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Coverage Policy Number IP0163

Taliglucerase

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Overview

This policy supports medical necessity review for taliglucerase (Elelyso®).

Medical Necessity Criteria

Taliglucerase (Elelyso) is considered medically necessary when the following are met:

1. **Gaucher Disease.** Individual meets **ALL** of the following criteria (A, B, and C):
 - A. Individual has symptomatic Type 1 or Type 3 Gaucher disease that results in at least **ONE** of the following: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly
 - B. Documented confirmation of diagnosis is established by **ONE** of the following (i or ii)
 - i. Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts
 - ii. Molecular genetic testing documenting glucocerebrosidase gene mutation (biallelic pathogenic variants in the *GBA* gene)

- C. Medication is being prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorder

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Note: Receipt of sample product does not satisfy any criteria requirements for coverage.

Reauthorization Criteria

Taliglucerase (Elelyso) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response (for example, reduced severity or resolution of anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly).

Authorization Duration

Initial approval duration is up to 12 months.

Reauthorization approval duration is up to 12 months.

Conditions Not Covered

Taliglucerase (Elelyso) is considered experimental, investigational or unproven for **ANY** other use.

Coding / Billing Information

- Note: 1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J3060	Injection, taliglucerase alfa, 10 units

Background

OVERVIEW

Elelyso, an analogue of β -glucocerebrosidase, is indicated for the treatment of patients 4 years and older with a confirmed diagnosis of Type 1 Gaucher disease.¹

Elelyso is produced via recombinant DNA technology in genetically modified carrot plant root cells.¹ Elelyso differs from human glucocerebrosidase by two amino acids at the N terminal and seven amino acids at the C terminal end of the protein. Elelyso catalyzes the breakdown of glucocerebroside to glucose and ceramide.

Dosing and Availability

Treatment-naïve patients: The recommended dosage of Elelyso for long-term treatment is 60 units/kg of body weight administered every other week as a 60 to 120 minute intravenous infusion.

Patients switching from imiglucerase: Patients currently being treated with imiglucerase for Type 1 Gaucher disease can be switched to Elelyso. Patients previously treated on a stable dosage of imiglucerase are

recommended to begin treatment with Eluelyso at that same units/kg dosage when they switch from imiglucerase to Eluelyso. Administer Eluelyso for long-term treatment every other week as a 60 to 120 minute intravenous infusion. Dosage adjustments can be made based on achievement and maintenance of each patient's therapeutic goals.¹

Eluelyso is available for injection: 200 units white to off-white lyophilized powder in a single-dose vial for reconstitution.

Disease Overview

Gaucher disease is a rare autosomal recessive, inherited, lysosomal storage disorder caused by a deficiency of the lysosomal enzyme β -glucocerebrosidase.²⁻⁴ Glucocerebrosidase is responsible for the breakdown of glucosylcerebroside (GluCer) into glucose and ceramide. A deficiency of this enzyme is characterized by an excessive accumulation of GluCer in the visceral organs such as the liver, spleen, and bone marrow. GluCer remains stored within lysosomes causing enlarged lipid-laden macrophages called "Gaucher cells".

Gaucher disease is classified into three phenotypes (Types 1 through 3).²⁻⁵ Type 1 is a non-neuropathic variant with asymptomatic or symptomatic clinical manifestations of splenomegaly, hepatomegaly, anemia, thrombocytopenia, skeletal complications, and occasional lung involvement. Type 2 is an acute neuropathic form characterized by an early onset (3 to 6 months of age) of rapidly progressive neurological disease with visceral manifestations; death generally occurs by the time patients reach 1 to 2 years of age. Type 3 is characterized by neurological symptoms and visceral symptoms with a later onset and includes abnormal eye movements, ataxia, seizures, and dementia. Type 1 is most prevalent in the Western world, accounting for an estimated 94% of patients with Gaucher disease.^{2,6} Types 2 and 3 represent < 1% and 5%, respectively, in Europe, North America, and Israel.^{2,5} The diagnosis of Gaucher disease is established by demonstrating deficient β -glucocerebrosidase activity in leukocytes or fibroblasts, or mutations in the glucocerebrosidase gene.^{7,8}

References

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