

VPRIV[®] (velaglucerase alfa) (Intravenous)

Document Number: MODA-0141

Last Review Date: 09/05/2023

Date of Origin: 01/01/2012

Dates Reviewed: 12/2011, 02/2013, 02/2014, 09/2014, 07/2015, 07/2016, 08/2017, 07/2018, 07/2019, 07/2020, 09/2021, 09/2022, 09/2023

I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- VPRIV 400 unit powder for injection: 18 vials per 14 days

B. Max Units (per dose and over time) [HCPC Unit]:

- 72 billable units every 14 days

III. Initial Approval Criteria ¹

Site of care specialty infusion program requirements are met (refer to [Moda Site of Care Policy](#)).

Coverage is provided in the following conditions:

- Patient is at least 4 years of age; **AND**

Universal Criteria ¹

- Used as a single agent; **AND**

Type 1 Gaucher Disease † Φ ^{1,6,12-16}

- Patient has a documented diagnosis of Type 1 Gaucher Disease confirmed by one of the following:
 - Significantly reduced or absent glucocerebrosidase enzyme activity as measured by a beta-glucosidase leukocyte (BGL) test
 - Detection of mutations in the glucocerebrosidase (*GBA*) gene; **AND**
- Patient's disease results in one or more of the following:
 - Anemia-related symptoms [i.e., blood transfusion dependency and/or hemoglobin \leq 11 g/dL (women and children) or \leq 12 g/dL (men)]
 - Thrombocytopenia (platelet count \leq 120,000/mm³)

- Hepatomegaly or splenomegaly
- Skeletal disease (e.g., lesions, remodeling defects and/or deformity of long bones, osteopenia/osteoporosis, etc.)
- Symptomatic disease (e.g., bone pain, fatigue, dyspnea, abdominal distension, diminished quality of life, etc.)

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria ^{1,6,11,13-16}

Coverage can be renewed based on the following criteria:

- Patient continues to meet the universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Disease response with treatment as defined by one or more of the following (compared to pre-treatment baseline):
 - Improvement in anemia-related symptoms (i.e., improvement in hemoglobin and/or decrease in blood transfusion dependency)
 - Improvement in platelet counts
 - Reduction in size of liver or spleen
 - Improvement in skeletal disease (e.g., increase in lumbar spine and/or femoral neck BMD, no bone crises or bone fractures, etc.)
 - Improvement in symptoms (e.g., bone pain, fatigue, dyspnea, abdominal distension, quality of life, etc.); **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions, including anaphylaxis, etc.

V. Dosage/Administration¹

Indication	Dose
Type 1 Gaucher Disease	<ul style="list-style-type: none"> - Administer up to 60 units/kg every other week as an intravenous infusion. - In patients switching from a stable imiglucerase dosage to VPRIV, initiate VPRIV intravenous treatment at the previous imiglucerase dosage two weeks after the last imiglucerase dose. - The dosage can be adjusted based on achievement and maintenance of each patient's therapeutic goals.

VI. Billing Code/Availability Information

HCPCS Code:

- J3385 – Injection, velaglucerase alfa, 100 units; 1 billable unit = 100 units

NDC:

