

# PET scans show gene therapy normalizes brain function in Parkinson's patients

November 19 2007

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Brain scans used to track changes in a dozen patients who received an experimental gene therapy show that the treatment normalizes brain function - and the effects are still present a year later.

Andrew Feigin, MD, and David Eidelberg, MD, of The Feinstein Institute for Medical Research collaborated with Michael Kaplitt, MD, of Weill Cornell Medical Center in Manhattan and others to deliver genes for glutamic acid decarboxylase (or GAD) into the subthalamic nucleus of the brain in Parkinson's patients. The study was designed as a phase I safety study, and the genes were delivered to only one side of the brain to reduce risk and to better assess the treatment.

A recently published study included the clinical results of the novel gene therapy trial, but this new report from the same study focuses on the power of modern brain scans to show that the gene therapy altered brain activity in a favorable way. This latest study is published this week in the *Proceedings of the National Academy of Sciences*.

The patients only received the viral vector-carrying genes to the side of the brain that controls movement on the side of their body most affected by the disease. It was a so-called open-label study -- everybody received the gene therapy so the scientists knew that there could be a placebo effect. That is why brain scans were so critical to the experiment. Dr. Eidelberg and his colleagues pioneered the technology and used it to identify brain networks in Parkinson's disease and a number of other neurological disorders.

In Parkinson's, they identified two discrete brain networks -- one that regulates movement and another that affects cognition. The results from the brain scan study on the gene therapy patients show that only the motor networks were altered by the therapy. "This is good news," said Dr. Eidelberg, the senior investigator of the study. "You want to be sure that the treatment doesn't make things worse." The gene makes an inhibitory chemical called GABA that turns down the activity in a key node of the Parkinson's motor network. The investigators were not expecting to see changes in cognition, and the scans confirmed that this did not occur.

Position emission tomography (PET) scans were performed before the surgery and repeated six months later and then again one year after the surgery. The motor network on the untreated side of the body got worse, and the treated side got better. The level of improvements in the motor network correlated with increased clinical ratings of patient disability, added Dr. Feigin.

"Having this information from a PET scan allows us to know that what we are seeing is real," Dr. Eidelberg added. The scans also detected differences in responses between dose groups, with the highest gene therapy dose demonstrating a longer-lasting effect. "This study demonstrates that PET scanning can be a valuable marker in testing novel therapies for Parkinson's disease," he said.

The gene therapy technique was developed by Neurologix Inc., a New Jersey-based company. Scientists are now working on a design for a phase 2 blinded study that would include a larger number of patients to test the effectiveness of the treatment.

Source: North Shore-Long Island Jewish (LIJ) Health System

Citation: PET scans show gene therapy normalizes brain function in Parkinson's patients (2007, November 19) retrieved 2 May 2024 from <https://medicalxpress.com/news/2007-11-pet-scans-gene-therapy-brain.html>

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