Clinical Policy: Taliglucerase Alfa (Elelyso)

Reference Number: PA.CP.PHAR.157
Effective Date: 01/18
Last Review Date: 04/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for taliglucerase alfa (Elelyso®).

FDA Approved Indication(s)
Elelyso is indicated for the treatment of patients with a confirmed diagnosis of type 1 Gaucher disease (GD1).

Policy/Criteria
It is the policy of health plans affiliated with Pennsylvania Health and Wellness that Elelyso is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Type 1 Gaucher Disease (must meet all):

1. Diagnosis of Type 1 Gaucher disease (GD1) confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase activity;
   b. DNA testing;
2. Age ≥ 4 years;
3. Member is symptomatic (e.g., anemia, thrombocytopenia, bone disease, hepatomegaly, splenomegaly);
4. Not prescribed concurrently with velaglucerase alfa or imiglucerase.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Type 1 Gaucher Disease (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, decreased bone pain;
3. Elelyso is not prescribed concurrently with velaglucerase alfa or imiglucerase.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or

2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background
Description/Mechanism of Action:
Gaucher disease is an autosomal recessive disorder caused by mutations in the human glucocerebrosidase gene, which results in a reduced activity of the lysosomal enzyme glucocerebrosidase. Glucocerebrosidase catalyzes the conversion of the sphingolipid glucocerebroside into glucose and ceramide. The enzymatic deficiency results in accumulation of substrate glucocerebroside primarily in the lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells," which accumulate in the liver, spleen and bone marrow. Elelyso, a long term enzyme replacement therapy, is a recombinant analog of human lysosomal glucocerebrosidase that catalyzes the hydrolysis of glucocerebroside to glucose and ceramide, reducing the amount of accumulated glucocerebroside. Elelyso uptake into cellular lysosomes is mediated by binding of Elelyso mannose oligosaccharide chains to specific mannose receptors on the cell surface leading to internalization and subsequent transport to the lysosomes.

Formulations:
Elelyso (taliglucerase alfa): Lyophilized product for reconstitution; for intravenous use
- 200 units/5 mL vial; 40 units/mL

Appendices
Appendix A: Abbreviation Key
GD1: Type 1 Gaucher disease

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3060	Injection, taliglucerase alfa, 10 units

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: added age limit; removed maximum dose limit, as dosing is individualized per patient response to therapy; added the requirement that Elelyso not be used concurrently with other enzyme replacement therapies; added specific examples of positive response to therapy, for reauthorization. Added coverage for Type 3 Gaucher disease; references reviewed and updated.	2.26.18	

References

1. Elelyso prescribing information. New York, NY: Pfizer, Inc.; June 2016. Available at http://www.elelyso.com. Accessed February 26, 2018.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with Type 1 Gaucher disease: Consensus recommendations. J Pediatr. 2004; 144: 112-20.
3. Hollak, CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: Therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. Best Pract Res Clin Endocrinol Metab. 2015; 29: 205-218.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. Semin Hematol. 2004; 41(suppl 5): 4-14.
5. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. Genet Med. 2005; 7(2): 105-110.
6. 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.
7. Vellodi A, Tylki-Szymanska A, Davies E, et al. Management of neuronopathic Gaucher disease: Revised recommendations. J Inherit Metab Dis. 2009;32:660-664.