Clinical Policy: Velaglucerase Alfa (VPRIV)

Reference Number: CP.PHAR.163
Effective Date: 02/16
Last Review Date: 02/17

Coding Implications
Revision Log

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

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

Policy/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. 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. Not prescribed concurrently with taliglucerase alfa or imiglucerase.

Approval duration: 6 months

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

II. Continued Approval

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

1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria;
2. Member is responding positively to therapy.

Approval duration: 12 months

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy; or
2. Refer to 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

FDA Approved Indications:
VPRIV is a hydrolytic lysosomal glucocerebroside-specific enzyme/intravenous formulation indicated for:
- Long-term enzyme replacement therapy for patients with type 1 Gaucher disease.

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
Policy split from CP.PHAR.48. Policy converted to new template.	01/16	02/16
Age restriction removed. DNA testing added to diagnostic methods. Allergy history is removed as the drug can be continued in some cases. ERT monotherapy added. Positive response added. Background section converted to new template.	12/16	02/17

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 December 21, 2016.

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.

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.

Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

Note: For Medicare members, to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs, LCDs, and Medicare Coverage Articles should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at http://www.cms.gov for additional information.