Docket #: S18-081

Therapeutic to restore vision loss

Researchers at Stanford have developed a new therapeutic to promote survival of retinal ganglion cells (RGCs) and optic nerve regeneration after traumatic injury or optic neuropathies. Visual information is conveyed from the eye to the brain via the optic nerve, which contains the axons of the RGCs. Traumatic injury or optic neuropathies can damage RGCs and the optic nerve causing permanent vision loss. Currently there are no approved medicines or therapies to protect or restore vision after RGC and optic nerve damage. Recent work by the inventors has identified a new regulatory mechanism that is important for RGC survival and optic nerve regeneration in disease. Based on these findings the Stanford researchers have developed a new therapeutic to provide long-lasting RGC neuroprotection. The therapeutic uses an AAV-gene therapy vector to deliver a specific peptide to the RGC. This, in turn, provides elevated cAMP levels which promote RGC survival and axon regeneration. This technology provides a much needed therapeutic to potentially restore vision after trauma.

Stage of research
Initial studies show great promise. Additional development is ongoing.

Applications

- Neuroprotective therapeutic to prevent or treat vision loss due to:
    - Traumatic injury
    - Optic neuropathies:
        - Glaucoma
        - Ischemic optic neuropathy
        - Traumatic optic neuropathy
        - Optic nerve drusen
        - Optic neuritis

Advantages

- First therapeutic that may allow vision restoration after traumatic injury
- Designed to minimize off target effects
- AAV vectors have been shown to be safe in human clinical trials
- Potential to provide long-lasting effects with a single dose

Publications

- T. Boczek, E.G. Cameron, W. Yu, X. Xia, S.H. Shah, B.C. Chabeco, J. Galvao, M. Nahmou, J. Li, H. Thakur, J.L. Goldberg and M.S. Kapiloff Regulation of Neuronal Survival and Axon Growth by a Perinuclear cAMP Compartment *The Journal of Neuroscience* July 10, 2019.

Patents

- Published Application: WO2020102374
- Published Application: 20220041667

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