

STANDARD MEDICARE PART B MANAGEMENT

VPRIV (velaglucerase alfa)

POLICY

I. 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.

A. FDA-Approved Indications

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

B. Compendial Uses

1. Gaucher disease type 2
2. Gaucher disease type 3

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:

For initial requests: beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis.

III. CRITERIA FOR INITIAL APPROVAL

A. Gaucher disease type 1

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

B. Gaucher disease type 2

Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

C. Gaucher disease type 3

Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:

- A. The member is currently receiving therapy with the requested medication.
- B. The requested medication is being used to treat an indication enumerated in Section III.
- C. The member is receiving benefit from therapy. Benefit is defined as not experiencing an inadequate response or any intolerable adverse events from therapy.

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for VPRIV.
2. The available compendium
 - a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
 - b. Micromedex DrugDex
 - c. American Hospital Formulary Service- Drug Information (AHFS-DI)
 - d. Lexi-Drugs
 - e. Clinical Pharmacology
3. Gaucher disease: GeneReviews
4. Revised recommendations for the management of Gaucher disease in children
5. Management of neuronopathic Gaucher disease: revised recommendations. European Working Group on Gaucher Disease

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for VPRIV are covered in addition to the following:

- A. Gaucher disease type 2
- B. Gaucher disease type 3

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

The diagnosis of Gaucher disease is made when deficient glucocerebrosidase (glucosylceramidase) enzyme activity in peripheral blood leukocytes or other nucleated cells or by the identification of biallelic pathogenic variants in *GBA* gene (Pastores and Hughes).

Support for using VPRIV to treat Gaucher disease type 2 can be found in the National Organization for Rare Disorders Guide to Rare Disorders. Enzyme replacement therapy (ERT) is effective for type 1 disease. Anemia and thrombocytopenia improve, hepatomegaly and splenomegaly are reduced, and skeletal damage is

ameliorated with ERT. These systemic manifestations also improve with ERT in patients with type 2 and 3 disease. However, it should be noted that ERT does not reverse brain damage in patients with type 2 disease.

Support for using VPRIV to treat Gaucher disease type 3 can be found in the Revised Recommendations for the Management of Gaucher Disease by Kaplan et al. The guideline indicates symptomatic children with types 1 or 3 disease should receive enzyme replacement therapy, which will prevent debilitating and often irreversible disease progression and allow those with non-neuropathic disease to lead normal healthy lives.

VII. REFERENCES

1. VPRIV [package insert]. Lexington, MA: Takeda Pharmaceuticals U.S.A., Inc.; September 2021.
2. Pastores GM, Hughes DA. Gaucher Disease. 2000 July 27 [Updated June 21, 2018]. In: Adam MP, Everman DB, Mirzaa GM, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2022.
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. National Organization for Rare Disorders. (2003). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.