Clinical Policy: Velaglucerase Alfa (VPRIV)

Reference Number: PA.CP.PHAR.163
Effective Date: 01/18
Last Review Date: 04/19

Coding Implications
Revision Log

Description
Velaglucerase alfa (VPRIV®) is a hydrolytic lysosomal glucocerebroside-specific enzyme.

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. Gaucher Disease (must meet all):

1. Diagnosis of Type 1 Gaucher disease or Type 3 Gaucher disease (GD3) 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. VPRIV is not prescribed concurrently with Elelyso® (taliglucerase alfa) or Cerezyme® (imiglucerase).

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

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 Elelyso® (taliglucerase alfa) or Cerezyme® (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.PMN.53

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
GD1: type 1 Gaucher disease
GD3: type 3 Gaucher disease

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s): none reported.
- Boxed warning(s): none reported.

Appendix D: General Information
- Measures of Therapeutic Response: GD1 is a heterogeneous disorder which involves the visceral organs, bone marrow, and bone in almost all affected patients. Common conditions resulting from GD1 include anemia, thrombocytopenia, hepatomegaly, splenomegaly, and bone disease. Therefore, hemoglobin level, platelet count, liver volume, spleen volume, and bone pain are clinical parameters that can indicate therapeutic response to GD1 therapies. In some clinical trials, stability has been defined as the following thresholds of change from baseline: hemoglobin level < 1.5 g/dL decrease, platelet count < 25% decrease, liver volume < 20% increase, and spleen volume < 25% increase.
- Enzyme replacement therapy such as Cerezyme may have beneficial palliative effects in Type 2 disease, but does not alter the outcome and is not generally used.
- According to the European consensus guidelines revised recommendations on the management of neuronopathic Gaucher disease by Vellodi et al: (1) there is clear evidence in most patients that enzyme replacement therapy (ERT) ameliorates systemic involvement in non-neuronopathic (Type 1) as well as chronic neuronopathic Gaucher disease (Type 3), enhancing quality of life; (2) There is no evidence that ERT has reversed, stabilized or slowed the progression of neurological involvement; (3) In patients with established acute neuronopathic Gaucher disease (Type 2), enzyme replacement therapy has had little effect on the progressively downhill course. It has merely resulted in prolongation of pain and suffering.
- There is currently insufficient clinical evidence that supports the combination use of enzyme replacement therapy with Zavesca® (miglustat).

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Gaucher Disease	Patients naïve to enzyme replacement therapy: 60 units/kg IV every other week Patients being treated with stable imiglucerase dosages: Switch to VPRIV at previous imiglucerase dose 2 weeks after last imiglucerase dose	Individualized

V. Product Availability

Single-use vial: 400 units

VI. References

1. VPRIV Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; April 2015. Available at http://www.vpriv.com. Accessed February 27, 2019.
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.

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	04.02 .18	

Reviews, Revisions, and Approvals	Date	Approval Date
response to therapy.Added ERT monotherapy requirement for re-auth requests in addition to the initial criteria; references reviewed and updated.		
2Q 2019 annual review: added coverage for Type 3 Gaucher disease; references reviewed and updated,	04/19	