

## POLICY Document for Elelyso

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, lower cost site of care and overall clinically appropriate use. This document provides specific information to each of the three 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

### EXCEPTIONS CRITERIA GAUCHER DISEASE AGENTS

#### PREFERRED PRODUCT: CEREZYME

#### POLICY

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

#### I. PLAN DESIGN SUMMARY

This program applies to the Gaucher disease products specified in this policy. Coverage for non-preferred 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 members who are new to treatment with a non-preferred product for the first time.

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

**Table. Gaucher Disease Agents**

	Product(s)
<b>Preferred</b>	<ul style="list-style-type: none"> <li>• <b>Cerezyme</b> (imiglucerase)</li> </ul>
<b>Non-preferred</b>	<ul style="list-style-type: none"> <li>• <b>Elelyso</b> (taliglucerase alfa)</li> <li>• <b>VPRIV</b> (velaglucerase alfa)</li> </ul>

#### II. EXCEPTION CRITERIA

Coverage for a non-preferred product is provided when any of the following criteria are met:

- Member is currently receiving treatment with the non-preferred product, excluding when the non-preferred product is obtained as samples or via manufacturer's patient assistance programs.
- Member has had a documented inadequate response or an intolerable adverse event with the preferred product (Cerezyme)

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## **Section 2: Site of Care**

### **Site of Care Criteria Administration of Intravenous Elelyso**

#### **POLICY**

#### **I. 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 2 doses when a member is new to therapy.

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:

- A. The member has experienced an adverse reaction that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids or other pre-medications) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion.
- B. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
- C. The member has severe venous access issues that require the use of a special intervention.
- D. 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.
- E. Alternative infusion sites are not available.
- F. The member is less than 21 years of age or 65 years of age or older.

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.

#### **II. REQUIRED DOCUMENTATION**

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

- A. 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
- B. Medical records supporting the member is medically unstable
- C. Medical records supporting the member has severe venous access issues
- D. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
- E. Records supporting alternative infusion sites are not available

## **Section 3: Clinical Criteria**

### **SPECIALTY GUIDELINE MANAGEMENT**

#### **ELELYSO (taliglucerase alfa)**

#### **POLICY**

##### **I. 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

ElELYSO is indicated for the treatment of patients with a confirmed diagnosis of type 1 Gaucher disease.

##### **II. CRITERIA FOR INITIAL APPROVAL**

##### **Gaucher disease type 1**

Indefinite authorization 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.

##### **III. CONTINUATION OF THERAPY**

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

## REFERENCES:

### SECTION 1

1. Elvelysio [package insert]. New York, NY: Pfizer, Inc; December 2016.
2. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; May 2011.
3. VPRIV [package insert]. Lexington, MA: Shire Human Genetic Therapies, Inc.; April 2015.

### SECTION 2

1. Elvelysio [package insert]. NY, NY: Pfizer Inc.; January 2017.
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. Elvelysio [package insert]. New York, NY: Pfizer, Inc; December 2016.
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. [Updated February 26, 2015]. In: Pagon RA, Adam MP, Ardinger HH, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2016.
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.