CVS Caremark®

|  |
| --- |
| 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 Indications1

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 26
* Gaucher disease type 33-5

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.

## 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 11,2

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 26

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 33-5

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.