Research study explores gene therapy treatment to reduce symptoms of Parkinson's disease

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Physicians at Rush University Medical Center are testing a unique gene therapy product called CERE-120 to evaluate if its use can improve the symptoms of Parkinson's disease. Rush is one of 11 sites in the U.S. and the only site in Illinois enrolling patients into the new, double-blinded trial.

CERE-120 is an experimental gene transfer drug being developed by Ceregene, Inc. It contains the human gene for neuturin, a naturally occurring protein also known as a neurotrophic factor. Neurosurgery is used to deliver the neuturin directly to degenerating or dying dopamine neurons in the brain. In earlier studies, neuturin has shown to improve function and repair brain cells that degenerate in Parkinson's disease.

"This gene therapy has the potential to improve the symptoms of Parkinson's disease while also delaying further disease progression," said Dr. Christopher Goetz, director of the Parkinson's Disease and Movement Disorders program at Rush University Medical Center and site principal investigator of the study. "Patients with Parkinson's disease urgently need therapeutic approaches that not only improve symptoms and function, but also to have the ability to favorably modify the underlying disease itself."

Three previous trials demonstrated that CERE-120 was safe in 50 Parkinson's disease patients who were followed for five years. Rush

neurological researchers have been involved in all three trials.

"The preliminary data in the Phase I study are encouraging, and the first Phase II trial showed improvement in patients who were assessed under blinded conditions at 15-18 months post treatment," said Goetz.

The new Phase IIb trial will test the efficacy of CERE-120 by delivering an increased dose of the gene therapy to two key areas of the brain called the substantia nigra and the putamen that are damaged by Parkinson's disease. The goal of this new approach is to assure wider distribution of neurturin and increase the likelihood of repairing and protecting brain cells from further degeneration due to Parkinson's disease.

Half of study participants will undergo surgery to receive a dosing of CERE-120. The other half will undergo a placebo surgery. A comparison between these two groups will help distinguish the effects of CERE-120 compared to those that receive placebo

If the study results demonstrate that CERE-120 administration is safe and beneficial, subjects who receive placebo surgery will have the option to have a second surgery to receive a dosing of CERE-120.

Provided by Rush University Medical Center