

<b>Clinical Policy Title:</b>	taliglucerase alfa
<b>Policy Number:</b>	RxA.113
<b>Drug(s) Applied:</b>	Elelyso®
<b>Original Policy Date:</b>	02/07/2020
<b>Last Review Date:</b>	03/09/2021
<b>Line of Business Policy Applies to:</b>	All lines of business

## Background

Taliglucerase alfa (Elelyso®) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for the treatment of patients 4 years and older with a confirmed diagnosis of Type 1 Gaucher disease

## Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
taliglucerase alfa (Elelyso®)	Type 1 Gaucher disease (GD1).	<p><u>Treatment-naïve patients:</u></p> <p>60 units/kg every other week, administered as a 60-120 minute IV infusion</p> <p><u>Patients switching from imiglucerase:</u></p> <p>Begin at the same unit/kg dose as the patient's previous imiglucerase dose; administer every other week</p>	Individualized (adjust based on achievement and maintenance of therapeutic goals)

## Dosage Forms

- Single-use vial: 200 units

## Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

### I. Initial Approval Criteria

#### A. Gaucher Disease (must meet all):

1. Diagnosis of Type 1 (GD1) confirmed by one of the following (a or b):
  - a. Enzyme assay demonstrating a deficiency of beta-glucocerebroside (glucosidase) activity;
  - b. DNA testing;
2. Age 4 years of age or older;
3. Member is symptomatic (e.g., anemia, thrombocytopenia, bone disease, hepatomegaly, splenomegaly);

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

4. Elelyso® is not prescribed concurrently with Vpriv® (velaglucerase alfa) or Cerezyme® (imiglucerase).

**Approval Duration**

**Commercial:** 6 months

**Medicaid:** 6 months

**II. Continued Therapy Approval**

**A. Gaucher Disease (must meet all):**

1. Currently receiving medication that has been authorized by RxAdvance or member has previously met initial approval criteria listed in this policy.
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, decreased bone pain;
3. Elelyso® is not prescribed concurrently with Vpriv® (velaglucerase alfa) or Cerezyme® (imiglucerase).

**Approval Duration**

**Commercial:** 6 months

**Medicaid:** 6 months

**III. Appendices**

**APPENDIX A: Abbreviation/Acronym Key**

FDA: Food and Drug Administration

GD1: Type 1 Gaucher disease

**APPENDIX B: Therapeutic Alternatives**

Not applicable

**APPENDIX C: Contraindications/Boxed Warnings**

- Contraindication(s):
  - none reported
- Boxed Warning(s):
  - none reported

**APPENDIX D: General Information**

- Measures of Therapeutic Response: GD1 is a heterogeneous disorder which involves the visceral organs, bone marrow, and bone in almost all affected patients. Common conditions resulting from GD1 include anemia, thrombocytopenia, hepatomegaly, splenomegaly, and bone disease. Therefore, hemoglobin level, platelet count, liver volume, spleen volume, and bone pain are clinical parameters that can indicate therapeutic response to GD1 therapies. In some clinical trials, stability has been defined as the following thresholds of change from baseline: hemoglobin level < 1.5 g/dL decrease, platelet count < 25% decrease, liver volume < 20% increase, and spleen volume < 25% increase.
- Enzyme replacement therapy such as Cerezyme may have beneficial palliative effects in Type 2 disease but does not alter the outcome and is not generally used.
- According to the European consensus guidelines revised recommendations on the management of neuronopathic Gaucher disease by Vellodi et al: (1) there is clear evidence in most patients that enzyme replacement therapy (ERT) ameliorates systemic involvement in non-neuronopathic (Type 1) as well as

chronic neuronopathic Gaucher disease (Type 3), enhancing quality of life; (2) There is no evidence that ERT has reversed, stabilized or slowed the progression of neurological involvement; (3) In patients with established acute neuronopathic Gaucher disease (Type 2), enzyme replacement therapy has had little effect on the progressively downhill course. It has merely resulted in prolongation of pain and suffering.

- There is currently insufficient clinical evidence that supports the combination use of enzyme replacement therapy with Zavesca® (miglustat).

**References**

1. Ellelyso® Prescribing Information. New York, NY: Pfizer, Inc.; November 2020. Available at <http://www.ellelyso.com>. Accessed January 16, 2021.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with type 1 Gaucher disease: consensus recommendations. J Pediatr. 2004; 144: 112-20.
3. Hollak, CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. Best Pract Res Clin Endocrinol Metab. 2015; 29: 205-218.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. Semin Hematol. 2004; 41(suppl 5): 4-14.
5. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. Genet Med. 2005; 7(2): 105-110.
6. Altarescu G, Hill S, Wiggs E, et al. The efficacy of enzyme replacement therapy in patients with chronic neuronopathic Gaucher`s disease. J Pediatr. 2001; 138: 539-547.
7. Vellodi A, Tylki-Szymanska A, Davies E, et al. Management of neuronopathic Gaucher disease: Revised recommendations. J Inherit Metab Dis. 2009; 32: 660-664.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	01/2020	02/07/2020
Updated references	05/01/2020	05/20/2020
Policy was reviewed:  1. Policy title table was updated: Line of business policy applies was updated to All lines of business.  2. Continued therapy criteria II.A.1 was rephrased to “Currently receiving medication that has been authorized by Rxadvance.  3. Initial Criteria I.A.1 was updated.	01/14/2021	03/09/2021