



## **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).

Cerezyme is an injectable enzyme replacement product for the treatment of pediatric and adult patients with type 1 Gaucher disease. Cerezyme catalyzes the hydrolysis of glucocerebroside to glucose and ceramide. In clinical trials, Cerezyme improved anemia and thrombocytopenia, reduced spleen and liver size, and decreased cachexia (1).

### **Regulatory Status**

FDA-approved indications: Cerezyme is an analogue of the human enzyme  $\beta$ -glucocerebrosidase for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions (1):

1. Anemia
2. Thrombocytopenia
3. Bone disease
4. Hepatomegaly or splenomegaly

The most common adverse effects are infusion reactions and allergic reactions. Anaphylaxis has been observed in some patients (1).

In patients who developed IgG antibody to Cerezyme, an apparent effect on serum enzyme levels resulted in diminished volume of distribution and clearance and increased elimination half-life compared to patients without antibody (1).

The safety and effectiveness of Cerezyme has been established in patients between 2 and 16 years of age. Cerezyme has been administered to patients younger than 2 years of age,



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## **Cerezyme (imiglucerase)**

however the safety and effectiveness in patients younger than 2 has not been established (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. It is important to begin intervention early to prevent damage to the liver and spleen. In clinical trials, Cerezyme improved anemia and thrombocytopenia, reduced spleen and liver size, and decreased cachexia. Cerezyme 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).

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

### **References**

1. Cerezyme [package Insert]. Cambridge, MA: Genzyme Corporation; December 2024.