

Gene therapy for Parkinson's symptoms shows promise

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(HealthDay)—A new gene therapy might help improve motor symptoms

in people with Parkinson's disease who aren't responding to other therapies, an early study has found.

"This is not a cure of Parkinson's disease," said James Beck, chief scientific officer of the Parkinson's Foundation. "This is a potentially good [treatment](#) for symptom control. It provides an additional way of providing dopamine to the [brain](#), but it doesn't stop the progression of Parkinson's disease."

The new treatment uses a virus to deliver gene [therapy](#) to a targeted area of the brain. The gene therapy affects an enzyme called AADC. This enzyme transforms levodopa into dopamine in the brain.

Cells that make the neurotransmitter dopamine—a chemical messenger in the brain—die off in Parkinson's disease, according to the U.S. National Institute on Aging. A loss of dopamine causes the symptoms of Parkinson's disease, such as tremor and slow movements.

Standard treatments attempt to replace the lost dopamine. For example, one current medication is levodopa, but the cells that transform levodopa into dopamine have to be functioning for this treatment to work. As the dopamine-producing cells die off, it becomes harder and harder for the brain to respond to medications like this, Beck explained.

And, that's where the new gene treatment may help.

Led by Dr. Chadwick Christine, at the University of California, San Francisco, researchers used MRI scanning to locate the right area of the brain. Then they infused the new gene therapy into a targeted area of the brain called the putamen. The study team chose this area because these brain cells aren't destroyed by Parkinson's disease.

The phase 1 trial included 15 people who were no longer responding to

other Parkinson's treatments. They all received one infusion of the gene therapy—known as VY-AADC.

After the treatment, researchers followed the patients' health for up to 36 months and found that the treatment was well-tolerated. The most serious side effects—a blood clot and an irregular heart rhythm caused by the blood clot—were related to the surgery used to deliver the treatment, and not the treatment itself, the researchers said.

The therapy also showed a meaningful improvement in the time people spent without movement symptoms each day. And the effect appeared to be lasting, with some of the patients followed for as long as three years.

Dr. Alessandro Di Rocco, director of the Movement Disorders Program at Northwell Health in Great Neck, N.Y., reviewed the findings.

"This study seems to be a step forward in perfecting the [gene therapy] vector and they were able to give it safely," he said.

Di Rocco added that the decrease in symptoms "is a real effect."

But, like Beck, he cautioned that this is an early study with only a small number of people in the trial. Di Rocco also noted that the trial wasn't "blinded," so it's possible there was a placebo effect for some.

Both Di Rocco and Beck also expressed concern about the possible cost of such therapy.

The University of California, San Francisco, and University of Pittsburgh Medical Center researchers aren't the only team working on gene therapy for Parkinson's symptoms. A group of British researchers is actively recruiting Parkinson's patients to take part in a [gene therapy](#) trial that's also aimed at increasing the availability of [dopamine](#) in the

brain.

Their trial will look at up to 30 patients receiving treatment in London or Paris. For this study, researchers are targeting a part of the brain called the striatum.

Results of the American study were presented Sunday at the American Neurological Association meeting, in Atlanta. Findings presented at meetings should be viewed as preliminary until they've been published in a peer-reviewed journal.

More information: James Beck, Ph.D., chief scientific officer, Parkinson's Foundation; Alessandro Di Rocco, M.D., director, Movement Disorders Program, Northwell Health, Great Neck, N.Y., and professor of neurology, Zucker School of Medicine at Hofstra University; Oct. 21, 2018, presentation, American Neurological Association meeting, Atlanta

Learn more about Parkinson's disease treatments from the [Parkinson's Foundation](#).

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