Repurposing of a Small Molecule Drug for ALS Treatment

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Principal investigator

Eran Hornstein

Faculty of Biochemistry
Department of Molecular Genetics

Overview

Amyotrophic Lateral Sclerosis (ALS) remains an incurable neurodegenerative disease, in need of innovative treatment options. Prof. Eran Hornstein and his team identified microRNAs (miRNA) as pivotal regulators of ALS progression. Furthermore, they have found a way to reverse the miRNA-associated deleterious effects. This innovative approach leverages an existing known drug, Enoxacin, thereby minimizing development risks. The REALS Phase 1b/2a Clinical Trial evaluation of Enoxacin's safety and tolerability in adults with ALS demonstrates promising progress in validating this groundbreaking ALS treatment.

Background and Unmet Need

ALS is a progressive neurodegenerative disorder causing paralysis due to the death of motor neurons in the spinal cord and brain. There are currently very few FDA-approved drugs for ALS, with only modest effects, providing marginal survival benefits without improving the quality of life. Given the aggressive progression, the limited life expectancy of patients and the scarcity of effective therapies, there is an urgent medical need for innovative ALS treatments.

The Solution

Enoxacin, a fluoroquinolone, globally enhances miRNA biogenesis, which is impaired in patients with ALS. Therefore, Enoxacin may be a novel ALS treatment.

Technology Essence

Prof. Hornstein and his team demonstrated that miRNA are globally down-regulated in motor neurons of both sporadic and familial ALS patients, and explore their roles as master regulators of ALS pathogenesis. They further demonstrated that administration of Enoxacin to two mouse models of ALS, SOD1 G93A, and TDP-43 A315T, improved neuromuscular function.

Applications and Advantages

Â· Effective ALS Drug: repurposing an FDA-approved medication reduces drug development risks.

Development Status

REALS is an ongoing Phase 1b/2a Study, aimed to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of Enoxacin in adults with ALS. This trial shows initial encouraging results and marks a significant step in understanding the impact of Enoxacin on ALS, providing essential data for its potential therapeutic application.

References

Emde, A., et al. (2015). Dysregulated mi RNA biogenesis downstream of cellular stress and ALS â€causing mutations: a new mechanism for ALS. The EMBO Journal, 34(21), 2633â€“2651. https://doi.org/10.15252/embj.201490493 [1]

Patent Status

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