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NEW STRATEGY FOR TREATMENT OF NEURODEGENERATIVE DISEASES WITH AXONOPATHY

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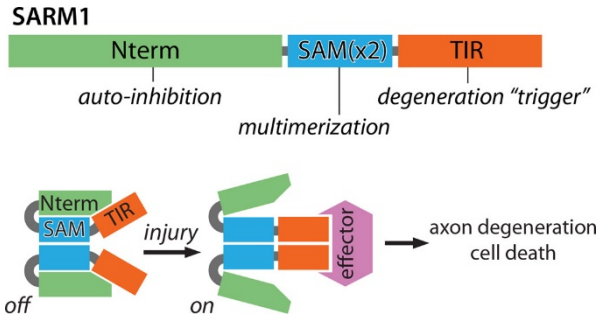
Value Proposition: This invention uses a novel gene therapy that blocks the axon degeneration typical of neurodegenerative diseases.

Technology Description

Researchers at Washington University in St. Louis have developed a new strategy for treatment of neurodegenerative diseases with axonopathy. Axon loss is a central element of many types of neurodegenerative diseases including but not limited to peripheral neuropathy, glaucoma, traumatic brain injury, ALS, Parkinson's Disease, MS, and Alzheimer's Disease. SARM1 is an NAD+ cleaving enzyme that is the central executioner of the axon degeneration pathway. Currently, there is no practical method to inhibit SARM1 in patients.

This strategy uses a potent SARM1 dominant negative gene therapy, that blocks SARM1 activity and prevents axon degeneration, to treat neurodegenerative diseases in which axon degeneration is a major component of disease symptomatology.



Stage of Research

The research team has demonstrated that SARM1 dominant negatives K193R, H685A, and combined K193/H685A have the most potent effect in blocking axon degeneration in an in vitro model of axon degeneration in DRG neurons.

Applications

- Treatment of neurodegenerative diseases

Key Advantages

- **Stops axon degeneration typical of neurodegenerative diseases**

Patents:

- **Issued Patent,** JP73679882
- **Additional application pending**

Related Web Links - Aaron DiAntonio Profile; DiAntonio Lab