

New study shows long-term safety of gene therapy in Parkinson's disease

July 27 2016



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New safety data from a study of patients with advanced Parkinson's disease five years after gene transfer-mediated delivery of the neuroprotective factor neurturin directly to patients' brains reveal no serious adverse events related to the treatment. The encouraging long-term safety profile of the surgically administered adeno-associated virus (AAV2)-neurturin gene therapy is described in an article in *Human Gene Therapy*.

In the article "Long-Term Safety of Patients with Parkinson's Disease

Receiving rAAV2-Neurturin (CERE-120) Gene Transfer" William Marks, Jr., University of California, San Francisco, Tiffany Baumann, Isis Pharmaceuticals (Carlsbad, CA), and Raymand Bartus, RTBioconsultants (San Diego, CA), representing the CERE-120 Study Group, describe the study design, which greatly extended the length of follow-up of the 53 [patients](#) evaluated compared to previous studies for the purpose of identifying any safety issues that might present months or years after the treatment.

"The longevity of rAAV vector expression makes it particularly useful for treatment of chronic neurodegenerative disorders," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "These long-term data lend support to the evidence that rAAV will prove to be an important platform for treating this highly debilitating disease."

More information: William J. Marks et al, Long-Term Safety of Patients with Parkinson's Disease Receiving rAAV2-Neurturin (CERE-120) Gene Transfer, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2015.134](#)

Provided by Mary Ann Liebert, Inc

Citation: New study shows long-term safety of gene therapy in Parkinson's disease (2016, July 27) retrieved 4 May 2024 from <https://medicalxpress.com/news/2016-07-long-term-safety-gene-therapy-parkinson.html>

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