MedicalXpress

Historic gene therapy trial to treat Alzheimer's disease underway at Georgetown

September 22 2009

Researchers in the Memory Disorders Program at Georgetown University Medical Center are now recruiting volunteers for a national gene therapy trial - the first study of its kind for the treatment of patients with dementia due to Alzheimer's disease.

The phase II study examines the safety and possible benefits of CERE-110. CERE-110 contains a gene and is injected during surgery into a part of the brain affected by Alzheimer's disease. The gene will instruct brain cells to produce more of a protein, called Nerve Growth Factor or NGF, which helps nerve cells survive and function properly. The transfer of this gene into the brain is a medical technique called gene therapy.

"Our goal is to stop the progression of Alzheimer's disease," explains R. Scott Turner, MD, PhD, director of Georgetown's Memory Disorders Program. "This is our first study of a gene therapy injected into brain, and thus the trial requires close collaboration with our neurosurgery colleagues at GUMC, in particular Dr. Chris Kalhorn."

Turner says Kalhorn, an associate professor of the department of neurosurgery at Georgetown University Hospital, routinely performs neurosurgical procedures similar to the one being utilized in this study.

About 50 people with Alzheimer's disease will participate in this study at fewer than 10 hospitals nationwide. Only persons with a mild form of Alzheimer's Disease, who are evaluated and deemed competent to

consent for themselves, will be permitted to participate in the study. The study requires each patient select a study partner for the length of the study. All patients in the study will undergo surgery to drill two small holes in the skull. Only those patients randomly assigned to receive CERE-110 will have the gene therapy injected into the brain. Those subjects randomized to the placebo group will not have the gene therapy injected.

This study is a phase II, double-blind, placebo-controlled study.

Phase II means the investigational agent has been studied in a small number of patients and this study is being conducted to determine its safety and possible benefits.

Double-blind means that the patients, clinical coordinators and treating physicians will not know if the patient received the investigational agent until the end of the study. Only the neurosurgeon and operating team delivering the gene therapy will know if the patient received the active agent.

Placebo-controlled means that patients will be selected randomly to either receive the active agent or not, but all patients will undergo surgery. This study has been approved by the FDA and the Institutional Review Board at GUMC.

More information: To learn more about this or other studies, contact Georgetown's Memory Disorders Program at 202-784-6671 or visit the website at memory.georgetown.edu.

Source: Georgetown University Medical Center (news : web)