Clinical Policy: Velaglucerase Alfa (VPRIV)

Reference Number: HIM.PA.163
Effective Date: 01.01.22
Last Review Date: 05.22
Line of Business: HIM

Coding Implications
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.

It is the policy of health plans affiliated with Centene Corporation® 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 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. One of the following (a or b):
   a. Request is for GD1: Failure of Cerezyme® or Cerdelga®, unless clinically significant adverse effects are experienced or both are contraindicated;
   b. Request is for GD3: Failure of Cerezyme, unless contraindicated or clinically significant adverse effects are experienced;

   **Prior authorization may be required for Cerezyme and Cerdelga*
5. VPRIV is not prescribed concurrently with Elelyso® (taliglucerase alfa) or Cerezyme (imiglucerase).

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): HIM.PA.154 for health insurance marketplace.

II. Continued Therapy

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

1. Currently receiving medication via Centene benefit 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, or 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 health plan benefit and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): HIM.PA.154 for health insurance marketplace.

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 – HIM.PA.154 for health insurance marketplace 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

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Cerdelga (eliglustat)	**GD1:** CYP2D6 EM, IM: 84 mg PO BID CYP2D6 PM: 84 mg PO QD	CYP2D6 EM, IM: 168 mg/day CYP2D6 PM: 84 mg/day
Cerezyme (imiglucerase)	**GD1 or GD3:** Individualize to each patient; initial dose ranges from 2.5 U/kg via IV infusion 3 times a week to 60 U/kg once every 2 weeks; disease severity may dictate	Individualized

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
	treatment be initiated at relatively high dose or relatively frequent administration	

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic

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 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 The dosage can be adjusted based on achievement and maintenance of each patient's therapeutic goals. Patients being treated with stable imiglucerase dosages:	Individualized

Indication	Dosing Regimen	Maximum Dose
	Switch to VPRIV at previous imiglucerase dose 2 weeks after last imiglucerase dose	

VI. Product Availability

Single-use vial: 400 units

VII. References

1. VPRIV Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; September 2021. Available at http://www.vpriv.com. Accessed February 24, 2022.
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. Gary SE, Ryan E, Steward AM, et al. Recent advances in the diagnosis and management of Gaucher disease. Expert Rev Endocrinol Metab. 2018 Mar;13(2):107–118.

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	P&T Approval Date
Policy created per March SDC; adapted from CP.PHAR.163.	08.11.21	11.21
2Q 2022 annual review: no significant changes; references reviewed and updated.	02.24.22	05.22

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. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members

and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.