Gene Therapies for the Treatment of Parkinson's Disease

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Technology description

Description

The investigators have used gene splicing to incorporate into host cells the genes to increase dopamine synthesis, enhance its storage, and control its transport out of the cell. These modified cells have been grafted into the central nervous system of rats where they have been shown to produce more dopamine from L-DOPA, store it, and release the dopamine to nerve cells gradually over an extended period. Previous attempts by other investigators that simply increased dopamine production have not provided the expected benefits, largely due to feedback inhibition by the dopamine itself. This new technology overcomes those problems by increasing storage and controlling the release of dopamine from the cell. In essence this process serves as a biological delivery pump in the brain for neurotransmitters and other small molecules..
Parkinsonâ€™s Disease currently affects 1.5 million Americans. The main symptoms of Parkinsonâ€™s are caused by the lack of dopamine in the brain. The most common treatment for Parkinsonâ€™s Disease is oral treatment with a dopamine precursor (L-DOPA) that can be converted to dopamine in the brain or drugs that slow dopamine breakdown. The drugs often have unpleasant side effects, yield fluctuating responses, and diminish in effectiveness over the long term. The alternative therapy, surgical implantation of fetal brain cells, faces serious logistical and ethical issues. This new technology from the University of Chicago creates genetically modified cells that are grafted into the brain where they stimulate greater and localized production and storage of dopamine and continuous release of dopamine to targeted nerve cells. These modified cells enhance the brainâ€™s response to L-DOPA and enable longer-term tolerance of the drug.

Institution

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