- VPRIV 400 unit powder for injection, single-dose vial: 54092-0701-xx

VII. References

1. VPRIV [package insert]. Cambridge, MA; Shire Human Genetic Therapies; September 2021. Accessed August 2023.
2. Anderson HC, et al. Consensus Statement by the International Collaborative Gaucher Group (ICGG) U.S. Coordinators on Individualization of ERT for Type-1 Gaucher Disease. September, 2000.
3. Charrow, et al. Gaucher Disease: Recommendations on Diagnosis, Evaluation and Monitoring (Special Article). *Archives of Internal Medicine* 1998; 158:1754-1760.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. *Semin Hematol* 2004; 41:4.
5. Baldellou A, Andria G, Campbell PE, et al. Paediatric non-neuronopathic Gaucher disease: recommendations for treatment and monitoring. *Eur J Pediatr* 2004; 163:67.
6. Charrow J, Andersson HC, Kaplan P, et al. The Gaucher Registry: Demographics and disease characteristics of 1698 patients with Gaucher disease. *Arch Intern Med* 2000; 160:2835.
7. Martins AM, Valadares ER, Porta G, et al. Recommendations on diagnosis, treatment, and monitoring for Gaucher disease. *J Pediatr*. 2009 Oct;155(4 Suppl):S10-8.
8. Balwani M, Burrow TA, Charrow J, et al. Recommendations for the use of eliglustat in the treatment of adults with Gaucher disease Type 1 in the United States. *Mol Genet Metab*. 2016 Feb; 117(2):95-103. Doi: `0.1016/j.ymgme.2015.09.002. Epub 2015 Sep 7.
9. Gonzalez DE, Turkia HB, Lukina EA, et al. Enzyme Replacement Therapy With Velaglucerase Alfa in Gaucher Disease: Results From a Randomized, Double-Blind, Multinational, Phase 3 Study. *Am J Hematol* 2013 Mar;88(3):166-71. doi:10.1002/ajh.23381. Epub 2013 Feb 6.
10. Zimran A, Pastores G, Szymanska A, et al. Safety and efficacy of velaglucerase alfa in Gaucher disease type 1 patients previously treated with imiglucerase. *Am J Hematol*. Author manuscript; available in PMC 2014 Mar 1. Published in final edited form as: *Am J Hematol*. 2013 Mar; 88(3): 172–178. Published online 2013 Jan 22. doi: 10.1002/ajh.23383.
11. Biegstraaten M, Cox TM, Belmatoug N, et al. Management goals for type 1 Gaucher disease: An expert consensus document from the European working group on Gaucher disease. *Blood Cells, Molecules and Diseases* 68 (2018) 203-208.
12. DuaPuri R, Kapoor S, Kishnani PS, et al. Diagnosis and Management of Gaucher Disease in India – Consensus Guidelines of the Gaucher Disease Task Force of the Society for Indian Academy of Medical Genetics and the Indian Academy of Pediatrics. *Indian Pediatrics*. 5;2018: 143-153.

13. Kaplan P, Andersson HC, Kacena KA, Yee JD. The clinical and demographic characteristics of nonneuronopathic Gaucher disease in 887 children at diagnosis. *Arch Pediatr Adolesc Med.* 2006 Jun;160(6):603-8.
14. Pastores GM, Hughes DA. Gaucher Disease. GeneReviews®. www.ncbi.nlm.nih.gov/books/NBK1269/. Initial Posting: July 27, 2000; Last Revision: March 9, 2023. Accessed on August 7, 2023.
15. Hughs D, Sidransky E. (2022). Gaucher disease: Pathogenesis, clinical manifestations, and diagnosis. In Hahn S, TePas E (Eds.), *UptoDate*. Last updated: June 29, 2022. Accessed on August 7, 2023. Available from https://www.uptodate.com/contents/gaucher-disease-pathogenesis-clinical-manifestations-and-diagnosis?search=gaucher%20disease%20type%201&source=search_result&selectedTitle=1~150&usage_type=default&display_rank=1#H11.
16. Hughs D, Sidransky E. (2022). Gaucher disease: Treatment. In Hahn S, TePas E (Eds.), *UptoDate*. Last updated: August 02, 2022. Accessed on August 15, 2023. Available from https://www.uptodate.com/contents/gaucher-disease-treatment?sectionName=ENZYME%20REPLACEMENT%20THERAPY&search=gaucher%20disease%20type%201&topicRef=2918&anchor=H4&source=see_link#H15131002.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E75.22	Gaucher disease

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Local Coverage Articles may exist and compliance with these policies is required where applicable. They can be found at: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	Applicable State/US Territory	Contractor
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)

Medicare Part B Administrative Contractor (MAC) Jurisdictions

Jurisdiction	Applicable State/US Territory	Contractor
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC