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**A Phase 1b Safety Study for MRI guided delivery of AAV2-GDNF for the treatment of Parkinson's disease**

**Grant Award Details**

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A Phase 1b Safety Study for MRI guided delivery of AAV2-GDNF for the treatment of Parkinson's disease

**Grant Type:** Clinical Trial Stage Projects

**Grant Number:** CLIN2-11661

**Project Objective:** To assess the safety and preliminary clinical effect of AAV2-GDNF delivered to the putamen in patients with Parkinson's Disease

**Investigator:**

<b>Name:</b>	Krystof Bankiewicz
<b>Institution:</b>	Brain Neurotherapy Bio
<b>Type:</b>	PI

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**Disease Focus:** Neurological Disorders, Parkinson's Disease

**Human Stem Cell Use:** Vital Research Opportunity

**Award Value:** \$5,510,462

**Status:** Active

**Grant Application Details**

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**Application Title:** A Phase 1b Safety Study for MRI guided delivery of AAV2-GDNF for the treatment of Parkinson's disease

**Public Abstract:****Therapeutic Candidate or Device**

AAV2-GDNF is a gene therapy product encoding Glial cell line-Derived Neurotrophic Factor (GDNF)

**Indication**

Parkinson's disease

**Therapeutic Mechanism**

AAV2-GDNF will be delivered into the putamen. GDNF is a growth factor expected to act by stimulating regeneration of the terminals of dopamine producing neurons that are progressively lost in PD. This is expected to result in an increase in dopamine production leading to improved motor and non-motor functions.

**Unmet Medical Need**

Current therapies such as L-DOPA and Deep Brain Stimulation help to alleviate the symptoms, but the loss of dopamine producing neurons continues, so they are progressively less effective. AAV2-GDNF is a disease-modifying approach, expected to slow and/or halt the progression of PD.

**Project Objective**

Phase 1b trial completed

**Major Proposed Activities**

- Activation of California clinical site for recruitment and treatment of study subjects
- Patient enrolment, randomization and dosing and completion of 18-month primary follow-up post-surgery.
- Manufacturing of AAV2-GDNF for Phase 2/3 clinical studies, and drug comparability studies.

**Statement of Benefit to California:**

The combined direct and indirect cost associated with PD, including treatments, social security payments, and loss of income is well over \$25 billion/yr in the US with an expected increase of 60,000 patients per year. Given that California is the most populous state in the US, and has an ageing population, AAV2-GDNF therapy could hugely lower the socioeconomic consequences on its citizens.

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**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/phase-1b-safety-study-mri-guided-delivery-aav2-gdnf-treatment-parkinson%E2%80%99s>