



## New Target for Diagnosing, Treating Neurodegenerative Diseases

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**The Wisconsin Alumni Research Foundation (WARF) is seeking commercial partners interested in exploring neurofilament regulation as a therapeutic target for ALS, Alzheimer's disease and other neurodegenerative conditions.**

### Overview

Amyotrophic lateral sclerosis (ALS) is a devastating disease with no effective treatment. In patients with ALS, the neurons in their brains degenerate, leading to cognitive and physical breakdown. Other neurodegenerative afflictions include Alzheimer's disease, Parkinson's disease and Huntington's disease.

All these diseases share a common feature – structural proteins called neurofilaments (which provide support to neurons) clump up or tangle. The common view is that these tangles are the result of the disease, not the cause. That view is now being challenged.

### The Invention

UW–Madison researchers have demonstrated that neurofilament tangles lead to subsequent degeneration and death of motor neurons in ALS patients. They also discovered that these tangles are caused by the reduced expression of a type of neurofilament mRNA. Thus, neurofilament regulation appears to be a promising target for drug screening and gene therapy.

The researchers conducted their studies using motor neurons derived from ALS patients.

### Applications

- Research models, drugs and gene therapies targeting neurofilament regulation

### Key Benefits

- New avenue for treating ALS and potentially other neurodegenerative diseases
- May lead to earlier diagnosis

### Stage of Development

In vitro testing.

### Additional Information

#### For More Information About the Inventors

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- [Su-Chun Zhang](#)

Publications

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- Chen et al. 2014. Modeling ALS with iPSCs Reveals that Mutant SOD1 Misregulates Neurofilament Balance in Motor Neurons. Cell Stem Cell. 14, 796-809.

#### Tech Fields

- [Drug Delivery : Other drug delivery technologies](#)
- [Drug Discovery & Development : Other drug discovery & development](#)

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