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Commentary

Enzyme Replacement Therapy for Different Diseases

Statin Brilan*

Department of Biotechnology, Thomas Jefferson University, Philadelphia, Pennsylvania, USA

DESCRIPTION

Enzyme Replacement Therapy (ERT) is a drug procedure in which patients with chronic diseases caused by enzyme shortages or failure are given replacement enzymes. IV infusions, in which the substitute enzyme is delivered directly into the bloodstream *via* a regulated drip of fluids, are the most prevalent type of ERT. Human, animal, and plant cells are used to make replacement enzymes for ERT, which are then genetically engineered and processed before being given to the patient. The body is able to successfully complete the processes that are impeded by the deficiency by getting these enzyme replacements.

Enzyme Replacement Therapy (ERT), which is based on the intravenous infusion of specific enzymes created using recombinant DNA technology on a regular basis, is now the most effective treatment option for a variety of lysosomal storage disorders. The recombinant enzymes are a purified form of the lysosomal enzymes that are produced in continuous human (fibroblasts) or animal cell lines (Chinese Hamster Ovary (CHO) cells) and plant cells. The oligosaccharide chains of the resultant glycoproteins contain mannose-6-phosphate (M6P) residues. This permits the enzyme to attach to M6P receptors on the cell surface, allowing it to enter the cell and be directed to lysosomes, where it can catabolize the accumulated substrates.

Process of replacement in different diseases

For gauchers disease: ERT presents the overview of the glucocerebrosidase (GCase) enzyme to counteract low levels of the enzyme. This enzyme degrades glucocerebroside, a fatty substance that builds up in the bodies of gaucher disease sufferers. (The therapy is called enzyme replacement because it compensates for the lost enzyme) gaucher disease can be treated with oral Substrate Reduction Therapy (SRT) in addition to ERT infusion medicines. This medicine differs from ERT in that it operates differently and can only be given to certain people.

For alpha-mannosidosis in children and adults: Alpha-mannosidosis is a rare genetic enzyme condition that causes cell damage in numerous organs and tissues throughout the body by accumulating mannose-rich oligosaccharides. Intellectual incapacity, liver or spleen enlargement, unusual facial traits, and skeletal anomalies are all symptoms of the condition. The signs and symptoms of alpha-mannosidosis range from mild to severe. Individuals with early-onset severe and rapidly advancing disease rarely live through childhood, although persons with milder forms of the disease often live into adulthood. Currently, there is no cure for this illness. Supportive care, such as symptom management, medication and surgical intervention of sequelae, and physical therapy, is provided to patients with less severe forms of the condition.

Lamzede is a recombinant human alpha mannosidase that was developed as an intravenous Enzyme Replacement Therapy (ERT) for alpha-mannosidosis. The goal of this therapy is to inject medicine into the bloodstream in replacing the functionality of the body's defective protein. The goal of the ERT is to normalise oligosaccharide levels in the body, prevent illness progression and anomalies, and enhance a patient's condition. Lamzede, on the other hand, does not pass the blood-brain barrier and hence is not expected to affect the neurological features of the condition, as indicated by replacement therapy.

Advantages

In comparison to SRT, ERT has less adverse effects. SRT is a newer treatment option than ERT, and some people prefer to use treatments and have been on the market for a considerable duration. Unlike SRT, ERT can be used by both children and adults, including pregnant and nursing mothers.

CONCLUSION

The use of ERT in cognitively affected individuals is a contentious issue in the scientific community right now, with the consensus being that ERT should be initiated in any patient who has the potential to transform some more of the disease's somatic symptoms. Other drugs are currently being developed, including various types of more powerful ERT, drugs depending on multiple principles of enzyme replacement, such as substrate deprivation, chaperone therapy, exon skipping, and gene therapy, and drugs depending on multiple principles of enzyme replacement, such as substrate deprivation, chaperone therapy, exon skipping, and gene therapy.

Correspondence to: Statin Brilan, Department of Biotechnology,Thomas Jefferson University, Philadelphia, Pennsylvania, USA, E-mail: Stabrl@gmail.com
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