CVS/caremark™

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. 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 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)

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

VIII. REFERENCES

1. Elelyso [package insert]. New York, NY: Pfizer, Inc; November 2015.
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.