

## Clinical Policy: Taliglucerase Alfa (Elelyso)

Reference Number: CP.PHAR.157

Effective Date: 02.01.16

Last Review Date: 05.23

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

### Description

Taliglucerase alfa (Elelyso<sup>®</sup>) is a hydrolytic lysosomal glucocerebroside-specific enzyme.

### FDA Approved Indication(s)

Elelyso is indicated for the treatment of patients 4 years and older with a confirmed diagnosis of type 1 Gaucher disease.

### Policy/Criteria

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

It is the policy of health plans affiliated with Centene Corporation<sup>®</sup> that Elelyso is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

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

1. Diagnosis of type 1 (GD1) or type 3 Gaucher disease (GD3) confirmed by one of the following (a or b):
  - a. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity;
  - b. DNA testing;
2. Age  $\geq$  4 years;
3. Member is symptomatic (e.g., anemia, thrombocytopenia, bone disease, hepatomegaly, splenomegaly);
4. One of the following (a or b):
  - a. For GD1: Failure of Cerezyme<sup>®</sup> and Cerdelga<sup>®</sup> unless clinically significant adverse effects are experienced or both are contraindicated;
  - b. For GD3: Failure of Cerezyme, unless contraindicated or clinically significant adverse effects are experienced;

*\*Prior authorization may be required for Cerezyme and Cerdelga*
5. Elelyso is not prescribed concurrently with VPRIV<sup>®</sup> (velaglucerase alfa) or Cerezyme<sup>®</sup> (imiglucerase);
6. Documentation of member's current weight (in kg);
7. Dose does not exceed 60 units/kg every two weeks.

##### Approval duration:

**HIM/Medicaid** – 6 months

**Commercial** – 6 months or to the member's renewal date, whichever is longer

**B. Other diagnoses/indications (must meet 1 or 2):**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
  - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
  - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

**II. Continued Therapy**

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

1. Member meets one of the following (a or b):
  - a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
  - b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B*);
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, or decreased bone pain;
3. Eleyso is not prescribed concurrently with VPRIV (velaglucerase alfa) or Cerezyme (imiglucerase);
4. Documentation of member's current weight (in kg);
5. If request is for a dose increase, new dose does not exceed 60 units/kg every 2 weeks.

**Approval duration:**

**HIM/Medicaid** – 12 months

**Commercial** – 6 months or to the member's renewal date, whichever is longer

**B. Other diagnoses/indications (must meet 1 or 2):**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
  - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business:

**CLINICAL POLICY**  
**Taliglucerase Alfa**

- CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
- b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

**III. Diagnoses/Indications for which coverage is NOT authorized:**

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

**IV. Appendices/General Information**

*Appendix A: Abbreviation/Acronym Key*

ERT: enzyme replacement therapy

FDA: Food and Drug Administration

GD1: type 1 Gaucher disease

GD3: type 3 Gaucher disease

*Appendix B: Therapeutic Alternatives*

*This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.*

<b>Drug Name</b>	<b>Dosing Regimen</b>	<b>Dose Limit/Maximum Dose</b>
Cerdelga (eliglustat)	<b>GD1:</b> CYP2D6 EM, IM: 84 mg PO BID CYP2D6 PM: 84 mg PO QD	CYP2D6 EM, IM: 168 mg/day CYP2D6 PM: 84 mg/day
Cerezyme (imiglucerase)	<b>GD1 or GD3:</b> Recommended dosage based upon disease severity ranges from 2.5 U/kg via IV infusion 3 times a week to 60 U/kg once every 2 weeks; titrate the dosage based on clinical manifestations of disease and therapeutic goals for the patient	60 U/kg every 2 weeks

*Therapeutic alternatives are listed as Brand name<sup>®</sup> (generic) when the drug is available by brand name only and generic (Brand name<sup>®</sup>) when the drug is available by both brand and generic*

*Appendix C: Contraindications/Boxed Warnings*

None reported

**CLINICAL POLICY**  
**Taliglucerase Alfa**

*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<sup>®</sup> (miglustat) or Cerdelga<sup>®</sup> (eliglustat), or concurrent use of two or more enzyme replacement therapies at once.

**V. Dosage and Administration**

Indication	Dosing Regimen	Maximum Dose
Gaucher disease	<p><u>Treatment-naïve patients:</u> 60 units/kg IV every other week</p> <p><u>Patients switching from imiglucerase:</u> 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)

**VI. Product Availability**

Single-use vial: 200 units

**VII. References**

1. Eleyso Prescribing Information. New York, NY: Pfizer, Inc.; August 2022. Available at <http://www.eleyso.com>. Accessed February 7, 2023.
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.

**CLINICAL POLICY**  
**Taliglucerase Alfa**

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.
8. Gary SE, Ryan E, Steward AM, et al. Recent advances in the diagnosis and management of Gaucher disease. *Expert Rev Endocrinol Metab.* 2018 Mar;13(2):107-118.
9. Leonart LP, Fachi MM, Boger B, et al. A systematic review and meta-analyses of longitudinal studies on drug treatments for Gaucher disease. *Ann Pharmacother.* 2022;0(0). doi:10.1177/10600280221108443.

**Coding Implications**

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3060	Injection, taliglucerase alfa, 10 units

Reviews, Revisions, and Approvals	Date	P&T Approval Date
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.27.19	05.19
2Q 2020 annual review: no significant changes; revised HIM-Medical Benefit to HIM line of business; references reviewed and updated.	02.05.20	05.20
RT4: updated indication with age extension to include patients 4 years and older as per FDA label.	11.24.20	
2Q 2021 annual review: no significant changes; references reviewed and updated.	02.27.21	05.21
For CY2022, per March SDC removed HIM line of business to separate policy.	08.11.21	11.21
2Q 2022 annual review: no significant changes; added requirement for documentation of member's current weight for dose calculation purposes; added max dosing recommendations per Prescribing Information; references reviewed and updated.	02.24.22	05.22

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Template changes applied to other diagnoses/indications and continued therapy section.	09.30.22	
2Q 2023 annual review: no significant changes; references reviewed and updated. Per February SDC and prior clinical guidance, added redirections to Cerdelga and Cerezyme; added Appendix B; added HIM line of business and retired HIM.PA.162.	02.21.23	05.23

**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

**CLINICAL POLICY**  
Taliglucerase Alfa

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**Note:**

**For Medicaid members**, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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