Premier Health Plan

POLICY AND PROCEDURE

POLICY TITLE:	**Glucocerebrosidase Replacement Enzymes (Ceremyze, Elelyso, VPRIV)**
DEPARTMENT:	**Clinical Pharmacy Services- Utilization Management**
ORIGINAL DATE:	***July 2008 (as adopted from UPMC Health Plan)***

Last P & T Committee Approval Date: February 2018

Product Applicability: *mark all applicable products below:*

COMMERCIAL	[ ] HMO [ ] PPO *Products*: [ ] Small [ ] Indiv. [ ] Large *Exchange*: [ ] Shop [ ] Indiv. [x] All
OTHER	[x] Self-funded/ASO

PURPOSE

The purpose of this policy is to define the prior authorization process for Glucocerebrosidase Replacement Enzymes.

Imiglucerase (Cerezyme) is indicated for use as long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type I Gaucher's disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, and hepatomegaly or splenomegaly.

Taliglucerase (Elelyso) is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease.

Velaglucerase (VPRIV) is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with type 1 Gaucher disease.

DEFINITIONS

Gaucher Disease: a rare, autosomal recessive disorder caused by mutations in the GBA gene that leads to a deficiency of the lysosomal enzyme beta-glucocerebrosidase. Glucocerebrosidase is responsible for converting sphingolipid glucocerebroside into

glucose and ceramide. The enzymatic deficiency leads to a build-up of glucocerebroside, causing an accumulation of "Gaucher cells" in the liver, spleen, bone marrow, and other organs. Extra Gaucher cells in the liver and spleen cause organomegaly whereas excess in the bone marrow and spleen causes anemia and thrombocytopenia.

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, as defined in CRM.015-Medical Necessity, and approval by the Pharmacy & Therapeutics Committee of the criteria for prior authorization, as described in RX.003-Prior Authorization Process.

The drugs, imiglucerase (Cerezyme), velaglucerase (VPRIV), and taliglucerase alfa (Elelyso), are subject to the prior authorization process.

PROCEDURE

Initial Authorization Criteria:

I. PLAN DESIGN SUMMARY

Requests for VPRIV and Elelyso are subject to the preferred medical drug list program. 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 all members requesting treatment with a non-preferred product for an indication that is also FDA-approved for the preferred product.
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	• VPRIV **(velaglucerase alfa)** • Elelyso **(taliglucerase alfa)**

Requests for a non-preferred drug must meet one of the following exception criteria in addition to clinical criteria:

II. EXCEPTION CRITERIA (*Use for VPRIV/Elelyso Requests Only*)

Coverage for a non-preferred product is provided when the member has experienced a confirmed adverse event with the preferred product (Cerezyme).

III. CLINICAL CRITERIA (*Use for ALL Drug Requests*)

Must meet all of the clinical criteria listed under the respective diagnosis:

For All Diagnoses:

- Must be prescribed by a physician that specializes in the treatment of inherited metabolic disorders or a center that specializes in the treatment of Gaucher disease, or in consultation with these specialties.
- Must have a diagnosis of Gaucher disease with any of the following:
    - Anemia, defined as:
        - For members >12 years of age
            - Hemoglobin <12 g/dL in males
            - Hemoglobin <11.0 g/dL in females
        - For children between 2 and 12 years of age
            - Hemoglobin <10.5 g/dL
        - For children between 6 months and 2 years of age
            - Hemoglobin <9.5 g/dL
        - For children under 6 months of age
            - Hemoglobin <10.1 g/dL
    - Thrombocytopenia
        - Defined as a platelet count <100,000
    - Bone disease
        - Defined as having one of the following:
        - Avascular necrosis
        - Ernlenmeyer flask deformity
        - Lytic disease
        - Marrow infiltrations
        - Osteopenia
        - Osteosclerosis
        - Pathological fracture

- Radiological evidence of joint deterioration
- Hepatomegaly
  - Defined as liver size 1.25 or more times normal. (Normal liver size is 2.5% of total body weight)
- Splenomegaly
  - Defined as a splenic mass greater than normal. (Normal spleen size is 0.2% of total body weight).

Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the medical necessity for continuation of therapy. Authorization may be extended at 1 year intervals based upon chart documentation from the prescriber that the member's condition has improved based upon the prescriber's assessment while on therapy.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 1 year
Reauthorization	Same as initial

If the established criteria are not met, the request is referred to a Medical Director for review.

REFERENCES

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2. Hollak CE, de Fost M, van Dussen L, Vom Dahl S, Aerts JM. Enzyme therapy for the treatment of type 1 Gaucher disease: clinical outcomes and dose - response relationships. Expert Opin Pharmacother. 2009 Nov;10(16):2641-52
3. Biegstraaten M; van Schaik IN; Aerts JM; Hollak CE. 'Non-neuronopathic' Gaucher disease reconsidered. Prevalence of neurological manifestations in a Dutch cohort of type I Gaucher disease patients and a systematic review of the literature. J Inherit Metab Dis. 2008 Jun;31(3):337-49.
4. Cerezyme [package insert]. Cambridge, MA: Genzyme Corpration; April 2005
5. VPRIV[package insert]. Cambridge, MA: Shire Human Genetic Therapies, Inc; Feb 2010
6. Elelyso [package insert]. New York, NY: Pfizer, Inc; May 2012
7. 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

8. Pastores GM, Weinreb NJ, Aerts NJ et al. Therapeutic goals in the treatment of Gaucher Disease. Semin Hematol 2004;41 (suppl 5):4-14
9. Charrow J. Enzyme replacement for Gaucher Disease. Expert Opin Biol Ther 2009;9(1):121-131
10. Grabowski GA. Lysosomal storage disease 1: phenotype, diagnosis, and treatment of Gaucher's disease. Lancet 2008;372:1263-71

RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review	*02/16, 02/17, 02/18*
Criteria update	*05/16*
Preferred Product Update (effective 4/1/18)	*02/18*