Reference number(s)
2053-A

Specialty Guideline Management
Elelyso

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Elelyso	taliglucerase alfa

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[1]

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[6]
- Gaucher disease type 3[3-5]

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: beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.

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Prescriber Specialties

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

Gaucher disease type 1[1,2]

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

Gaucher disease type 2[6]

Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

Gaucher disease type 3[3-5]

Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

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.
- Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

References

1. Elelyso [package insert]. New York, NY: Pfizer, Inc; July 2024.
2. 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;118:5767-5773.

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, Tylki-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.