Docket #: S21-252

Targeting a Mitochondrial Complex to Prolong Lifespan and Treat Friedreich's Ataxia

Researchers in the Xinnan Wang lab have discovered a novel target and developed a new therapeutic approach for Friedreich's Ataxia.

Friedreich's Ataxia (FRDA) is an inherited genetic disease caused by mutations in the mitochondrial *FXN* gene. FRDA is associated with high levels of oxidative stress and results in progressive nervous system degeneration. This degeneration leads to impairments in locomotor functions and loss of sensory abilities, completely incapacitating many individuals with FRDA. Despite being the most commonly inherited ataxia, there are no currently effective treatments for FRDA.

Aiming to address this unmet clinical need, this new therapeutic approach from the Xinnan Wang lab has demonstrated protection against oxidative stress, improved locomotor function, increased cellular respiration, and prolonged lifespan in animal models of FRDA.

Applications

- Friedreich's Ataxia (FRDA)
- Oxidative stress related conditions

Advantages

- Novel therapeutic target and approach
- No currently effective treatments for FRDA

Publications

- Li, L., Conradson, D.M., Bharat, V. et al. A mitochondrial membrane-bridging machinery mediates signal transduction of intramitochondrial oxidation. Nat Metab 3, 1242–1258 (2021).

Patents

- Published Application: WO2023034232

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