Enhancing Neuronal Resistance to Neurodegeneration

Novel therapeutic approach to reverse or prevent the progression of neurodegenerative diseases such as Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD)

Technology

Dr. Mazzoni's lab discovered an innovative method of enhancing neuronal resistance to neurodegeneration in FTD/ALS patients by targeting RNA-binding proteins. This technology is inspired by understanding cellular mechanisms that make certain neurons more resistant to neurodegenerative stress while others are highly susceptible. By regulating the expression and splicing of certain key factors, the technology provides a mechanism to maintain and improve neural health under neurodegenerative stress. This technology offers promising insights into creating targeted therapies for neurodegenerative diseases.

Background

Neurodegenerative diseases like ALS and FTD are characterized by the degeneration of specific neuronal populations, leading to motor dysfunction, paralysis, and death. A key paradox in these diseases is that certain neurons resist degeneration while others succumb, even under the same conditions. Understanding the cellular and molecular bases for this differential sensitivity has been a longstanding challenge, and current treatments fail to address the root causes of neurodegeneration. This technology provides a platform to study this differential response at the cellular level, offering insights that could inform the development of novel therapeutic approaches for patients with FTD/ALS.

Applications

- Treatment of ALS and FTD by enhancing neuronal resistance to neurodegenerative stress.
- Developing therapeutic interventions that target mechanisms of cellular resistance to neurodegeneration.
- Potential applications in other neurodegenerative diseases that exhibit selective neuronal vulnerability or splicing defects.

Advantages

- Targets the underlying cause of neurodegeneration in ALS and FTD.
- Potential to slow down or reverse neurodegenerative processes by restoring neuronal physiology.
- Provides a platform for further research into selective neuronal vulnerability in ALS and FTD.
- First therapeutic strategy to specifically target the disease-causing splicing mechanism directly downstream of the triggering defect in ALS and FTD.

Technology ID

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Category

Life Sciences/Therapeutics/Neurodeg
Diseases

Authors

Esteban O. Mazzoni, PhD

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Development Stage

Preclinical studies have shown promising results in identifying key mechanisms that control neuronal resistance to neurodegenerative stress. Further validation in animal models is ongoing.

Intellectual Property

Provisional patent application pending.