RX.PA.011.MPC Glucocerebrosidase Replacement Enzymes (Ceremyze® and VPRIV®)

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

Cerezyme® (imiglucerase) 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.

VPRIV® (velaglucerase) 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.

PROCEDURE

A. Initial Authorization Criteria:

Must meet all of the 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)

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Glucocerebrosidase replacement enzymes will be considered investigational or experimental for any other use and will not be covered.

D. <u>Reauthorization Criteria:</u>
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.

<u>Limitations:</u>

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, if required for the plan and level of request.

Codes: J Code(s)

Code	Description
J1786	Injection, imiglucerase, 10 units
J3385	Injection, velaglucerase alfa, 100 units

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
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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.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual review	*02/2022*
Addition of dosing requirements and off-label restriction	*12/2021*
P&T Review	*11/2020*