

Reference number
2058-A

SPECIALTY GUIDELINE 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 Indication

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 are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

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

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

- A. Member meets the criteria for initial approval.
- B. Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

V. 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 March 9, 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, Tytki-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.