Docket #: S21-425

Genetic and Peptide regulators of Ataxin-2 levels

Researchers in the Gitler lab have discovered new therapeutic targets for the treatment of Amyotrophic lateral sclerosis (ALS).

ALS is a devastating progressive nueromuscular disease that affects thousands of individuals in the United States alone and more than a hundred thousand individuals worldwide. The few currently approved treatments for ALS only provide modest improvements to symptoms and life expectancy. Mutations within the ATXN2 gene are genetic risk factors for Amyotrophic Lateral Sclerosis (ALS), possibly contributing to as many as 3-5% of sporadic ALS cases. Additionally, transgenic mice models of ALS have also implicated wild-type Ataxin-2 protein levels as a modifier of the severity and disease progression of ALS.

The Gitler lab's newly identified therapeutic targets for ALS modulate Ataxin-2 protein levels. These discoveries represent an expanded set of potential therapies for improving upon the available treatments for ALS.

Applications

- Amyotrophic lateral sclerosis (ALS)
- Spinocerebellar Ataxia Type 2 (SCA2)

Advantages

- Expansion on the number of therapeutic targets to treat ALS and SCA2 through modulation of Ataxin-2 with potential higher efficacy, as the current treatment options for ALS or SCA2 are very limited and only modestly effective

Publications

- Kim, Garam et al. "Genome-wide CRISPR screen reveals v-ATPase as a drug target to lower levels of ALS protein ataxin-2." Cell reports vol. 41,4 (2022): 111508.
- Rodriguez, Caitlin M et al. "Targeting RTN4/NoGo-Receptor reduces levels of ALS protein ataxin-2." Cell reports vol. 41,4 (2022): 111505.

Patents

- Published Application: WO2023107893

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