Effect of Alglucosidase Alfa (Myozyme) Therapy in Lafora Disease

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AIM

Alglucosidase alfa (Myozyme) is a therapeutic enzyme approved for the treatment of Pompe disease, another glycogen storage disorder. It works by breaking down glycogen molecules in the compartment of the cell responsible for the degradation of waste (the lysosome). Given its success in treating Pompe disease, researchers investigated whether Myozyme could similarly alleviate the pathological features of Lafora disease by targeting Lafora Bodies (located in the cytoplasm of the cell).

METHODS

To assess the efficacy of Myozyme in Lafora disease, experiments were conducted using mouse models of the disease. The treatment was given directly into the brain using different methods like injections or infusions with pumps. These methods aimed to deliver the enzyme directly into the brain, ensuring that it bypasses the blood-brain barrier, in the hope of reducing their burden and improving disease symptoms.

RESULTS

However, despite these efforts, the study's results revealed that neither injections nor prolonged infusions of Myozyme effectively reduced the number of Lafora Bodies in the brains of Lafora disease mouse models. Additionally, there were no observable improvements in cognitive function, anxiety levels, or propensity for seizures/myoclonic jerks following treatment with Myozyme. These findings suggest that Myozyme may not effectively target Lafora Bodies. This may be because the enzyme acts in a different cellular location to where Lafora Bodies are found.

CONCLUSION

In summary, while Myozyme has shown therapeutic benefits in other glycogen storage disorders, such as Pompe disease, it does not appear to be an effective treatment for Lafora disease.