Testing Gene Therapy to Improve Brain Function in Alzheimer's Disease Patients

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Researchers at the Shiley-Marcos Alzheimer's Disease Research Center at the University of California San Diego are about to launch a Phase 2 clinical trial to test a gene therapy treatment for Alzheimer's disease (AD) called CERE-110. Previously, CERE-110 was carefully studied in animals as well as in a small study to assess safety in humans. These studies showed that CERE-110 can safely induce long-term production of a natural brain cell-survival molecule called Nerve Growth Factor (NGF) in the brains of AD patients.

Researchers at 12 U.S. sites, including the Shiley-Marcos Alzheimer's Disease Research Center at the University of California San Diego are now seeking 50 study participants with mild to moderate AD for the Phase 2 clinical study. The experimental treatment utilizes viral-based gene transfer. Brain cells that receive gene transfer will, in turn, make NGF, a naturally occurring protein that maintains nerve cell function and survival in the brain.

"NGF is known to support the survival and function of the neurons that deteriorate in Alzheimer's disease," said Michael Rafii, MD, PhD, assistant professor in the UC San Diego Department of Neurosciences. "These neurons produce a chemical called acetylcholine, which is important in memory and cognitive function. The hope is that restoration of this system's function may improve memory in Alzheimer's patients." AD is a degenerative and ultimately fatal disorder affecting as many as five million Americans. That number is expected to soar to more than 11 million by 2040.

In the trial, a neurosurgeon will inject CERE-110 directly into the nucleus basalis of Meynert (NBM) of the brain, an area where neuron death occurs in AD. The brain cells contained in this nucleus, located deep within the brain, form the cholinergic system. The cholinergic system uses acetylcholine in transmitting nerve impulses involved in memory and cognition, and these cells are known to profoundly degenerate in the course of Alzheimer's disease. Researchers hope that exposing these cells to NGF may preserve function and prevent further cell loss and potentially slow intellectual decline seen in Alzheimer's patients.

Previous Research

A Phase 1 study was conducted at Rush University in Chicago and the University of California San Diego, where the treatment was found to be generally safe and well-tolerated. The 10 subjects with AD underwent cognitive testing, measures of activities of daily living, and MRI and PET (positron emission tomography) scans. When compared to other severity-matched individuals with AD, researchers observed increases in brain metabolism in several cortical regions of the brain in some of the trial participants at six months and 12 months, suggesting a potential reversal of patterns typically observed in AD. In addition, in follow up ranging from six months to more than four years post-treatment, there have been no side effects thought to be caused by CERE-110.

In previous animal studies, CERE-110 reversed brain degeneration in aged monkeys and deficits in rats with brain defects similar to those seen in AD.

The Phase 2 Clinical Study

In this blinded study, all eligible participants will be randomized by chance to one of two treatment groups: half will receive CERE-110 and half will undergo a placebo surgery. Once the study is completed, if the results are promising, participants in the placebo group will be eligible to be treated with CERE-110.

The NGF study is being conducted by the Alzheimer's Disease Cooperative Study, a nationwide consortium of research centers and clinics supported by the National Institute on Aging, part of the National Institutes of Health, and coordinated by the UC San Diego School of Medicine. Ceregene, Inc., the study sponsor, is a San Diego-based biotechnology company focused on the delivery of nervous system growth factors via gene transfer for the treatment of neurodegenerative disorders. Ceregene is providing CERE-110 for the study.

To learn how to participate, contact Christina Gigliotti, PhD. For more information:

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