Docket #: S22-413

Neuroprotection and axon regeneration therapies for CNS axonopathies by modulating mitochondria trafficking

Stanford researchers have developed a new therapeutic approach to protect neurons and promote axon regeneration by restoring mitochondrial transport within axons, a key process disrupted in many neurodegenerative diseases.

Axon degeneration is an early and common feature of central nervous system (CNS) disorders such as glaucoma and amyotrophic lateral sclerosis (ALS). In adult CNS neurons, damaged axons have a very limited ability to regenerate, leading to irreversible loss of function. Current treatments primarily manage symptoms and do not address the underlying causes of neuronal degeneration.

This approach is based on the discovery that the protein optineurin (OPTN) plays a critical role in maintaining axonal health by stabilizing the mitochondrial transport machinery. Disruption of this process leads to reduced mitochondrial distribution in axons and precedes neuronal degeneration. The researchers demonstrated that enhancing this pathway by increasing the levels or activity of OPTN and related proteins can restore mitochondrial movement, improve neuronal survival, and promote axon regeneration in disease and injury models.

By directly targeting a fundamental mechanism of axonal degeneration, this approach offers a potential path toward disease-modifying and regenerative therapies for CNS neurodegenerative diseases.

Applications

- Therapeutic targets for glaucoma, ALS, and related neurodegenerative diseases

- Small molecule or biologic modulators of axonal mitochondrial trafficking
- Neuroprotection and axon regeneration in CNS injury and disease

Advantages

- Targets a defined mechanism of axonal degeneration
- Demonstrated in vivo feasibility
- Supports both neuroprotection and regeneration
- Broad relevance across CNS neurodegenerative diseases
- Compatible with gene therapy, biologics, and small-molecule approaches

Publications

- Liu, D., Webber, H.C., Bian, F. et al. Optineurin-facilitated axonal mitochondria delivery promotes neuroprotection and axon regeneration. *Nat Commun* 16, 1789 (2025).

Patents

- Published Application: WO2025029880

Innovators

- Yang Hu
- Dong Liu
- Haoliang Huang
- Pingting Liu

Licensing Contact

David Mallin

Licensing Manager, Physical Sciences

Email