Antisense Oligonucleotides for ALS Treatment

Tech ID: 33873 / UC Case 2025-755-0

BRIEF DESCRIPTION

A novel biological therapy that targets the ataxin-3 mRNA to treat ALS by increasing ataxin-3 protein levels.

FULL DESCRIPTION

Researchers at UC Irvine devised an innovative therapeutic strategy for ALS that leverages the use of antisense oligonucleotides (ASOs) to target the 3' untranslated region (UTR) of the ataxin-3 mRNA. Conceptually, the ASOs stabilize the ataxin-3 mRNA by blocking the microRNA binding sites that promote its degradation, leading to an increase in ataxin-3 protein levels. This approach was inspired by a recent discovery indicating that genetic variants affecting the alternative polyadenylation (APA) of Ataxin-3 (ATXN3) are strongly associated with ALS.

SUGGESTED USES

- Treatment for Amyotrophic Lateral Sclerosis (ALS).
- Potential application in other neurodegenerative diseases linked to alternative polyadenylation (APA) genetic variants.
- Use in research and development of targeted therapies for genetic diseases.

ADVANTAGES

- Targets a newly discovered genetic mechanism associated with ALS.
- Utilizes antisense oligonucleotides (ASOs), a proven class of biological agents with an existing drug approved for a similar neuromuscular condition.
- Offers a potential treatment option for ALS, a disease with limited effective therapies.

PATENT STATUS

Patent Pending

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OTHER INFORMATION

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Disease: Central Nervous System
  - Gene Therapy
  - Therapeutics

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