

CIRM Funded Trial for Parkinson's Treats First Patient

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Oakland, CA – Brain Neurotherapy Bio, Inc. (BNB) is pleased to announce the treatment of the first patient in its Parkinson's gene therapy study. The CIRM-funded study, led by Dr. Krystof Bankiewicz, is one of the 64 clinical trials funded by the California state agency to date.

Parkinson's is a neurodegenerative movement disorder that affects one million people in the U.S alone and leads to shaking, stiffness, and problems with walking, balance, and coordination. It is caused by the breakdown and death of dopaminergic neurons, special nerve cells in the brain responsible for the production of dopamine, a chemical messenger that is crucial for normal brain activity.

The patient was treated at The Ohio State University Wexner Medical Center with a gene therapy designed to promote the production of a protein called GDNF, which is best known for its ability to protect dopaminergic neurons, the kind of cell damaged by Parkinson's. The treatment seeks to increase dopamine production in the brain, alleviating Parkinson's symptoms and potentially slowing down the disease progress.

"We are pleased to support this multi-institution California collaboration with Ohio State to take a novel first-in-human gene therapy into a clinical trial for Parkinson's Disease." says Maria T. Millan, M.D., President and CEO of CIRM. "This is the culmination of years of scientific research by the Bankiewicz team to improve upon previous attempts to translate the potential therapeutic effect of GDNF to the neurons damaged in the disease. We join the Parkinson's community in following the outcome of this vital research opportunity."

CIRM Board Member and patient advocate David Higgins, Ph.D. is also excited about this latest development. For Dr. Higgins, advocating for Parkinson's is a very personal journey since he, his grandmother, and his uncle were diagnosed with the disease.

"Our best chance for developing better treatments for Parkinson's is to test as many logical approaches as possible. CIRM encourages out-of-the-box thinking by providing funding for novel approaches. The Parkinson's community is a-buzz with excitement about the GDNF approach and looks to CIRM to identify, fund, and promote these kinds of programs."

In a news release Dr. Sandra Kostyk, director of the Movement Disorders Division at Ohio State Wexner Medical Center said this approach involves infusing a gene therapy solution deep into a part of the brain affected by Parkinson's: "This is a onetime treatment strategy that could have ongoing lifelong benefits. Though it's hoped that this treatment will slow disease progression, we don't expect this strategy to completely stop or cure all aspects of the disease. We're cautiously optimistic as this research effort moves forward."

Other trial sites located in California that are currently recruiting patients are the University of California, Irvine (UCI) and the University of California, San Francisco (UCSF). Specifically, the Irvine trial site is using the UCI Alpha Stem Cell Clinic, one of five leading medical centers throughout California that make up the CIRM Alpha Stem Cell Clinic (ASSC) Network. The ASSC Network specializes in the delivery of stem cell therapies by providing world-class, state of the art infrastructure to support clinical research.

For more information on the trial and enrollment eligibility, you can directly contact the study coordinators by email at the trial sites listed:

1. **The Ohio State University:** OSUgenetherapyresearch@osumc.edu
2. **University of California, San Francisco:** GDNF@ucsf.edu
3. **University of California, Irvine:** chewbc@hs.uci.edu

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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