CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Georgia Medicaid

DRUG NAME	ENZYME REPLACEMENT THERAPY (ERT) FOR GAUCHER DISEASE: Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), Vpriv (velaglucerase alfa)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Gaucher disease is a rare, inherited, lysosomal storage disorder. In Gaucher disease, mutations of the GBA gene cause deficiency of the enzyme glucocerebrosidase (acid beta-glucosidase), resulting in the accumulation of glucocerebroside (glucosylceramide [GLC]) in the lysosomes of macrophages to form "Gaucher cells," especially in the bone marrow, spleen, and liver. Prominent symptoms include hepatosplenomegaly, anemia, thrombocytopenia, and skeletal problems (e.g., bone pain, osteopenia, osteonecrosis, fracture, deformity).
Type 1 Gaucher disease is the most common form and does not affect the central nervous system. Type 2 and 3 Gaucher disease are characterized by the presence of primary neurologic disease. Type 2 has an onset before age two years and is rapidly progressive with death by age two to four years. Individuals with type 3 often have a more slowly progressive course. Available treatments are indicated for Type 1 Gaucher disease and include enzyme replacement therapy (ERT) or substrate reduction therapy (SRT). Individuals with type 2 Gaucher disease are not likely to respond to ERT or SRT. This policy focuses on ERT.
Cerezyme was the first ERT product approved by the FDA for Gaucher disease, approved in 1994. Notably, Gaucher disease was the first lysosomal storage disorder for which an effective ERT was developed.

Enzyme replacement therapy for Gaucher disease will be considered for coverage when the following criteria are met:

Gaucher Disease

For **initial** authorization:

1. Member meets the labeled age requirement:
   a) Cerezyme: At least 2 years of age
   b) Elelyso: At least 4 years of age
   c) Vpriv: At least 4 years of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, hematologist, or metabolic specialist; AND
3. Member has a diagnosis of Gaucher disease Type 1 or Type 3 confirmed by documentation of at least one of the following:
   a) Reduced activity of glucocerebrosidase via enzyme assay (0 to 15% of normal), and/or
   b) Molecular genetic test documenting 2 mutations (biallelic variants) of the GBA gene; AND
4. Member has at least one of the following as a result of Gaucher disease:
   a) Anemia
   b) Thrombocytopenia
   c) Bone symptoms
   d) Enlarged spleen or liver; AND
5. Member does NOT have any of the following:

a) Type 2 Gaucher disease,
b) Severe or rapidly progressing neurological complications,
c) Concomitant use of miglustat or eliglustat.

6. **Dosage allowed/Quantity limit:**
   Type 1 Gaucher disease: Up to 60 units/kg every other week IV infusion
   Type 3 Gaucher disease: Based on clinical literature and physician expertise.

NOTE: Treatment of Type 3 Gaucher disease is off label.

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

1. Chart notes must show improvement from baseline in at least one of the following signs or symptoms:
   a) Hemoglobin level
   b) Platelet count
   c) Reduced liver and/or spleen volume(s)
   d) Skeletal manifestations (e.g., less bone pain, fewer bone crises, etc.)

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), Vpriv (velaglucerase alfa) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
08/02/2021	New policy for ERT for Gaucher disease created.
05/17/2023	Updated and added references. Clarified that the gene mutation should be biallelic.

References:

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2. Elelyso [package insert]. NY, NY: Pfizer Inc.; Revised 2023.
3. Vpriv [package insert]. Lexington, MA: Shire Human Genetic Therapies, Inc.; Revised 2021.
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15. Biegstraaten M, Cox TM, Belmatoug N, et al. Management goals for type 1 Gaucher disease: An expert consensus document from the European working group on Gaucher disease. *Blood Cells Mol Dis*. 2018;68:203-208. doi:10.1016/j.bcmd.2016.10.008

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