

Treatment of Neurological Diseases with Fractalkine Gene Therapy

Researchers at the University of South Florida have discovered that an increase in the soluble form of the chemokine fractalkine (sFKN), via recombinant adeno-associated virus (rAAV), is capable of decreasing the proinflammatory response in neurodegenerative diseases and thus increasing the neuroprotection of dopaminergic neurons.

Neurological diseases, including Parkinson's disease (PD), Alzheimer's disease (AD) and Frontotemporal dementia (FTD), are often characterized by increased inflammation in the brain, which can result in increased neurodegeneration. Although there are therapies available to treat symptoms of these diseases, there is currently no medication that can reverse the underlying pathology.

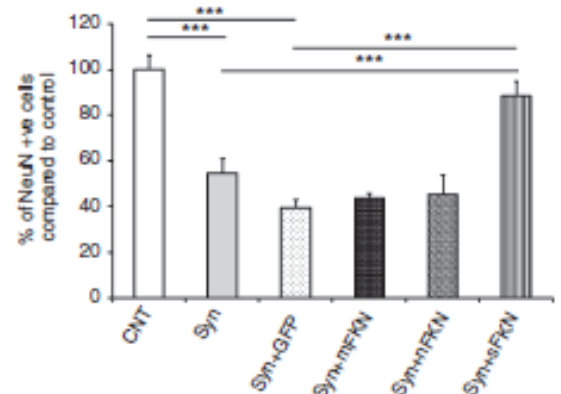
USF scientists have demonstrated in vivo that the up-regulation of sFKN expression using a gene therapy approach (rAAV) reduces α -synuclein mediated neurodegeneration. FKN serves as an endogenous neuronal modulator (calming signal), which controls the over-production of certain cytokines generated from microglia.

This novel and effective approach of gene therapy may have a distinct advantage for targeted therapeutic delivery to the CNS, reducing significant alteration of the peripheral immune system, thus reducing undesired side effects. The therapeutic potential of this invention would significantly improve the outcome of patients affected by these neurological disorders, particularly PD.

ADVANTAGES:

- Treats the underlying physiology
- Decreases neuronal loss
- Reduced immunogenicity
- Novel rAAV gene therapy

Effective Therapy for α -synuclein Mediated Neurological Diseases



sFKN Rescues Neuronal Loss in a Rat Model for α -Synuclein Mediated Parkinson's Disease