

Researchers to test first gene therapy For Alzheimer's patients

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Mount Sinai School of Medicine is one of 12 sites nationwide participating in the first Phase 2 clinical trial to test gene therapy treatment for Alzheimer's disease. The study is the first multicenter neurosurgical intervention in Alzheimer's research in the U.S.

The experimental treatment utilizes a viral-based gene transfer system, CERE-110, that makes Nerve Growth Factor (NGF), a naturally occurring protein that helps maintain nerve cell survival in the brain. CERE-110 has been previously studied in animals, where it reversed brain degeneration in aged monkeys and rats. For this study, CERE-110, will be injected by a neurosurgeon directly into the nucleus basalis of Meynert (NBM) of the brain, an area where neuronal death occurs in Alzheimer's patients.

In animal studies, NGF has been shown to support the survival and function of the neurons that deteriorate in Alzheimer's patients. These neurons produce the chemical acetylcholine, which is important in [memory](#) and cognitive function. The hope is that improvement of this system's function may lead to better memory performance in Alzheimer's patients.

A Phase 1 study in Alzheimer's patients has been conducted at Rush University in Chicago and the University of California San Diego, where researchers observed increases in brain metabolism in several cortical regions of the [brain](#) at 6- and 12-month follow-up in some of the participants. With 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.

Participants in the Phase 2 study will be randomly placed into one of two treatment groups, with half receiving CERE-110 via neurosurgery and half receiving placebo surgery without any cranial injections. Once the study is completed, and if the results are promising, participants in the [placebo](#) group will be eligible to be treated with CERE-110. All participants will receive a thorough medical examination and cognitive testing. In addition, participants will be closely monitored by a team of physicians for the duration of the two-year study. Participants will also be encouraged to participate in long-term follow-up.

Source: The Mount Sinai Hospital

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