Agents for Gaucher's Disease

WA.PHAR.26 Agents for Gaucher's Disease Background:

Gaucher Disease is an inherited autosomal recessive disease characterized by deficient glucocerebrosidase and consequent accumulation of glucocerebroside in the reticuloendothelial cells of the liver, spleen, bone marrow, and other tissues. Type-1 Gaucher disease is the most common subtype, accounting for more than 90% of all cases, and is characterized by systemic manifestations without primary central nervous system involvement (nonneuronopathic). Type-2 Gaucher disease is characterized by severe early neurologic manifestations (acute neuronopathic) with death usually occurring before 2 years of age. Type-3 Gaucher disease is characterized by subacute neurologic symptoms (chronic neuronopathic) and systemic manifestations.

Medical necessity

Drug	Medical Necessity
eliglustat (Cerdelga®) imiglucerase (Cerezyme®) miglustat (Zavesca®) taliglucerase (Elelyso®) velaglucerase alfa (VPRIV®)	Eliglustat, imiglucerase, miglustat, taliglucerase, and velaglucerase may be considered medically necessary when used for treating Type -1 Gaucher's disease. Imiglucerase, taliglucerase, and velaglucerase may be considered medically necessary when used for treating Type-3 Gaucher's disease.

Clinical policy:

Drug	Clinical Criteria (Initial Approval)
eliglustat (Cerdelga®) imiglucerase (Cerezyme®) miglustat (Zavesca®) taliglucerase (Elelyso®) velaglucerase alfa (VPRIV®)	**Type 1 Gaucher disease, adult [Cerdelga, Cerezyme, Zavesca, Elelyso, VPRIV]** 1. Documented diagnosis of type 1 Gaucher's disease 2. ANY of the following symptoms (a, b, c, d, or e): a. Moderate to severe anemia (hemoglobin ≤ 11.5 g/dL [adult women] or ≤ 12.5 g/dL [adult men] or ≤ 1.0 g/dL or more below the lower limit of normal for age and sex) b. Significant hepatomegaly (liver size 1.25 or more times normal [1,750 cc in adults]) or splenomegaly (spleen size 5 or more times normal [875 cc in adults]) c. Skeletal disease beyond mild osteopenia and Erlenmeyer flask deformity d. Symptomatic disease, including abdominal or bone pain, fatigue, exertional limitation, weakness, or cachexia e. Thrombocytopenia (platelet count less than or equal to 120,000/mm3). 3. Age Limits:

	a. eliglustat (Cerdelga®): Greater than or equal to (≥) 18 years of age b. imiglucerase (Cerezyme®): Greater than or equal to (≥) 12 years of age c. miglustat (Zavesca®): Greater than or equal to (≥) 18 years of age d. taliglucerase (Elelyso®): Greater than or equal to (≥) 4 years of age e. velaglucerase alfa (VPRIV®): Greater than or equal to (≥) 4 years of age 4. Zavesca only: Treatment with enzyme-replacement therapy (i.e. imiglucerase [Cerezyme], taliglucerase alfa [Elelyso], velaglucerase alfa [VPRIV]) was ineffective, not tolerated, or is contraindicated 5. Cerdelga only: The member has been tested to determine CYP2D6 genotype is one of the following: extensive metabolizer (EM), intermediate metabolize (IM), or poor metabolizer (PM) **Approve for 12 months**
imiglucerase (Cerezyme®) taliglucerase (Elelyso®) velaglucerase alfa (VPRIV®)	**<u>Type 3 Gaucher's disease, adult and pediatric [Cerezyme, Elelyso, VPRIV only]</u>** 1. Documented diagnosis of type 3 Gaucher's disease 2. Neurologic findings consistent with type 3 Gaucher's disease, including encephalopathy, opthalmoplegia, progressive myoclonic epilepsy, cerebellar ataxia, spasticity, or dementia 3. ANY of the following symptoms (a, b, c, d, or e): a. Moderate to severe anemia (hemoglobin ≤ 11.5 g/dL [adult women] or ≤ 12.5 g/dL [adult men] or ≤ 1.0 g/dL or more below the lower limit of normal for age and sex) b. Significant hepatomegaly (liver size 1.25 or more times normal [1,750 cc in adults]) or splenomegaly (spleen size 5 or more times normal [875 cc in adults]) c. Skeletal disease beyond mild osteopenia and Erlenmeyer flask deformity d. Symptomatic disease, including abdominal or bone pain, fatigue, exertional limitation, weakness, or cachexia e. Thrombocytopenia (platelet count less than or equal to 120,000/mm3). **Approve for 12 months**
	Criteria (Reauthorization)
	Documentation of positive clinical response **Approve for 12 months**

Dosage and quantity limits

Drug Name	Dose and Quantity Limits
eliglustat (Cerdelga)	168mg maximum daily dose; 60 capsules in 30 days.
imiglucerase (Cerezyme®)	Dose does not exceed 60 units/Kg every 2 weeks
miglustat (Zavesca®)	100 mg orally 3 times daily

Coding:

HCPCS Code	Description
J3060	Injection, imiglucerase, 10 units
J3060	Injection, taliglucerase alfa, 10 units
J3385	Injection, velaglucerase alfa, 100 units
ICD-10 Diagnosis Code	**Description**
E75.22	Gaucher Disease

References

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4. PDR® Electronic Library™ [Internet database]. Greenwood Village, CO: Thomson Micromedex. Updated periodically.
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6. Cerezyme [prescribing information]. Cambridge, MA: Genzyme Corp.; March 2003.
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9. Zavesca [prescribing information]. South San Francisco, CA: Actelion Pharmaceuticals US Inc.; Feb. 2014.
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