX-1017

This is a Phase 2a trial that explores if a drug that stimulates brain cell growth and survival could be a novel therapy for Alzheimer's.

PI

- Ph.D. in Biomedical Engineering, UCLA, 2008
- Founding Faculty Member, Imaging Genetics Center at University of Southern California

STUDY

- CADRO category: Translational Research & Clinical Interventions
- This is Dr. Hua's first Association award.

Background

Many current studies in Alzheimer's drug therapy focus on removing or blocking the formation of beta-amyloid plaques and tau tangles, the two main hallmarks of Alzheimer's hypothesized to lead to brain cell damage and cell death. Dr. Xue Hua aims to combat brain cell damage in Alzheimer's using a different approach, by modulating proteins in the brain capable of stimulating brain cell growth and survival. A special factor important for this process is a powerful molecule known as Hepatocyte growth factor (HGF). Activity of the HGF system is reduced in Alzheimer's and therefore presents an opportunity for drug therapy.

In studies with genetically engineered Alzheimer's-like mice, a drug candidate called NDX-1017 has been shown to activate the HGF system. By stimulating this special protein in the mice, the drug NDX-1017 not only promoted the survival of nerve cells, but also enhanced cell-to-cell communication, improved memory and other forms of cognition. Based on these findings, Dr. Hua believes that the drug may have beneficial effects in human Alzheimer's and clinical testing of NDX-1017 is now underway. Recent findings from a Phase 1 clinical trial conducted by Dr. Hua and colleagues demonstrated that the drug is safe and well tolerated in healthy human volunteers.

Research Plan

Dr. Hua and colleagues will conduct a larger Phase 2a clinical trial of NDX-1017 with human participants who have early-stage Alzheimer's. The researchers will administer NDX-1017 treatment for three months to each participant, determining whether the treatment is safe and how the drug may affect brain cell health and activity. Additionally, Dr. Hua will examine if the treatment can modify beta-amyloid and tau levels and whether it promotes cognitive functions such as attention and memory.

Impact

If successful, the results of this study could provide vital information for a larger Phase 3 clinical trial, which would more thoroughly assess the drug's effectiveness. Since NDX-1017 is a relatively inexpensive drug to produce, it could offer a safe and cost-efficient treatment for modifying the progression of Alzheimer's.