



UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Gaucher Disease – Enzyme Replacement Therapy – Vpriv Utilization Management Medical Policy

- Vpriv[®] (velaglucerase intravenous infusion – Shire Human Genetic Therapies)

REVIEW DATE: 03/16/2022

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}

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Vpriv. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Vpriv as well as the monitoring required for adverse events and long-term efficacy, approval requires Vpriv to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Vpriv is recommended in those who meet the following criteria:

FDA-Approved Indication

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1. **Gaucher Disease.** Approve for 1 year if the patient meets the following criteria (A, B, and C):
 - A) Patient has Type 1 Gaucher disease; AND
 - B) The diagnosis is established by one of the following (i or ii):
 - i. Demonstration of deficient β -glucocerebrosidase activity in leukocytes or fibroblasts; OR
 - ii. Molecular genetic testing documenting glucocerebrosidase gene mutation; AND
 - C) Vpriv is 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 disorders.

Dosing. Each individual dose must not exceed 60 U/kg administered intravenously no more frequently than once every 2 weeks.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Vpriv is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Vpriv[®] intravenous infusion [prescribing information]. Lexington, MA: Shire Human Genetic Therapies; December 2020.
2. Burrow TA, Barnes S, and Grabowski GA. Prevalence and management of Gaucher disease. *Pediatric Health Med Ther.* 2011;2:59-73.
3. Cox T. Gaucher disease: clinical profile and therapeutic development. *Biologics.* 2010;4:299-313.
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.
6. Zimran A. How I treat Gaucher disease. *Blood.* 2011;118:1463-1471.
7. Stirnemann J, Belmatoug N, Camou F, et al. A review of Gaucher disease pathophysiology, clinical presentation and treatments. *Int J Mol Sci.* 2017;18:441.
8. Baris HN, Cohen IJ, Mistry PK. Gaucher disease: The metabolic defect, pathophysiology, phenotypes and natural history. *Pediatr Endocrinol Rev.* 2014;12:72-81.

HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	Gaucher Disease: Moved the designation of “Type 1” disease from indication to the criteria.	03/17/2021
Annual Revision	No criteria changes.	03/16/2022