BlueCross BlueShield Federal Employee Program

ELELYSO
(taliglucerase alfa)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. Accumulation of lipids in these areas results in the enlargement of the liver and spleen, anemia, thrombocytopenia, lung disease and bone abnormalities. Symptoms of Gaucher disease usually become apparent in early childhood or adolescence but can be diagnosed at any stage of life. It is important to begin intervention early to prevent damage to the liver and spleen (1).

Elelyso is an injectable enzyme replacement product for the treatment of adults with type 1 Gaucher disease. There are three clinical subtypes of Gaucher disease differentiated by the presence or absence of neurological involvement: type 1, type 2 and type 3. Type 1, known as non-neuronopathic, is the most common. There is insufficient evidence supporting the use of Elelyso for the treatment of type 2 and type 3 Gaucher disease (1).

Regulatory Status

FDA-approved indication: Elelyso is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for the treatment of patients 4 years and older with a confirmed diagnosis of type 1 Gaucher disease (1).

Elelyso has a boxed warning regarding hypersensitivity reactions including anaphylaxis. Elelyso should be administered in a healthcare setting with appropriate medical monitoring and support measures, including access to cardiopulmonary resuscitation equipment (1).

The safety of Elelyso has not been established in pediatric patients younger than 4 years of age (1).

Summary

Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. Elelyso is a form of the human

lysosomal enzyme, glucocerebrosidase, and is effective in replacing the enzyme deficiency in type 1 (non-neuronopathic) Gaucher disease. The safety of Elelyso has not been established in pediatric patients younger than 4 years of age (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Elelyso while maintaining optimal therapeutic outcomes.

References

1. Elelyso [Package Insert]. New York, NY: Pfizer Labs; January 2025.