

**POLICY:** Gaucher Disease – Enzyme Replacement Therapy – Elelyso® (taliglucerase for injection – Pfizer)

**DATE REVIEWED:** 03/25/2020

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### OVERVIEW

Elelyso is an analogue of  $\beta$ -glucocerebrosidase produced via recombinant DNA technology in genetically modified carrot plant root cells.<sup>1</sup> Elelyso differs from human glucocerebrosidase by two amino acids at the N terminal and seven amino acids at the C terminal end of the protein. Elelyso catalyzes the breakdown of glucocerebroside to glucose and ceramide.

Elelyso is indicated for the treatment of patients with a confirmed diagnosis of Type 1 Gaucher disease.<sup>1</sup>

### Disease Overview

Gaucher disease is a rare autosomal recessive, inherited, lysosomal storage disorder caused by a deficiency of the lysosomal enzyme  $\beta$ -glucocerebrosidase.<sup>2-4</sup> 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).<sup>2-5</sup> Type 1 is a non-neuropathic variant with asymptomatic or symptomatic clinical manifestations of splenomegaly, hepatomegaly, anemia, thrombocytopenia, skeletal complications, and occasional lung involvement. Type 2 is an acute neuropathic 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 characterized by neurological symptoms and visceral symptoms with a later onset and includes abnormal eye movements, ataxia, seizures, and dementia. Type 1 is most prevalent in the Western world, accounting for an estimated 94% of patients with Gaucher disease.<sup>2,6</sup> Types 2 and 3 represent < 1% and 5%, respectively, in Europe, North America, and Israel.<sup>2,5</sup> The diagnosis of Gaucher disease is established by demonstrating deficient  $\beta$ -glucocerebrosidase activity in leukocytes or fibroblasts, or mutations in the glucocerebrosidase gene.<sup>7,8</sup>

### 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 indication(s). 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.

### RECOMMENDED AUTHORIZATION CRITERIA

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

### FDA-Approved Indications

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1. **Gaucher Disease, Type 1.** Approve for 1 year if the patient meets the following criteria (A and B):
  - A) The diagnosis is established by one of the following (i or ii):
    - i. Demonstration of deficient  $\beta$ -glucocerebrosidase activity in leukocytes or fibroblasts; OR
    - ii. Molecular genetic testing documenting glucocerebrosidase gene mutation; AND
  - B) 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.<sup>1</sup>

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### CONDITIONS NOT RECOMMENDED FOR APPROVAL

Elelyso has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

1. 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® for injection [prescribing information]. New York, NY: Pfizer; December 2016.
2. Burrow TA, Barnes S, and Grabowski GA. Prevalence and management of Gaucher disease. *Pediatric Health, Medicine and Therapeutics*. 2011;2:59-73
3. Cox T. Gaucher disease: clinical profile and therapeutic development. *Biologics: Targets & Therapy*. 2010;4:299-313
4. Jmoudiak, M. and Futerman, AH. Gaucher disease: Pathological mechanisms and modern management. *British Journal of Haematology*. 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.
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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.

### HISTORY

Type of Revision	Summary of Changes	Date Reviewed
New Policy	--	03/20/2019
Annual revision	No criteria change	03/25/2020