Drug Coverage Policy

Effective Date.....6/15/2026
Coverage Policy Number.....IP0163
Policy Title.....Elelyso

Gaucher Disease – Enzyme Replacement Therapy – Elelyso

- Elelyso® (taliglucerase intravenous infusion – Pfizer)

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

OVERVIEW

Elelyso, an analogue of β-glucocerebrosidase, is indicated for the treatment of a confirmed diagnosis of **Type 1 Gaucher disease** in patients ≥ 4 years of age.[1] The recommended dose is 60 U/kg every other week administered intravenously.

Disease Overview
Gaucher disease is a rare autosomal recessive, inherited, lysosomal storage disorder caused by a deficiency of the lysosomal enzyme β-glucocerebrosidase.[2-4] Glucocerebrosidase is responsible for the breakdown of glucosylcerebroside (GluCer) into glucose and ceramide. The deficiency of this enzyme is characterized by an excessive accumulation of GluCer in the visceral organs such as the liver, spleen, and bone marrow. GluCer remains stored within lysosomes causing enlarged lipid-laden macrophages called "Gaucher cells".

Gaucher disease is classified into three phenotypes (Types 1 through 3).[2-5] Type 1 is a non-neuronopathic variant with asymptomatic or symptomatic clinical manifestations of splenomegaly, hepatomegaly, anemia, thrombocytopenia, skeletal complications, and occasional lung involvement. Although historically Type 1 was characterized by the absence of neurological involvement, the prevalence of peripheral neuropathy in adults with Type 1 Gaucher disease has been reported to be higher than the general population.[12] In addition, evidence suggests that central nervous system involvement may also occur. The risk of Parkinson's disease is increased in patients with Type 1 Gaucher disease and has a more aggressive course than in individuals without Gaucher disease. Further, patients with Type 1 disease may also have evidence of impaired cognitive function, sleep disturbance, hallucinations, apraxia, functional and structural eye abnormalities, and impaired sense of smell. Type 2 is an acute neuronopathic form characterized by an early onset (3 to 6 months of age) of rapidly progressive neurological disease with visceral manifestations; death generally occurs by the time patients reach 1 to 2 years of age. Type 3 is referred to as a chronic neuronopathic form and characterized by a later onset. Patients present with neurological, hematological, and visceral symptoms. Type 1 is most prevalent in the Western world, accounting for an estimated 94% of patients with Gaucher disease.[2,6] Types 2 and 3 represent < 1% and 5%, respectively, in Europe, North America, and Israel.[2,5] The diagnosis of Gaucher disease is established by demonstrating deficient β-glucocerebrosidase activity in leukocytes or fibroblasts, or mutations in the glucocerebrosidase gene.[7,8]

Guidelines
Treatment guidelines for Type 1 Gaucher disease (non-neuronopathic form) recommend initiating enzyme replacement therapy (ERT) in patients with significant and/or progressive disease.[9,10] Additionally, ERT should be initiated immediately in all patients with Type 3 Gaucher disease (chronic neuronopathic form) at a starting dose of 60 U/kg every other week as soon as possible after diagnosis in children with GD3, or at 30 to 60 U/kg every other week in adults.[10,11] Guidelines note that there is no evidence that ERT has reversed, stabilized, or slowed the progression of neurological involvement.[11] However, ERT ameliorates systemic involvement (skeletal deterioration, visceromegaly, hematological abnormalities) in non-neuronopathic as well as chronic neuronopathic disease, ultimately enhancing the quality of life. Additionally, it is noted that higher doses may be needed to control visceral symptoms associated with chronic neuronopathic disease.

Coverage Policy

POLICY STATEMENT
Prior Authorization is required for benefit coverage of Elelyso. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indications. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Elelyso as well as the monitoring required for adverse events and long-term efficacy,

approval requires Elelyso to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required where noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, laboratory tests, claims records, prescription receipts and/or other information. All documentation must include patient-specific identifying information.

