RXADVANCE
2 Park Central Drive
Southborough, MA 01772

Clinical Policy Title:	velaglucerase alfa
Policy Number:	RxA.560
Drug(s) Applied:	VPRIV®
Original Policy Date:	03/06/2020
Last Review Date:	12/07/2020
Line of Business Policy Applies to:	All lines of business

Background

Velaglucerase alfa (VPRIV®) is a hydrolytic lysosomal glucocerebroside-specific enzyme. It is indicated for long-term enzyme replacement therapy for patients with type 1 Gaucher disease (GD1).

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
velaglucerase alfa (VPRIV®)	Gaucher Disease	Patients naive 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

Dosage Forms

- Single-use vial: 400 units

Clinical Policy

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

I. Initial Approval Criteria

A. Gaucher Disease (must meet all):

1. Diagnosis of Type 1 (GD1) or Type 3 Gaucher disease (GD3) 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

Commercial: 6 months

Medicaid: 6 months

II. Continued Therapy Approval

A. Gaucher Disease (must meet all):

1. Currently receiving medication that has been authorized by RxAdvance or member has previously met initial approval criteria listed in this policy.
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
Commercial: 6 months
Medicaid: 12 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
GD1: type 1 Gaucher disease
GD3: type 3 Gaucher disease
ERT: enzyme replacement therapy

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), or Cerdelga® (eliglustat), or concurrent use of two or more enzyme

replacement therapies at once.

References

1. VPRIV® Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; November 2019. Available at http://www.vpriv.com. Accessed September 23, 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. Accessed September 23, 2020.
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. Accessed September 23, 2020.
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. Accessed September 23, 2020.
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. Accessed September 23, 2020.
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. Accessed September 23, 2020.
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. Accessed September 23, 2020.
8. Gary SE, Ryan E, Steward AM, Sidransky E. Recent advances in the diagnosis and management of Gaucher disease. Expert Rev Endocrinol Metab. 2018; 13(2): 107-118. doi:10.1080/17446651.2018.1445524. Accessed October 5, 2020.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	01/2020	03/06/2020
Policy was reviewed: 1. Policy title table was updated: Line of business policy applies was updated to all lines of business. 2. Continued therapy criteria II.A.1 was rephrased to “Currently receiving medication that has been authorized by RxAdvance…”. 3. HIM was removed from initial and continued therapy approval. 4. Appendix A was updated to include ERT. 5. Appendix D for general information was updated to include additional information. 6. References were updated.	10/05/2020	12/07/2020