VENTURA COUNTY
HEALTH CARE PLAN

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Gaucher Disease – Enzyme Replacement Therapy – Elelyso Utilization Management Medical Policy

- Elelyso® (taliglucerase intravenous infusion – Pfizer)

REVIEW DATE: 04/02/2025

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]

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. A 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. 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).[11] Guidelines note that there is no evidence that ERT has reversed, stabilized, or slowed the progression of neurological involvement. 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.

POLICY STATEMENT

Prior Authorization is recommended for medical 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.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Elelyso is recommended in those who meet one of the following criteria:

FDA-Approved Indication

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1. **Gaucher Disease – Type 1.** Approve for 1 year if the patient meets ALL of the following (A, B, and C):
   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 β-glucocerebrosidase activity in leukocytes or fibroblasts; OR
      ii. Molecular genetic testing documenting biallelic pathogenic variants in the glucocerebrosidase (*GBA*) gene; AND
   C) 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.

   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

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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 β-glucocerebrosidase activity in leukocytes or fibroblasts; OR
      ii. Molecular genetic testing documenting biallelic pathogenic variants in the glucocerebrosidase (*GBA*) gene; AND
   C) The patient meets BOTH of the following (i and ii):
      i. 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.
      ii. Medication is being used for the management of impaired growth, hematologic, or visceral symptoms; AND
      Note: Examples of visceral symptoms include splenomegaly and hepatomegaly. Examples of hematologic symptoms include anemia and thrombocytopenia.
   D) 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.

   Dosing. Each individual dose must not exceed 120 U/kg administered intravenously no more frequently than once every 2 weeks.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Elelyso is not recommended in the following situations:

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).

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Elelyso® intravenous infusion [prescribing information]. New York, NY: Pfizer; January 2025.
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, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. *J Inherit Metab Dis*. 2009 Oct;32(5):660-664.

HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	04/05/2023
Annual Revision	No criteria changes.	04/10/2024
Selected Revision	**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. For diagnosis established by genetic testing, genetic testing demonstrating a mutation in the glucocerebrosidase (*GBA*) gene was further specified to state a genetic test documenting biallelic pathogenic variants in the *GBA* gene. **Gaucher Disease – Type 3**: This new condition of approval was added under other uses with supportive evidence. Concomitant use with other approved therapies for Gaucher disease was added under conditions not recommended for approval.	07/17/2024
Annual Revision	No criteria changes.	04/02/2025