Elelyso is considered medically necessary when the following criteria are met:

FDA-Approved Indication

1. **Gaucher Disease – Type 1.** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
   Note: Type 1 Gaucher disease is also known as non-neuronopathic Gaucher disease.
   A) Patient is ≥ 4 years of age; AND
   B) The diagnosis is established by ONE of the following (i or ii):
      i. Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts **[documentation required]**; OR
      ii. Molecular genetic testing documenting biallelic pathogenic variants in the glucocerebrosidase *(GBA)* gene **[documentation required]**; AND
   C) The medication is not being used for the management of neurological manifestations; AND
   Note: Examples of neurological manifestations may include abnormal ocular movement, auditory impairment, cognitive impairment, and seizures.
   D) The medication 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

   Dosing. Each individual dose must not exceed 60 U/kg administered intravenously no more frequently than once every 2 weeks.

Other Uses with Supportive Evidence

2. **Gaucher Disease – Type 3.** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
   Note: Type 3 Gaucher disease is also known as chronic neuronopathic Gaucher disease.
   A) Patients is ≥ 4 years of age; AND
   B) The diagnosis is established by ONE of the following (i or ii):
      i. Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts **[documentation required]**; OR
      ii. Molecular genetic testing documenting biallelic pathogenic variants in the glucocerebrosidase (*GBA*) gene **[documentation required]**; AND
   C) Medication is not being used for the management of neurological manifestations; AND
      Note: Examples of neurological manifestations may include abnormal ocular movement, auditory impairment, cognitive impairment, and seizures.
   D) The medication 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.

Dosing. Each individual dose must not exceed 60 U/kg administered intravenously no more frequently than once every 2 weeks

Conditions Not Covered

Elelyso for any other use is considered not medically necessary, including the following (this list may not be all inclusive; criteria will be updated as new published data are available):

1. **Concomitant Use with Other Approved Therapies for Gaucher Disease.** Concomitant use with other treatments approved for Gaucher disease has not been evaluated. Of note, examples of medications approved for Gaucher disease include Cerdelga (eliglustat capsules), Cerezyme (imiglucerase intravenous infusion), Vpriv (velaglucerase alfa intravenous infusion), and Zavesca (miglustat capsules).

Coding Information

Note: 1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J3060	Injection, taliglucerase alfa, 10 units

References

1. Elelyso® intravenous infusion [prescribing information]. New York, NY: Pfizer; December 2025.
2. Burrow TA, Barnes S, and Grabowski GA. Prevalence and management of Gaucher disease. *Pediatric Health Med Ther*. 2011;2:59-73.
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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, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. *J Inherit Metab Dis.* 2009 Oct;32(5):660-664.
12. Weinreb NJ, Goker-Alpan O, Krishnani PS, et al. The diagnosis and management of Gaucher disease in pediatric patients: Where do we go from here? *Mol Genet Metab.* 2022;136:4-21.

Revision Details

Summary of Changes	Review Date	Effective Date
Policy Name Change: Updated Policy Name from "Taliglucerase" to "Gaucher Disease – Enzyme Replacement Therapy – Elelyso." **Gaucher Disease – Type 1: Added** qualifier "Type 1" to the condition name and Note to indicate Type 1 disease is also referred to as non-neuronopathic disease. **Removed** statement "… or type 3 Gaucher disease that results in at least one of the following: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly." **Added** age requirement of 4 years or older. **Added** dosing information. **Gaucher Disease – Type 3**: **Added** the new condition of approval under other uses with supportive evidence.	8/8/2024	10/15/2024
No criteria changes	4/17/2025	6/15/2025
Gaucher Disease – Type 1 and – Type 3: **Updated** diagnostic criteria with documentation requirements specified.	3/19/2026	5/1/2026
Gaucher Disease – Type 1: A requirement was added that the medication is not being used for the management of neurological manifestations. **Gaucher Disease – Type 3:** The requirement that the medication is being used for the management of impaired growth, hepatologic, or visceral symptoms was removed. Dosing for Gaucher Disease Type 3 was revised such that each individual dose must not exceed 60 U/kg administered intravenously no more frequently than once every 2 weeks.	4/16/2026	6/15/2026

The policy effective date is in force until updated or retired.

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