

VPRIV® (velaglucerase alfa) (Intravenous)

Document Number: OHSU HEALTHSERVICES-0141

Last Review Date: 04/01/2026

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, 09/2024, 04/2025, 04/2026

I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for 12 months (365 days).
- Renewal: Prior authorization validity may be renewed every 12 months (365 days) thereafter.

II. Dosing Limits

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

- 72 billable units every 14 days

III. Initial Approval Criteria ¹

Prior authorization validity is provided in the following conditions:

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

Universal Criteria ¹

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

Type 1 Gaucher Disease † Φ ^{1,6,12-17}

- Member 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**
- Member's disease results in one or more of the following:
 - Anemia-related symptoms [i.e., blood transfusion dependency and/or hemoglobin ≤ 11 g/dL (women and children) or ≤ 12 g/dL (men)]
 - Thrombocytopenia (platelet count $\leq 120,000/\text{mm}^3$)

- 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); Ⓢ Orphan Drug

IV. Renewal Criteria ^{1,6,11,13-17}

Prior authorization validity can be renewed based on the following criteria:

- Member 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**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions, including anaphylaxis, etc.; **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.)

V. Dosage/Administration ¹

Indication	Dose
Type 1 Gaucher Disease	– Administer up to 60 units/kg every other week as an intravenous infusion. – In members 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 member'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; Takeda Pharmaceuticals U.S.A., Inc.; September 2024. Accessed February 2026.
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 GM, Tytki-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*. 2018 Feb 15;55(2):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. Hughes DA, Pastores GM. Gaucher Disease. *GeneReviews*®. www.ncbi.nlm.nih.gov/books/NBK1269/. Initial Posting: July 27, 2000; Last Update: December 7, 2023. Accessed on February 25, 2026.
15. Hughs D, Sidransky E. (2025). Gaucher disease: Pathogenesis, clinical manifestations, and diagnosis. In Kaplan SL, Kremen J (Eds.), *UptoDate*. Last updated: August 6, 2024. Accessed on February 25, 2026. 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. (2026). Gaucher disease: Treatment. In Sutton VR, Kremen J (Eds.), *UptoDate*. Last updated: January 29, 2026. Accessed on February 25, 2026. 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.
17. Weinreb, NJ, Aggio MC, Andersson, et al. Gaucher disease type 1: Revised recommendations on evaluation and monitoring for adult patients. *Semin Hemat*. 2004;41(4 Suppl 5):15.

Appendix A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime’s assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	Yes: Consider for PA
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

Appendix 1 – Covered Diagnosis Codes

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

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

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications, including any preceding information, may be applied 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)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
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