Docket #: S22-079

Neuroprotection and Axon Regeneration Therapies for CNS Axonapathies By Modulating Membrane Structure and Signaling Molecules

Stanford researchers have developed a novel technique, enabling specific labeling and purification of regenerating and non-regenerating retinal ganglion cells from the same animals with the same genetic background/modification/injuries. The approach allows for identification of axon regeneration-associated genes, which are promising therapeutic targets for neurodegenerative diseases.

Axonopathy is a common early feature of central nervous system (CNS) neurodegenerative diseases, including glaucoma and ALS. which is characterized by axon degeneration followed by progressive neuronal cell body death. The axons of adult CNS neurons do not regenerate spontaneously after degeneration, which causes irreversible neuronal function deficits. There are currently no neural repair therapies. The application of the novel technique is exemplified by the researchers' successful identification of several novel regeneration-associated genes (Anxa2, tPA, Mpp1, ILK, and more) that significantly promote axon regeneration, dramatically protect retinal ganglion cells and optic nerves, and preserves visual function in a clinically relevant model of glaucoma.

Stage of Development
In vivo: proof-of-concept demonstration of AAV-mediated expression of several identified genes, including Anxa2, ILK, and Mpp1, for neuroprotection and axon regeneration in mouse glaucoma model.

Related Technoloies

Stanford docket 21-363 describes a neuroprotective gene therapy for glaucoma by driving expression of NMNAT2 in retinal ganglion cells

Stanford docket S21-382 describes repurposed neuroprotective agents and a novel therapeutic target for the treatment of glaucoma and other optic neuropathies.

See Stanford docket S19-014 for more on the Hu team's foundational work on neuronal ER stress and AAV-mediated gene therapy strategy.

Stanford docket S19-013 describes glaucoma animal models based on silicone oil-induced mild/chronic and severe/acute reversible ocular hypertension.

Applications

- Identify therapeutic targets for: i) glaucoma and other optic neuropathies ii) ALS iii) Other axonopathies and neurodegenerative diseases
- Identifies neural repair targets

Advantages

- First potential neural repair therapy

Publications

- Li, L., Fang, F, et al. (2022). Neuron 110(16), 2646-2663.

Patents

- Published Application: WO2023201188
- Published Application: 20250242055

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