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 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 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

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 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.