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5.85.012

Section:	Prescription Drugs	**Effective Date:**	July 1, 2024
Subsection:	Hematological Agents	**Original Policy Date:**	September 8, 2011
Subject:	VPRIV	**Page:**	1 of 5

Last Review Date: June 13, 2024

VPRIV

Description

VPRIV (velaglucerase alfa)

Background
Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. Accumulation of lipids in these areas results in the enlargement of the liver and spleen, anemia, thrombocytopenia, lung disease and bone abnormalities. Symptoms of Gaucher disease usually become apparent in early childhood or adolescence but can be diagnosed at any stage of life. It is important to begin intervention early to prevent damage to the liver and spleen (1).

VPRIV is an injectable enzyme replacement product for the treatment of children and adults with type 1 Gaucher disease. There are three clinical subtypes of Gaucher disease differentiated by the presence or absence of neurological involvement: type 1, type 2 and type 3. Type 1, known as non-neuropathic, is the most common. There is insufficient evidence supporting the use of VPRIV for the treatment of type 2 and type 3 Gaucher disease (1).

Regulatory Status
FDA-approved indication: VPRIV is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease (2).

The most common adverse effects are infusion reactions and allergic reactions. Anaphylaxis has been observed in some patients (2).

The safety of VPRIV has not been established in pediatric patients younger than 4 years of age (2).

Related policies
Cerdelga, Cerezyme, Elelyso

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

VPRIV may be considered **medically necessary** if the conditions indicated below are met.

VPRIV may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 4 years of age or older

Diagnosis

Patient must have the following:

Gaucher disease, Type 1

AND the following:

1. **NO** dual therapy with another medication for Type 1 Gaucher disease (see Appendix 1)

Prior–Approval *Renewal* Requirements

Same as above

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 2 years

Prior–Approval *Renewal* Limits

Same as above

Rationale

Summary

Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. It is important to begin intervention early to prevent damage to the liver and spleen. VPRIV is a form of the human lysosomal enzyme, glucocerebrosidase, and is effective in replacing the enzyme deficiency in type 1 (non-neuronopathic) Gaucher disease (1-2).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of VPRIV while maintaining optimal therapeutic outcomes.

References

1. National Institute of Neurological Disorders and Stroke – National institute of health (NINDS-NIH) website. Gaucher disease information page.
2. VPRIV [Package Insert]. Cambridge, MA: Shire Human Genetic Therapies, Inc.; September 2021.

Policy History

Date	Action
September 2011	New policy
September 2012	Annual editorial and reference update.
March 2013	Annual editorial and reference update.
March 2014	Annual review and reference update
December 2014	Annual editorial and reference update.
December 2015	Annual editorial review and reference update Addition of no dual therapy with another hydrolytic lysosomal glucocerebroside agent
December 2016	Annual editorial review and reference update Policy number change from 5.10.12 to 5.85.12
September 2017	Annual editorial review and reference update
September 2018	Annual review and reference update
September 2019	Annual editorial review and reference update. Changed approval duration from lifetime to 2 years
September 2020	Annual review and reference update

December 2021	Annual review and reference update
December 2022	Annual review and reference update. Changed policy number to 5.85.012
June 2023	Annual review
June 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 13, 2024 and is effective on July 1, 2024.

Appendix 1 - List of Medications for Type 1 Gaucher Disease

Generic Name	Brand Name
eliglustat	Cerdelga
imiglucerase	Cerezyme
miglustat	Zavesca
taliglucerase alfa	Elelyso
velaglucerase alfa	VPRIV