

Brain Neurotherapy Bio, Inc. Announces Treatment of First Patient in New GDNF Gene Therapy Trial for Parkinson's Disease

First Open Label GDNF Gene Therapy Trial for Early and Later Stages of Parkinson's Disease

COLUMBUS, OHIO and OAKLAND, CALIFORNIA, [August 25, 2020](#) - - Brain Neurotherapy Bio, Inc. (BNB), a clinical stage biotechnology company focused on advancing central nervous system gene therapies for neurological disorders, today announced treatment of the first Parkinson's disease patient in a Phase 1b glial cell line-derived neurotrophic factor (GDNF) gene therapy study. The patient treated at The Ohio State University Wexner Medical Center is participating in a BNB sponsored clinical study evaluating GDNF gene therapy in participants with early to moderate stages of Parkinson's disease ([NCT04167540](#)). This clinical safety study is supported by the California Institute for Regenerative Medicine. The one-time treatment involves infusion of a gene therapy solution into deep structures of the brain that are affected by Parkinson's disease. With lessons learned from over three decades of neurotrophic factor development, BNB's protocol utilized novel intraoperative magnetic resonance imaging (iMRI) technologies to precisely target GDNF gene therapy to a collection of nerve cells in a region called the putamen.

By enhancing levels of a naturally occurring growth factor, GDNF gene therapy is intended to promote the survival and functioning of vulnerable brain cells that degenerate in Parkinson's disease. Harnessing the brain's own cellular machinery, GDNF gene therapy provides continuous production of GDNF that may provide an advantage over intermittent protein infusions of synthetic GDNF where levels may be subtherapeutic between infusions.

"I've been investigating therapeutic gene therapy approaches for Parkinson's disease for nearly 30 years, and today marks a significant milestone for the field and may lead to major therapeutic opportunities for those suffering with this devastating condition, along with their families," said Krystof S. Bankiewicz, MD, PhD, Chief Executive Officer of BNB. "We thank the current and future participating patients and the Neurology and Neurosurgery teams conducting the clinical study at The Ohio State University, the University of California San Francisco and the University of California Irvine medical centers."

"There is an urgent need in the clinic for a treatment that may alter course of disease progression for patients with Parkinson's disease," said Amber Van Laar, MD, movement disorder neurologist and Senior Director for Clinical Affairs of BNB. "GDNF gene therapy, if proven safe, is one such therapeutic that may mitigate and possibly reverse such clinical features of Parkinson's disease," added Adrian Kells, PhD, BNB's Chief Operating Officer.

About GDNF Gene Therapy

GDNF gene therapy consists of a delivery particle, made up of the outer shell structure of the non-pathogenic adeno-associated virus serotype 2 (AAV2), that allows attachment to specific types of brain cells, and transfer of its genetic payload. The viral particle and DNA payload together are designated by the specific acronym AAV2-GDNF and make up the unique therapeutic being developed by BNB. The AAV2-GDNF targets brain nerve cells within the

specific target, inducing them to produce and release GDNF protein. The GDNF gene payload consists of a human GDNF DNA sequence that is attached to additional regulatory sequences that provide long-term stability of the transferred DNA and continuous production of GDNF protein by the recipient nerve cell.

Direct brain delivery of the AAV2-GDNF therapeutic agent is carried out using customized neurosurgical methods and technologies, featuring small openings on each side of the scalp and skull, stereotactic guidance and passage of a delivery cannula into the brain target, and a convection-enhanced delivery (CED) infusion using iMRI guidance and monitoring. Real-time MR imaging during surgery allows for the distribution of the AAV2-GDNF therapeutic agent to be tailored to each individual patient for optimal administration to precise brain regions.

Additional clinical study details are available at <https://clinicaltrials.gov/show/NCT04167540>
Or by directly contacting study coordinators at the clinical sites:

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About Parkinson's Disease

Affecting more than 10 million individuals worldwide and 1 million in the United States (US), Parkinson's disease is the most common neurodegenerative movement disorder in the world. In the US, there are approximately 60,000 new Parkinson's disease diagnoses per year. As many as 90% of Parkinson's disease cases have no known specific cause, while the remainder show genetic inheritance. Only 4% of PD diagnoses are made prior to age 50. The vast majority of subjects are diagnosed between ages 60 – 70 years, and incidence increases with age. The cardinal features of the disease include resting tremor, rigidity, slowness of movement, and postural instability. There are a variety of clinical features that also commonly occur prior to the cardinal features, including but not limited to sleep disturbances, constipation, and behavioral abnormalities. The primary neuropathology is degenerative cell loss in the nigrostriatal dopaminergic pathways connecting the substantia nigra to the putamen.

About Brain Neurotherapy Bio, Inc

BNB is privately-owned clinical biotechnology company with operations in Oakland CA and Columbus OH. www.brainneubio.com

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