Clinical Policy: Velaglucerase Alfa (VPRIV)

Reference Number: PA.CP.PHAR.163
Effective Date: 01/18
Last Review Date: 04/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for velaglucerase alfa (VPRIV®).

FDA Approved Indication(s)
VPRIV is indicated for long-term enzyme replacement therapy for patients with type 1 Gaucher disease (GD1).

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that VPRIV is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Type 1 Gaucher Disease (must meet all):

1. Diagnosis of Type 1 Gaucher disease confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity;
   b. DNA testing.
2. Age ≥ 4 years;
3. Member is symptomatic (e.g., anemia, thrombocytopenia, bone disease, hepatomegaly, splenomegaly);
4. Not prescribed concurrently with taliglucerase alfa or imiglucerase.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy

II. Continued Approval

A. Type 1 Gaucher Disease (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, decreased bone pain;
3. VPRIV is not prescribed concurrently with taliglucerase alfa or imiglucerase.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

Gaucher disease is an autosomal recessive disorder caused by mutations in the GBA gene, which results in a deficiency of the lysosomal enzyme beta-glucocerebrosidase. Glucocerebrosidase catalyzes the conversion of the sphingolipid glucocerebroside into glucose and ceramide. The enzymatic deficiency causes an accumulation of glucocerebroside primarily in the lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells". Velaglucerase alfa catalyzes the hydrolysis of glucocerebroside, reducing the amount of accumulated glucocerebroside. In clinical trials VPRIV reduced spleen and liver size, and improved anemia and thrombocytopenia. In this lysosomal storage disorder, clinical features are reflective of the accumulation of Gaucher cells in the liver, spleen, bone marrow, and other organs. The accumulation of Gaucher cells in the liver and spleen leads to organomegaly. Presence of Gaucher cells in the bone marrow and spleen lead to clinically significant anemia and thrombocytopenia.

Formulations:

VPRIV (velaglucerase alpha): Lyophilized product for reconstitution; for intravenous use

- 400 units/4 mL vial; 100 units/mL

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3385	Injection, velaglucerase alfa, 100 units

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: Added age restriction; Added requirement for presence of symptoms. Added examples of what can constitute a positive response to therapy.Added ERT monotherapy requirement for re-auth requests in addition to the initial criteria; references reviewed and updated.	04.02 .18	

References

1. VPRIV prescribing information. Lexington, MA: Shire Human Genetic Therapies, Inc.; April 2015. Available at http://pi.shirecontent.com/PI/PDFs/Vpriv_USA_ENG.pdf. Accessed February 26.2018.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with Type 1 Gaucher disease: Consensus recommendations. *J Pediatr.* 2004; 144: 112-20.
3. Hollak, CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: Therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. *Best Pract Res Clin Endocrinol Metab.* 2015; 29: 205-218.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. Semin Hematol. 2004; 41(suppl 5): 4-14.
5. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. Genet Med. 2005; 7(2): 105-110.
6. Altarescu G, Hill S, Wiggs E, et al. The efficacy of enzyme replacement therapy in patients with chronic neuronopathic Gaucher`s disease. J Pediatr. 2001;138:539-547.
7. Vellodi A, Tylki-Szymanska A, Davies E, et al. Management of neuronopathic Gaucher disease: Revised recommendations. J Inherit Metab Dis. 2009;32:660-664.
8.