

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

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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, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. *J Inherit Metab Dis.* 2009 Oct;32(5):660-664.