

Market Applicability/Effective Date														
Market	FL & FHK	FL MMA	FL LTC	GA	KS	KY	LA	MD	NJ	NV	NY	TN	TX	WA
Applicable	X	NA	NA	X	NA	X	X	X	X	X	X	NA	NA	X

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Enzyme Replacement Therapy for Gaucher Disease

CG-DRUG-08

Override(s)	Approval Duration
Prior Authorization	1 Year

Medications	Quantity Limit
Enzyme Replacement Therapy for Gaucher Disease: <ul style="list-style-type: none"> • Imiglucerase (Cerezyme) • Taliglucerase alfa (ELELYSO) • Velaglucerase alfa (VPRIV) 	N/A

APPROVAL CRITERIA

- I. Imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO) or velaglucerase alfa (VPRIV) may be approved for the treatment of **Adults** (18 years of age and older) with **type 1 Gaucher disease** when the criteria below are met:
 - a. The drug is used as monotherapy; **AND**
 - b. Type 1 Gaucher disease is confirmed by either of the following:
 - i. Glucocerebrosidase activity in the white blood cells or skin fibroblasts less than or equal to 30% of normal activity;

-OR-

 - ii. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome; **AND**
 - c. Clinically significant manifestations of Gaucher disease, including **any** of the following:
 - i. Skeletal disease as demonstrated by **any** of the following:
 - Avascular necrosis; or
 - Erlenmeyer flask deformity (failure of bone remodeling) ; or
 - Lytic disease; or
 - Marrow infiltration; or
 - Osteopenia; or

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.

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- Osteosclerosis; or
- Pathological fracture; or
- Radiological evidence of joint deterioration;

-OR-

- ii. Presents with two or more of the following:
 - Clinically significant hepatomegaly; **or**
 - Clinically significant splenomegaly; **or**
 - Hemoglobin less than or equal to 11.5 g/dL for females and less than 12.5 g/dL for males, or 1.0g/dL below lower limit for normal for age and sex; **or**
 - Platelet count less than or equal to 120,000mm³; **or**

II. Imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO), or velaglucerase alfa (VPRIV) may be approved for the treatment of **Children** (less than 18 years of age) with **type 1 Gaucher disease** when the following criteria are met:

- a. The drug is used as monotherapy; **AND**
- b. Type 1 Gaucher disease is confirmed by **either** of the following:
 - i. Glucocerebrosidase activity in the white blood cells or skin fibroblasts less than or equal to 30% of normal activity;

-OR-

- ii. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome; **AND**
- c. There are clinically significant manifestations of Gaucher disease including **any** of the following:
 - i. Abdominal or bone pain; or
 - ii. Hepatosplenomegaly; or
 - iii. Documented growth failure not associated with other conditions; or
 - iv. Cachexia; or
 - v. Exertional limitations; or
 - vi. Fatigue; or
 - vii. Evidence of skeletal involvement, including, but not limited to, Erlenmeyer flask deformity (failure of bone remodeling); or
 - viii. Anemia with hemoglobin less than 2.0 g/dL below lower limit of normal for age and sex; or

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- ix. Platelet count less than 60,000 mm³ or documented abnormal bleeding episode(s).
- III. Imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO) or velaglucerase alfa (VPRIV) may be approved for the treatment of **Adults** (18 years of age or older) with type 3 Gaucher disease when the criteria below are met:

- a. The drug is used as monotherapy; **AND**
- b. Type 3 Gaucher disease is confirmed by genotype testing indicating the presence of two homozygous alleles for neuropathic Gaucher disease; **AND**
- c. There are clinically significant manifestations of Gaucher disease, including **any** of the following:
 - i. Skeletal disease as demonstrated by **any** of the following:
 - Avascular necrosis; or
 - Erlenmeyer flask deformity (failure of bone remodeling); or
 - Lytic disease; or
 - Marrow infiltration; or
 - Osteopenia; or
 - Osteosclerosis; or
 - Pathological fracture; or
 - Radiological evidence of joint deterioration;

-OR-

- ii. Presents with two or more of the following:
 - Clinically significant hepatomegaly
 - Clinically significant splenomegaly
 - Hemoglobin less 1.0 g/dL below lower limit for normal for age and sex)
 - Platelet count less than or equal to 120,000mm³ ; **AND**
- d. There are neurological findings consistent with type 3 Gaucher disease based on evaluation which includes **all** of the following tests:
 - Neurological examination; and
 - Eye movement examination; and
 - Neuro-ophthalmological investigation with direct ophthalmoscopy; and
 - Measurement of peripheral hearing (electro-acoustical emission in small children, pure tone audiometry in older individuals); and
 - Brain Imaging, preferably by magnetic resonance imaging (MRI), or if MRI is unavailable, by computed tomography; and
 - Electroencephalography (EEG); and
 - Diagnostic brain stem evoked responses (BSER); and
 - Intelligence quotient (IQ) testing, when appropriate and reasonable.

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IV. Alglucerase (Ceredase), imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO), or velaglucerase alfa (VPRIV) may be approved for the treatment of **Children** (less than 18 years of age) with **type 3** Gaucher disease when the following criteria are met:

- a. The drug is used as monotherapy; **AND**
- b. Type 3 Gaucher disease is confirmed by genotype testing indicating the presence of two homozygous alleles for neuropathic Gaucher disease; **AND**
- c. There are clinically significant manifestations of type 3 Gaucher disease including **any** of the following:
 - i. Abdominal or bone pain; or
 - ii. Hepatosplenomegaly; or
 - iii. Documented growth failure not associated with other conditions; or
 - iv. Cachexia; or
 - v. Exertional limitations; or
 - vi. Fatigue; or
 - vii. Evidence of skeletal involvement, including, but not limited to, Erlenmeyer flask deformity (failure of bone remodeling); or
 - viii. Hemoglobin less than 2.0 g/dL below lower limit of normal for age and sex; or
 - ix. Platelet count less than 60,000 mm³ or documented abnormal bleeding episode(s); **AND**
- d. There are neurological findings consistent with type 3 Gaucher disease based on evaluation which includes **all** of the following tests.
 - Neurological examination; and
 - Eye movement examination; and
 - Neuro-ophthalmological investigation with direct ophthalmoscopy; and
 - Measurement of peripheral hearing (electro-acoustical emission in small children, pure tone audiometry in older patients); and
 - Brain Imaging, preferably by magnetic resonance imaging (MRI), or if MRI is unavailable, by computed tomography; and
 - Electroencephalography (EEG); and
 - Diagnostic brain stem evoked responses (BSER); and
 - Intelligence quotient (IQ) testing, when appropriate and reasonable.

May **NOT** be approved for the following:

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The use of imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO), or velaglucerase alfa (VPRIV) for the treatment of patients with **type 2 Gaucher disease**

The use of imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO), or velaglucerase alfa (VPRIV) in conjunction with each other

The use of imiglucerase (Cerezyme), taliglucerase alfa (ELELYSO), or velaglucerase alfa (VPRIV) for the treatment of **Adults** and **Children** with type 1 or type 3 Gaucher disease for all other uses, including when the criteria above have not been met.

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.