Clinical Policy: Velaglucerase Alfa (VPRIV)

Reference Number: ERX.SPA.98
Effective Date: 10.01.16
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ 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 (GD1) or type 3 (GD3) Gaucher disease confirmed by one of the following (a or b):
   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

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
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 a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.

Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ERT: enzyme replacement therapy
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

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 VPRIV 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) or Cerdelga® (eliglustat), or concurrent use of two or more enzyme replacement therapies at once.

V. 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

VI. Product Availability

Single-use vial: 400 units

VII. References

1. VPRIV Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; November 2019. Available at http://www.vpriv.com. Accessed February 5, 2020.
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.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added age restriction per PI. Modified approval duration to 6 months for initial and 12 months for re-auth.	08.16	09.16
Converted to new template. Initial: Added prescriber requirement and ERT monotherapy requirement. Added DNA testing to diagnostic methods. Re-auth: Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Initial: Removed prescriber requirement. Added requirement for presence of symptoms. Re-auth: Added ERT monotherapy requirement.	09.11.17	11.17
2Q 2018 annual review: Added coverage for type 3 Gaucher disease; References reviewed and updated.	02.26.18	05.18
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.27.19	05.19
2Q 2020 annual review: added specific examples of positive response to therapy, for reauthorization; references reviewed and updated.	02.05.20	05.20

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.