

ElELYso® (taliglucerase alfa)

When requesting ElELYso® (taliglucerase alfa), the individual requiring treatment must be diagnosed with the following FDA-approved indication or approved off-label indication and meet the specific coverage guidelines and applicable safety criteria for the covered indications.

FDA-Approved Indication

ElELYso is indicated for the treatment of individuals with Type 1 Gaucher disease.

Approved Off-Label Compendial Use

ElELYso is indicated for the treatment of individuals with Type 3 Gaucher disease.

Coverage Guidelines

Type 1 Gaucher Disease

The individual must meet **all** of the following criteria for approval:

- The patient is at least 4 years of age or older;
- The diagnosis is established by one of the following:
 - Demonstration of deficient β - glucocerebrosidase activity in leukocytes or fibroblasts; OR
 - Molecular genetic testing documenting biallelic pathogenic variance in the glucocerebrosidase (GBA) gene;
- ElELYso is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Type 3 Gaucher Disease

The individual must meet **all** of the following criteria for approval:

- The patient is at least 4 years of age or older;
- The diagnosis is established by one of the following:
 - Demonstration of deficient β - glucocerebrosidase activity in leukocytes or fibroblasts; OR
 - Molecular genetic testing documenting biallelic pathogenic variance in the glucocerebrosidase (GBA) gene;
- The patient meets **both** of the following:
 - Medication is not being used for the management of neurological manifestations; AND

- Medication is being used for the management of impaired growth, hematologic, or visceral symptoms;
- Elelyso is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 1 year

Dosing Recommendations

Type 1 Gaucher Disease

The recommended dosage is 60 units/kg administered intravenously every other week.

Type 3 Gaucher Disease

The recommended dosage should not exceed 120 units/kg administered intravenously once every two weeks.

References

1. Elelyso® for injection [prescribing information]. New York, NY: Pfizer; May 2023.
2. Burrow TA, Barnes S, and Grabowski GA. Prevalence and management of Gaucher disease. *Pediatric Health Med Ther.* 2011;2:59-73.
3. Cox T. Gaucher disease: clinical profile and therapeutic development. *Biologics.* 2010;4:299-313.
4. Jmoudiak, M. and Futerman, AH. Gaucher disease: Pathological mechanisms and modern management. *Br J Haematol.* 2005;129(2):178–188.
5. Grabowski GA. Lysosomal storage disease 1- phenotype, diagnosis, and treatment of Gaucher's disease. *Lancet.* 2008;372:1263-1271.
6. Zimran A. How I treat Gaucher disease. *Blood.* 2011;118:1463-1471.
7. Stirnemann J, Belmatoug N, Camou F, et al. A review of Gaucher disease pathophysiology, clinical presentation and treatments. *Int J Mol Sci.* 2017;18:441.
8. Baris HN, Cohen IJ, Mistry PK. Gaucher disease: The metabolic defect, pathophysiology, phenotypes and natural history. *Pediatr Endocrinol Rev.* 2014;12:72-81.
9. Kishnani PS, Al-Hertani W, Balwani M, et al. Screening, patient identification, evaluation, and treatment in patients with Gaucher disease: Results from a Delphi consensus. *Mol Genet Metab.* 2022 Feb;135(2):154-162.
10. 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.
11. Vellodi A, Tytki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. *J Inherit Metab Dis.* 2009 Oct;32(5):660-664.