POLICY Document for ELELYSO (taliglucerase alfa)

The overall objective of this policy is to support the appropriate and cost-effective use of the medication, specific to use of preferred medication options, and overall, clinically appropriate use. This document provides specific information to both sections of the overall policy.

Section 1: Preferred Product
- Policy information specific to preferred medications

Section 2: Site of Care
- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 3: Clinical Criteria
- Policy information specific to the clinical appropriateness for the medication

Section 1: Preferred Product

CAREFIRST: EXCEPTIONS CRITERIA
GAUCHER DISEASE AGENTS

PREFERRED PRODUCTS: CEREZYME, VPRIV

Client Requested: The intent of the criteria is to ensure that patients follow selection elements as established by CareFirst.

POLICY
This policy informs prescribers of preferred products and provides an exception process for targeted products through prior authorization.

I. PLAN DESIGN SUMMARY

This program applies to the Gaucher disease products specified in this policy. Coverage for targeted products is provided based on clinical circumstances that would exclude the use of the preferred product and may be based on previous use of a product. The coverage review process will ascertain situations where a clinical exception can be made. This program applies to all members requesting treatment with a targeted product.

Each referral is reviewed based on all utilization management (UM) programs implemented for the client.

Table. Gaucher Disease Products

	Product(s)
Preferred*	• **Cerezyme** (imiglucerase) • **Vpriv** (velaglucerase alfa)
Targeted	• **Elelyso** (taliglucerase alfa) • **Cerdelga** (eliglustat)

*: Medications considered formulary or preferred on your plan may still require a clinical prior authorization review

II. EXCEPTION CRITERIA

This program applies to members requesting treatment for an indication that is FDA-approved for the preferred product.

Coverage for a targeted product is provided when member has a documented inadequate response, contraindication, or intolerable adverse event to both preferred products.

Section 2: Site of Care

CareFirst Site of Care Criteria
Elelyso

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated.

Brand Name	Generic Name	Dosage Form
Elelyso	taliglucerase alfa	intravenous

Criteria For Approval For Administration In Outpatient Hospital Setting

This policy provides coverage for administration of Elelyso in an outpatient hospital setting for up to 22 days when a member is new to therapy or is reinitiating therapy after not being on therapy for at least 6 months.

This policy provides coverage for administration of Elelyso in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

- The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids, other pre-medications or slowing of infusion rate) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion.
- The member has developed anti-drug antibodies which increases the risk for infusion related reactions.
- The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
- The member has severe venous access issues that require the use of special interventions only available in the outpatient hospital setting.

- The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
- Alternative infusion sites (pharmacy, physician office, ambulatory care, etc.) are greater than 30 miles from the member's home.
- The member is less than 14 years of age.

For situations where administration of Elelyso does not meet the criteria for outpatient hospital infusion, coverage for Elelyso is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

Required Documentation

The following information is necessary to initiate the site of care prior authorization review (where applicable):

- Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
- Medical records supporting the member has developed anti-drug antibodies
- Medical records supporting the member is medically unstable
- Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
- Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
- Records supporting alternative infusion sites are greater than 30 miles from the member's home
- Medical records supporting the member is new to therapy

Section 3: Clinical Criteria

Specialty Guideline Management
Elelyso

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Elelyso	taliglucerase alfa

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1]

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

Compendial Uses

- Gaucher disease type 2[6]
- Gaucher disease type 3[3-5]

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review: beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Gaucher disease type 1[1,2]

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

Gaucher disease type 2[6]

Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

Gaucher disease type 3[3-5]

Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment of an indication listed in coverage criteria section when all of the following criteria are met:

- Member meets the criteria for initial approval.
- Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

REFERENCES:

SECTION 1

1. Cerdelga [package insert] Waterford, Ireland: Genzyme Ireland, Ltd; August 2018.
2. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2022.
3. Elelyso [package insert]. New York, NY: Pfizer, Inc; July 2024.
4. VPRIV [package insert]. Lexington, MA: Takeda Pharmaceuticals U.S.A., Inc.; July 2024.

SECTION 2

1. Elelyso [package insert]. NY, NY: Pfizer Inc.; July 2024.
2. Zimran A, Brill-Almon E, Chertkoff R, et al. Pivotal trial with plant cell-expressed recombinant glucocerebrosidase, taliglucerase alfa, a novel enzyme replacement therapy for Gaucher disease. *Blood.* 2011;118(22):5767-5773.
3. Pastores GM, Shankar SP, Petakov M, et al. Enzyme replacement therapy with taliglucerase alfa: 36-month safety and efficacy results in adult patients with Gaucher disease previously treated with imiglucerase. *Am J Hematol.* 2016;91(7):661-665.
4. Zimran A, Gonzalez-Rodriguez DE, Abrahamov A, et al. Safety and efficacy of two dose levels of taliglucerase alfa in pediatric patients with Gaucher disease. *Blood Cells Mol Dis.* 2015;54(1):9-16.

SECTION 3

1. Elelyso [package insert]. New York, NY: Pfizer, Inc; July 2024.

2. Zimran A, Brill-Almon E, Chertkoff R, et al. Pivotal trial with plant cell-expressed recombinant glucocerebrosidase, taliglucerase alfa, a novel enzyme replacement therapy for Gaucher disease. *Blood*. 2011;118:5767-5773.
3. Pastores GM, Hughes DA. Gaucher Disease. 2000 July 27 [Updated December 7, 2023]. In: Adam MP, Everman DB, Mirzaa GM, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2023.
4. Kaplan P, Baris H, De Meirleir L, et al. Revised recommendations for the management of Gaucher disease in children. *Eur J Pediatr*. 2013;172:447-458.
5. Vellodi A, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. European Working Group on Gaucher Disease. *J Inherit Metab Dis*. 2009;32(5):660.
6. Gaucher Disease. National Organization for Rare Disorders. (2024). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.