

## Elelyso® (taliglucerase alfa) (Intravenous)

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### I. Length of Authorization

- Initial: Prior authorization validity will be provided for 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) [HCPCS Unit]:**

- 700 billable units every 14 days

### III. Initial Approval Criteria <sup>1</sup>

For groups in scope for the Site of Care (SOC) specialty infusion program, all program requirements are met.

Prior authorization validity is provided in the following conditions:

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

**Universal Criteria <sup>1</sup>**

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

**Type 1 Gaucher Disease † Φ <sup>1,6,13-18</sup>**

- 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  $\leq 11$  g/dL (women and children) or  $\leq 12$  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 <sup>1,6,12,14-18</sup>

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 <sup>1</sup>

| Indication             | Dose                                                                                                                                                                                                                                                                                                                                                |
|------------------------|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Type 1 Gaucher Disease | <ul style="list-style-type: none"> <li>– Administer up to 60 units/kg every other week as an intravenous infusion.</li> <li>– In members switching from a stable imiglucerase dosage to Elelyso, initiate Elelyso intravenous treatment with the same units/kg imiglucerase dosage and subsequently administer Elelyso every other week.</li> </ul> |

| Indication | Dose                                                                                                      |
|------------|-----------------------------------------------------------------------------------------------------------|
|            | – Dosage adjustments can be made based on achievement and maintenance of each member's therapeutic goals. |

## VI. Billing Code/Availability Information

### HCPCS Code:

- J3060 – Injection, taliglucerase alfa, 10 units; 1 billable unit = 10 units

### NDC:

- Eleyso 200 unit powder for injection, single-dose vial: 00069-0106-xx

## VII. References

1. Eleyso [package insert]. New York, NY; Pfizer Inc.; December 2025. 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. Kaplan P, Baris H, De Meirleir L, et al. Revised recommendations for the management of Gaucher disease in children. Eur J Pediatr. 2013 Apr;172(4):447-58: doi: 10.1007/s00431-012-1771-z. Epub 2012 Jul 8.
9. Zimran A, Duran G, Mehta A, et al. Long-term Efficacy and Safety Results of Taliglucerase Alfa Up to 36 Months in Adult Treatment-Naïve Patients With Gaucher Disease. Am J Hematol. 2016 Jul;91(7):656-60. doi: 10.1002/ajh.24369. Epub 2016 Apr 24.
10. Zimran A, Brill-Almon E, Chertkoff R, et al. Pivotal Trial With Plant Cell-Expressed Recombinant Glucocerebrosidase, Taliglucerase Alfa, a Novel Enzyme Replacement Therapy for Gaucher Disease. Blood 2011 Nov 24;118(22):5767-73. doi: 10.1182/blood-2011-07-366955. Epub 2011 Sep 6.

11. Pastores GM, Petakov M, Giraldo P, et al. A Phase 3, Multicenter, Open-Label, Switchover Trial to Assess the Safety and Efficacy of Taliglucerase Alfa, a Plant Cell-Expressed Recombinant Human Glucocerebrosidase, in Adult and Pediatric Patients With Gaucher Disease Previously Treated With Imiglucerase. *Blood Cells Mol Dis* 2014 Dec;53(4):253-60. doi: 10.1016/j.bcmd.2014.05.004. Epub 2014 Jun 18.
12. 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.
13. 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.
14. 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.
15. Hughes DA, Pastores GM. Gaucher Disease. GeneReviews®. [www.ncbi.nlm.nih.gov/books/NBK1269/](http://www.ncbi.nlm.nih.gov/books/NBK1269/). Initial Posting: July 27, 2000; Last Update: December 7, 2023. Accessed on February 24, 2026.
16. 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 24, 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](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).
17. Hughs D, Sidransky E. (2026). Gaucher disease: Treatment. In Sutton VR, Kremen J (Eds.), *UptoDate*. Last updated: January 29, 2026. Accessed on February 24, 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](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).
18. 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                           |

