Review of: "Gene therapy in the putamen for curing AADC deficiency and Parkinson's disease"

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This is an excellent manuscript on Gene Therapy in the putamen for curing AADC deficiency and Parkinson's disease".

AADC deficiency is a rare genetic disease, which symptom appears just after birth in the perinatal period and results in deficiency of dopamine and serotonin, important neurotransmitters synthesized by AADC from L-DOPA and 5-hydorxytryptophan. Deficiency of dopamine and serotonin causes severe developmental and movement disorders. The authors transfected human AADC gene via AAV-vector in the putamen of patients with AADC deficiency to produce dopamine from the endogenous L-DOPA. The clinical effects were remarkable; some patients could develop normally, stand up, walk, and even rides bicycle.

Parkinson's disease (PD), which occurs in old ages with movement disorders as the main symptom, is an aging-related dopamine deficiency caused by neurodegeneration of nigro-striatal dopamine neurons in the substantia nigra, producing dopamine deficiency in the dorsal striatum including putamen. L-DOPA administration could recover dopamine deficiency to improve movement disorders, but could not slow or stop the progress of neurodegeneration. Transfection of AADC gene via AAV-vector in the putamen with L-DOPA administration could produce dopamine in the dorsal striatum, resulting in significant improvement in movement disorders for a long period.

This gene therapy has been established after preclinical trials in experimental animal models for more than 10 years. The remaining problem in this gene therapy may be variable clinical efficacy in individual patients and finding the conditions for most efficient clinical effects.