WashU

Office of Technology Management

ANTISENSE OLIGONUCLEOTIDES (ASOS) TO TREAT NEURODEGENERATION IN ALS AND FTD

Djuranovic, Sergej, Pirzada, Mujeeb

Richards, Jennifer

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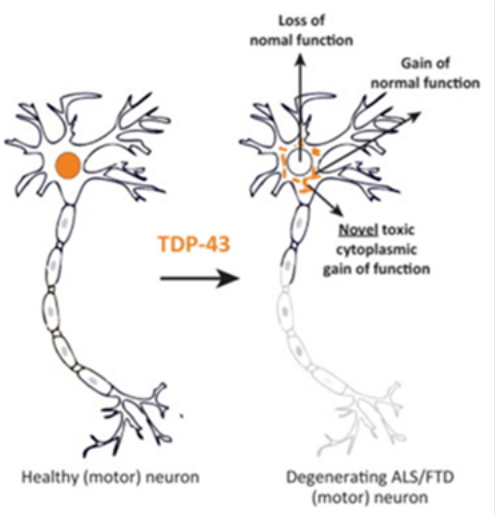
Value proposition: *Novel ASOs that increase key protein production to limit neurodegeneration caused by ALS and frontotemporal dementia (FTD).*

Technology Description

TDP-43 pathology is present in ~97% of ALS, ~50% of FTD, and frequently observed in other neurodegenerative diseases. Researchers at Washington University in St. Louis and UC Irvine have teamed up to develop novel antisense oligonucleotides (ASOs) to increase mRNA and protein levels of key effected proteins of TDP-43 pathology and tauopathies, ATXN3 and MARK3, to prevent or at least limit ALS-driven neurodegeneration.

Currently, there are no reliable drug treatments for ALS-driven neurodegeneration. Because our ASO solution addresses critical proteins affected in TDP-43 pathology and tauopathies we can reasonably anticipate that it will also prevent neurodegeneration FTD and Alzheimer's disease.



Stage of Research

Ex vivo validation underway.

Applications

- Prevention of neurodegeneration caused by TDP-43 pathology and tauopathies, such as ALS, FTD, and Alzheimer's disease.

Key Advantages:

- Restores ATXN3 and MARK3 to normal/physiological levels.
- Increases ATXN3 mRNA and protein levels.
- Decreases MARK3, a tau kinase, relevant in multiple tauopathies: Alzheimer's disease, ALS, etc.
- Sequence specificity to key transcription regulatory elements.

Patents

Patent applications filed.

Related Web Links - Sergej Djuranovic; Djuranovic Lab