CVS/caremark™

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

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the prior authorization review: enzyme assay or genetic testing results supporting diagnosis.

III. EXCLUSIONS

Coverage will not be provided for concomitant use with substrate reduction therapy (eg, miglustat, eliglustat)

IV. CRITERIA FOR INITIAL APPROVAL

A. Gaucher disease type 1

Authorization for up to 12 months may be granted for treatment of Gaucher disease type 1 when all of the following criteria are met:

1. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
2. Member exhibits one or more complications of type 1 Gaucher disease (see Appendix)

B. Gaucher disease type 3

Authorization for up to 12 months may be granted for treatment of Gaucher disease type 3 when all of the following criteria are met:

1. Diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing
2. Member exhibits one or more complications of type 3 Gaucher disease (see Appendix)

V. CONTINUATION OF THERAPY

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

VI. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

VII. APPENDIX: Complications of Gaucher disease

Gaucher disease type 1

- Anemia
- Thrombocytopenia
- Bone disease (eg, bone pain, bone crises, fractures, osteonecrosis, subchondral joint collapse)
- Hepatomegaly or splenomegaly

Gaucher disease type 3

- Anemia
- Thrombocytopenia
- Bone disease (eg, bone pain, bone crises, fractures, osteonecrosis, subchondral joint collapse)
- Hepatomegaly or splenomegaly
- Developmental delay
- Ophthalmoplegia (gaze palsy)

VIII. REFERENCES

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.