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STAUFEN-1 TARGETING THERAPEUTIC FOR TREATMENT OF FATAL NEURODEGENERATIVE DISEASES

THERAPEUTICS

Antisense oligonucleotides that modulate expression of Staufen-1 for the treatment of neurodegeneration.

TECHNOLOGY TYPE

Rare Disease
Antisense Therapeutic
Neurology

STAGE OF DEVELOPMENT

- Testing indicates antisense oligonucleotides can modulate STAU1 levels in a cell culture model.

- Testing in tg-ATXN2 mice resulted in an improvement of their motor phenotype.

IP PROTECTION

PCT Pending
Staufen-1 Formulations and Related Methods

LEARN MORE

Reference Number: U-6263

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TECHNOLOGY SUMMARY

Neurodegenerative diseases represent an ever-increasing societal and economic burden with World Health Organization estimates indicating that they will replace cancer as the 2nd leading cause of death by 2040. Spinocerebellar ataxia type 2 (SCA2) is a part of a family of progressive, often fatal neurodegenerative diseases with no known treatments or cures. Dominantly-acting mutations lead to expansion of a polyQ domain in the ataxin-2 (ATXN2) protein. Assembly of RNA-binding protein Staufen-1 (STAU1) with mutant ATXN2 in stable inclusions is causative, resulting in aberrant RNA processing in SCA2 and other neuronal diseases such as ALS.

The proposed technology identifies STAU1 as an interventional target with STAU1 antisense therapeutic alleviating the severity of the disease in a mouse model of SCA2.

FEATURES AND BENEFITS

- Novel therapeutic intervention for a broad array of neurodegenerative diseases involving polyQ domain expansion.
- Targets disease pathogenesis and expected to stall disease progression.
- Antisense therapy approach holds strong promise with recent FDA approval of Eteplirsen for DMD and Nusinersen for SMA.

RECENT PUBLICATIONS

Paul, S., Dansithong, W., Figueroa, K., Scoles, D., Pulst, S. (2016). The role of staufen1 in aberrant RNA metabolism in SCA2. *Neurology*. 86(16): 6.396. doi: 10.1038/s41467-018-06041-3

INVENTOR PROFILE

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