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POLICY Document for Cerezyme

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)
Preferred	• **Cerezyme** (imiglucerase)
Non-preferred	• **Elelyso** (taliglucerase alfa) • **VPRIV** (velaglucerase alfa)

II. EXCEPTION CRITERIA

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

A. 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.

B. 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 Cerezyme

POLICY

I. CRITERIA FOR APPROVAL FOR ADMINISTRATION IN OUTPATIENT HOSPITAL SETTING

This policy provides coverage for administration of Cerezyme in an outpatient hospital setting for up to 2 doses when a member is new to therapy.

This policy provides coverage for administration of Cerezyme 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 has developed laboratory confirmed imiglucerase IgG antibodies which increases the risk for infusion related reactions.
C. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
D. The member has severe venous access issues that require the use of a special intervention.
E. 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.
F. Alternative infusion sites are not available.
G. The member is less than 21 years of age or 65 years of age or older.

For situations where administration of Cerezyme does not meet the criteria for outpatient hospital infusion, coverage for Cerezyme 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 has imiglucerase IgG antibodies
C. Medical records supporting the member is medically unstable
D. Medical records supporting the member has severe venous access issues
E. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
F. Records supporting alternative infusion sites are not available.

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Section 3: Clinical Criteria

SPECIALTY GUIDELINE MANAGEMENT

CEREZYME (imiglucerase)

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.

A. FDA-Approved Indications
Cerezyme is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly.

B. Compendial Uses
Gaucher disease type 3

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR INITIAL APPROVAL

A. 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

B. Gaucher disease type 3
Indefinite authorization 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

III. CONTINUATION OF THERAPY

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

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REFERENCES:

SECTION 1

1. Elelyso [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. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation.; April 2018.
2. Serratrice C, Carballo S, Serratrice J, Stirnemann J. Imiglucerase in the management of Gaucher disease type 1: an evidence-based review of its place in therapy. *Core Evid.* 2016;11:37-47.
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4. Kishnani PS, DiRocco M, Kaplan P, et al. A randomized trial comparing the efficacy and safety of imiglucerase (Cerezyme) infusions every 4 weeks versus every 2 weeks in the maintenance therapy of adult patients with Gaucher disease type 1. *Mol Genet Metab.* 2009;96(4):164-170.

SECTION 3

1. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; May 2011.
2. 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.
3. Erikson A, Forsberg H, Nilsson M, Astrom M, Mansson JE. Ten years' experience of enzyme infusion therapy of Norrbottnian (type 3) Gaucher disease. *Acta Paediatr*. 2006;95:312-317.
4. 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.
5. 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.