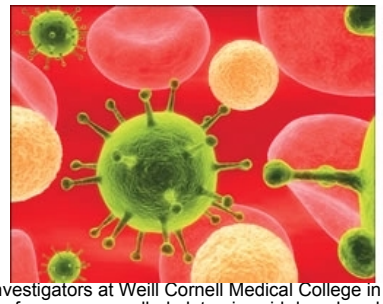
Treating Parkinson’s with Gene Therapy

By: NEAL HERMANOWICZ, MD MEDICAL DIRECTOR PHILLIP AND CAROL TRAUB PARKINSON’S CENTER EISENHOWER MEDICAL CENTER



In a June 23, 2007 article in the medical journal The Lancet, investigators at Weill Cornell Medical College in New York City described the results of an early phase study of altering brain chemistry by instructing brain cells to make more of an enzyme called glutamic acid decarboxylase (GAD). Investigators injected viruses containing the genetic code for making GAD, which in turn, is responsible for making a naturally occurring brain chemical called gamma-aminobutyric acid (GABA). They believe more GABA in certain parts of the brain could benefit Parkinson’s patients.

Viruses can be thought of as tiny envelopes—a delivery method for whatever genetic material they contain. Once inside the body, viruses attach themselves to a target, and then inject their genetic material into the target. Viruses that cause the common cold, for example, attach themselves to cells that line the nasal passages, and then inject these tissues with their genes. The virus’ genes redirect the function of the cell in an abnormal way and symptoms of a cold ensue—a raw throat, nasal congestion and a runny nose.

Authors of the Cornell study removed genes that might make people sick from their viruses and replaced them with genes that instruct brain cells to make more GABA, which affects dopamine in the brain. Although GABA will not cure Parkinson’s, investigators hope that increasing the amount of GABA in the brain will reduce the symptoms of Parkinson’s disease.

Investigators injected these viruses into the brain via a long, thin tube, which was carefully directed to a specific location in the brain called the putame. Then, a solution containing the viruses was injected. The Cornell investigators indicated this method appears to be safe, at least in a very short-term assessment. Although the authors have not yet proven the technique works, they hope to investigate further by conducting more trials.

A similar study is now underway at the University of California, San Francisco (UCSF) in collaboration with scientists at Rush University in Chicago. This group is also using viruses with new genetic material that are injected into the brain, but instead of GAD, the gene provides instructions for Neurturin, a nervous system growth factor.

Both the Cornell and UCSF studies reported positive results. The studies were conducted in an open label fashion — both the doctors and the patients knew they were receiving the treatment. To truly scrutinize whether a treatment is working, scientists usually rely upon studies that are placebo-controlled (some of the subjects enrolled in the study receive a placebo rather than the actual treatment) and “blinded” (neither the patients nor the doctors know who receives the treatment and who receives a placebo). For most scientists and physicians, this is a definitive way to measure if a treatment is working.

The studies of GAD and Neurturin are in the very early stages of development. Currently, it has not been determined which is better, or if in fact, either one will truly work, but the techniques seem safe enough to proceed cautiously. However, several years of testing remain to determine the true safety and efficacy of the studies.

EVENT PD in Motion Tuesday/Thursdays, 9 to 10 a.m. Lisa Manning, CET $40 per month Annenberg Center for Health Sciences 760-773-1480

Healthy Living is a publication of Eisenhower Medical Center · © Copyright 2014 All Rights Reserved · www.emc.org