Weill Cornell Medicine

Viral Method to Express Regenerative Proteins in Spinal Cord Injured Axons

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Background & Unmet Need

- Spinal cord injury and other traumatic axonal injuries currently have limited treatment options
- Goal of therapy: promote axonal growth and regeneration, to improve motor and sensory function in patients
- Gene therapies that increase the expression of axonal regeneration-promoting proteins is a potential treatment strategy, but is limited by the unique morphology of the affected neurons
- **Unmet Need:** Method that enables delivery of gene therapy and expression of regenerative proteins directly to injured axons, without requiring administration at cell bodies in the brain

Technology Overview

- **The Technology:** Method for targeted gene expression in axons using expression of regenerative proteins from an alphavirus vector
- **Discovery:** Protein translation occurs in the axons of neuronal cells when axonal ribosomes are presented with an RNA including an internal ribosome entry site (IRES)
- RNA alphaviruses successfully enter axons and induce the express of genes linked to an IRES
- Technology may be used to express a variety of regenerative proteins, such as dominant-negative variants of RhoA or RhoA-activated kinase, adenylyl cyclase, src kinase, and cyclic AMP-response element-binding protein (CREB)

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Patents:
US Patent 10,941,186

Publications:
Cox et al. *Nat Cell Biol.* 2018

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Technology Applications

- Treatment of spinal cord injuries
- Treatment of axonopathies such as Charco-Marie-Tooth disease and diabetic neuropathy
- Promoting axonal growth and regeneration

Technology Advantages

- Targeted axonal expression of regenerative proteins
- Modular alphavirus expression system

Supporting Data / Figures

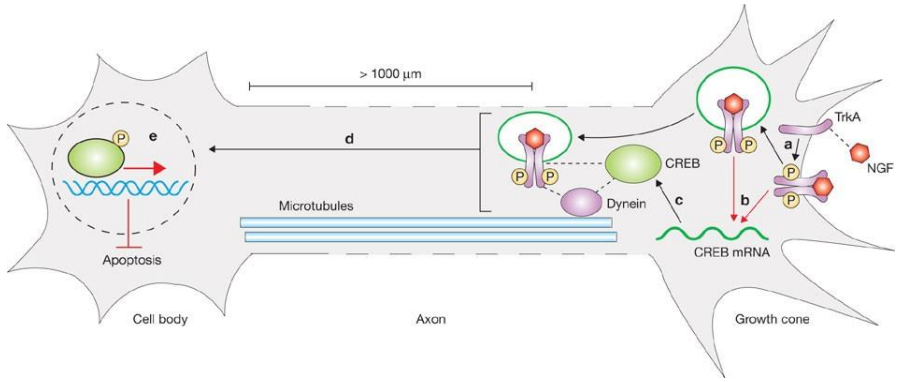

Figure 1: Local translation and retrograde transport of CREB mediates neuronal survival, and is thus an ideal system to co-opt for controllable expression of regenerative proteins at injured axons

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