BlueCross BlueShield Federal Employee Program

VPRIV
(velaglucerase 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).

VPRIV is an injectable enzyme replacement product for the treatment of children and 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-neuropathic, is the most common. There is insufficient evidence supporting the use of VPRIV for the treatment of type 2 and type 3 Gaucher disease (1).

Regulatory Status

FDA-approved indication: VPRIV is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease (2).

The most common adverse effects are infusion reactions and allergic reactions. Anaphylaxis has been observed in some patients (2).

The safety of VPRIV has not been established in pediatric patients younger than 4 years of age (2).

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. It is important to begin intervention early to prevent damage to the liver and spleen. VPRIV is a form of the human

lysosomal enzyme, glucocerebrosidase, and is effective in replacing the enzyme deficiency in type 1 (non-neuronopathic) Gaucher disease (1-2).

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

References

1. National Institute of Neurological Disorders and Stroke – National institute of health (NINDS-NIH) website. Gaucher disease information page.
2. VPRIV [Package Insert]. Cambridge, MA: Shire Human Genetic Therapies, Inc.; September 2021.