Effective Date **10/1/2023**
Next Review Date **10/1/2024**
Coverage Policy Number **IP0164**

Velaglucerase

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INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

This policy supports medical necessity review for velaglucerase (Vpriv®).

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

Medical Necessity Criteria

Velaglucerase (Vpriv) 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 (for example, 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.

Reauthorization Criteria

Velaglucerase (Vpriv) 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 and reauthorization approval duration is up to 12 months.

Conditions Not Covered

Any other use is considered experimental, investigational or unproven for **ANY** other use including the following:

1. Concomitant use with other treatments (for example, Cerdelga, Cerezyme, Elelyso, Vpriv, and Zavesca) approved for Gaucher disease.

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
J3385	Injection, velaglucerase alfa, 100 units

Background

OVERVIEW
Vpriv, an analogue of β-glucocerebrosidase, is indicated for long-term enzyme replacement therapy for patients with **Type 1 Gaucher disease**.[1]

Vpriv is produced via gene activation technology in a human fibroblast cell line.[1] Vpriv has the same amino acid sequence as the naturally occurring human glucocerebrosidase. Vpriv catalyzes the breakdown of glucocerebroside to glucose and ceramide.

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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4. Jmoudiak, M. and Futerman, AH. Gaucher disease: Pathological mechanisms and modern management. Br J Haematol. 2005;129(2):178–188.
5. Grabowski GA. Lysosomal storage disease 1- phenotype, diagnosis, and treatment of Gaucher's disease. Lancet. 2008;372:1263-1271.
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8. Baris HN, Cohen IJ, Mistry PK. Gaucher disease: The metabolic defect, pathophysiology, phenotypes and natural history. Pediatr Endocrinol Rev. 2014;12:72-81.

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