The Disease Mechanisms and Targeted Interventions of Parkinsonism Gene Therapy

Ayse Keskus[1*] **and Yongmei Zhao**[2*]

[1]*Department of Pathology, University of Hanoi, Hanoi, Vietnam*

[2]*Department of Medicine, University of Can Tho, Can Tho, Vietnam*

Corresponding author:

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Description

Parkinsonism, encompassing various neurodegenerative disorders, presents significant challenges to patients and medical professionals alike. Characterized by motor symptoms such as tremors, rigidity and bradykinesia, Parkinsonism profoundly impacts individuals' quality of life. While traditional treatments such as medication and Deep Brain Stimulation (DBS) have shown efficacy in managing symptoms, they often come with limitations and side effects. However, recent advancements in gene therapy offer an assuring avenue for more targeted and potentially transformative treatments for Parkinsonism.

Gene therapy involves the delivery of genetic material to cells to correct or modulate dysfunctional genes. In Parkinsonism, gene therapy primarily targets the malfunctioning neurons responsible for dopamine production in the brain. Dopamine deficiency lies at the core of Parkinson's disease, the most prevalent form of Parkinsonism, leading to motor impairments and cognitive decline.

One of the pioneering approaches in Parkinsonism gene therapy involves the use of viral vectors to deliver therapeutic genes into the brain. Adeno Associated Viruses (AAVs) are commonly employed due to their ability to efficiently transfer genetic material into neurons without causing significant immune responses. Many studies have developed AAV-based vectors carrying genes encoding for key enzymes involved in dopamine synthesis, aiming to restore dopamine levels in the brain [1].

One such promising therapy involves the delivery of the *Aromatic L-Amino Acid Decarboxylase* (*AADC*) gene, which plays a crucial role in converting levodopa, a standard Parkinson’s medication, into dopamine within the brain. By enhancing the brain's ability to produce and utilize dopamine, AADC gene therapy seeks to alleviate motor symptoms and potentially reduce the dosage and frequency of traditional medications.

Clinical trials evaluating AADC gene therapy have shown encouraging results. Patients who underwent the procedure demonstrated improvements in motor function, with some experiencing sustained benefits over several years. Moreover, several studies reported a reduction in levodopa-induced dyskinesias, a common side effect of long-term medication use in Parkinson's disease [2].

Another innovative approach in Parkinsonism gene therapy involves the modulation of specific neuronal circuits implicated in therapy for Parkinson’s disease, involves the surgical implantation of electrodes into targeted brain regions to deliver electrical impulses. However, traditional DBS techniques lack specificity and may lead to unintended side effects.

To address this limitation, some of them are exploring optogenetics, a technique that enables precise control of neuronal activity using light-sensitive proteins. By incorporating optogenetic tools into viral vectors, scientists aim to selectively activate or inhibit neuronal circuits involved in motor control, providing a more tailored and reversible approach to symptom management [3].

Despite the promising potential of gene therapy in Parkinsonism treatment, several challenges remain. One major hurdle is the precise targeting of therapeutic genes to specific brain regions while minimizing off-target effects. Additionally, the long-term safety and efficacy of gene therapies require further investigation through rigorous clinical trials and post-marketing surveillance.

Moreover, the high cost of gene therapy poses a barrier to widespread adoption and access, highlighting the need for continued study efforts to optimize therapeutic strategies and reduce production costs. Collaborations between academia, industry and regulatory agencies are crucial for advancing gene therapy from experimental treatments to clinically viable options for patients with Parkinsonism [4].

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