

Medical Policy Manual **Approved Rev: Do Not Implement until 10/31/26**

Taliglucerase Alfa (Elelyso®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Elelyso is indicated for the treatment of patients 4 years and older with a confirmed diagnosis of Type 1 Gaucher disease.

Compendial Uses

- Gaucher disease type 2
- Gaucher disease type 3

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

- Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- **Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).**

Continuation Requests:

- **Chart notes or medical records documenting benefit from therapy (e.g. improvement in liver volume, spleen volume, hemoglobin concentration, platelet count).**

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

COVERAGE CRITERIA

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Gaucher disease type 1

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when **all of the following criteria are met:**

- Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
- **Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).**

Gaucher disease type 2

Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when **all of the following criteria are met:**

- Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
- **Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).**

Gaucher disease type 3

Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when **all of the following criteria are met:**

- Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
- **Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).**

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment of an indication listed in coverage criteria section when all of the following criteria are met:

- Member meets the criteria for initial approval.
- **The member is receiving benefit from therapy (e.g. improvement in live volume, spleen volume, hemoglobin concentration, platelet count) and is not experiencing any intolerable adverse events.**

MEDICATION QUANTITY LIMITS

Drug Name	Diagnosis	Maximum Dosing Regimen
Elelyso (Taliglucerase alfa)	Gaucher Disease	Route of Administration: Intravenous 60Units/kg every 2 weeks

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.



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ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Eluelyso [package insert]. New York, NY: Pfizer, Inc; **January 2025**.
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3. Pastores GM, Hughes DA. Gaucher Disease. 2000 July 27 [Updated December 7, 2023]. In: Adam MP, Everman DB, Mirzaa GM, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2023.
4. Kaplan P, Baris H, De Meirleir L, et al. Revised recommendations for the management of Gaucher disease in children. *Eur J Pediatr*. 2013;172:447-458.
5. Vellodi A, Tytki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. European Working Group on Gaucher Disease. *J Inherit Metab Dis*. 2009;32(5):660.
6. Gaucher Disease. National Organization for Rare Disorders. (2024). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.

EFFECTIVE DATE 10/31/26

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