

Empagliflozin Repurposing for Lafora Disease

Investigating New Therapeutic Targets

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RESEARCH SIMPLIFIED

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AIMS AND METHODS

In this article, the researchers propose a study to determine the safety and effectiveness of empagliflozin for slowing the progression of Lafora disease (LD). Empagliflozin is designed to elevate glucose removal from the body through the kidneys. In mouse models, empagliflozin helps with conditions like heart failure and other glycogen storage disorders that disrupt glucose metabolism, like LD. Empagliflozin works by blocking the activity of a protein called Sodium-Glucose Cotransporter-2 (SGLT2). SGLT2 helps the kidneys reabsorb glucose into the bloodstream, keeping it in the body. When the activity of this protein is blocked, more glucose is removed from the body through urine. Since the storage of excess glucose in glycogen plays a role in Lafora body formation, researchers wanted to investigate whether empagliflozin can reduce the amount of glucose being stored in glycogen, and therefore slow Lafora disease progression. Beyond its effects on glucose metabolism, empagliflozin also improved movement and reduced the overactivity of brain cells in other diseases, two notable symptoms in LD.

THE PROPOSAL

Two patients with early to intermediate LD will be treated with 10 milligrams of empagliflozin. Researchers will monitor changes in seizures, brain activity, movement, behavior, and overall cognition over 6 months. They hope to examine how empagliflozin interacts with glucose and glycogen, which will explain how it works and when to administer it to a patient. As the study does not involve a control, published data evaluating Metformin, a drug proven to slow disease progression, will be used as comparisons for the patient study. Similar drugs will be tested in tandem with the other experiments to create a standard for comparison.

CONCLUSION

While current medications are designed to alleviate or reduce symptoms, the most effective LD therapy would be a treatment that can halt or slow disease progression. If empagliflozin's safety and effectiveness in treating patients with Lafora disease is clinically successful, trials can begin with a larger population to strengthen these findings. Finding a potential treatment in empagliflozin will encourage the screening of other FDA-approved medications to see if they can be repurposed for LD, ultimately accelerating the progress for a definitive treatment.

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