

## Elelyso® (taliglucerase alfa)

When requesting Elelyso® (taliglucerase alfa), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indications.

### FDA-approved indication

- Elelyso is indicated for the treatment of individuals with Type 1 Gaucher disease.

### Approved Off-label Compendial use

- None

### Coverage Guidelines

#### Type 1 Gaucher Disease

The individual must meet **all** of the following criteria for approval:

- The diagnosis is established by one of the following:
  - Demonstration of deficient  $\beta$ - glucocerebrosidase activity in leukocytes or fibroblasts; OR
  - Molecular genetic testing documenting glucocerebrosidase gene mutation; AND
- This is being prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

**Approval duration: 1 year**

### Dosing Guidelines

- 60 U/kg administered intravenously no more frequently than once every 2 weeks

### References

1. Elelyso® for injection [prescribing information]. New York, NY: Pfizer; December 2016.
2. Burrow TA, Barnes S, and Grabowski GA. Prevalence and management of Gaucher disease. *Pediatric Health, Medicine and Therapeutics*. 2011;2:59-73
3. Cox T. Gaucher disease: clinical profile and therapeutic development. *Biologics: Targets & Therapy*. 2010;4:299-313
4. Jmoudiak, M. and Futerman, AH. Gaucher disease: Pathological mechanisms and modern management. *British Journal of Haematology*. 2005;129(2):178–188
5. Grabowski GA. Lysosomal storage disease 1- phenotype, diagnosis, and treatment of Gaucher's disease. *Lancet*. 2008;372:1263-1271.
6. Zimran A. How I treat Gaucher disease. *Blood*. 2011;118:1463-1471.
7. Stirnemann J, Belmatoug N, Camou F, et al. A review of Gaucher disease pathophysiology, clinical presentation and treatments. *Int J Mol Sci*. 2017;18:441.
8. Baris HN, Cohen IJ, Mistry PK. Gaucher disease: The metabolic defect, pathophysiology, phenotypes and natural history. *Pediatr Endocrinol Rev*. 2014;12:72-81.