Reference number(s)
2058-A

Specialty Guideline Management
VPRIV

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
VPRIV	velaglucerase alfa

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

VPRIV is indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.

Compendial Uses

- Gaucher disease type 2[5]
- Gaucher disease type 3[2-4]

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial request:

- Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).

Continuation request:

- Chat notes or medical records documenting benefit from therapy (e.g. improvement in liver volume, spleen volume, hemoglobin concentration, platelet count).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Gaucher disease type 1[1]

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when all of the following criteria are met:

- Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).

Gaucher disease type 2[5]

Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when all of the following criteria are met:

- Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).

Gaucher disease type 3[2-4]

Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when all of the following criteria are met:

- Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment of an indication listed in the Coverage Criteria section when all of the following criteria are met:

- Member meets the criteria for initial approval.
- The member is receiving benefit from therapy (e.g. improvement in liver volume, spleen volume, hemoglobin concentration, platelet count) and is not experiencing any intolerable adverse events.

References

1. VPRIV [package insert]. Lexington, MA: Takeda Pharmaceuticals U.S.A., Inc.; September 2024.
2. Pastores GM, Hughes DA. Gaucher Disease. 2000 July 27 [Updated December 7, 2023]. In: Adam MP, Everman DB, Mirzaa GM, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2023.
3. Kaplan P, Baris H, De Meirleir L, et al. Revised recommendations for the management of Gaucher disease in children. Eur J Pediatr. 2013;172:447-458.
4. Vellodi A, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. European Working Group on Gaucher Disease. J Inherit Metab Dis. 2009;32(5):660.
5. Gaucher Disease. National Organization for Rare Disorders. (2024). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.