Department:	Pharmacy	**Original Approval:**	11/14/2018
Policy #:	PM152	**Last Approval:**	11/14/2018
Title:	Enzymes for Gaucher Disease (Taliglucerase alfa, Imiglucerase, Velaglucerase alfa)		
Approved By:	UM Pharmacy Subcommittee		

BACKGROUND

Gaucher Disease is an inherited autosomal recessive disease characterized by deficient glucocerebrosidase and consequent accumulation of glucocerebroside in the reticuloendothelial cells of the liver, spleen, bone marrow, and other tissues. Type-1 Gaucher disease is the most common subtype, accounting for more than 90% of all cases, and is characterized by systemic manifestations without primary central nervous system involvement (nonneuronopathic). Type-2 Gaucher disease is characterized by severe early neurologic manifestations (acute neuronopathic) with death usually occurring before 2 years of age. Type-3 Gaucher disease is characterized by subacute neurologic symptoms (chronic neuronopathic) and systemic manifestations.

Cerezyme (imiglucerase for injection) is indicated for long-term enzyme replacement therapy 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, or hepatomegaly or splenomegaly. Elelyso (taliglucerase alfa) for injection is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for the treatment of pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease. VPRIV (velaglucerase alfa) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy for pediatric and adult patients with type 1 Gaucher disease. Gaucher disease is a rare and debilitating genetic disorder in which patients lack the enzyme bglucocerebrosidase, which is essential for the proper lipid metabolism. As a result of this missing enzyme, there is a build-up of the glycolipid glucocerebroside, which can cause a host of problems, most importantly, hepatomegaly (enlarged liver), splenomegaly (enlarged spleen), bone disease and severe anemia (low blood counts). The mainstay of treatment for this disease focuses on replacing the missing enzyme, which provides some relief, but is not a cure.

DEFINITIONS

None

INDICATIONS/CRITERIA

Medicaid Members	***Continue to criteria for approval below.***

Medicare Members	***Step-utilization of Part D drugs not required.***

For Type 1 Gaucher Disease Adult:

1. Documented diagnosis of type 1 Gaucher's disease
2. ANY of the following symptoms (a, b, c, d, or e):
    a. Moderate to severe anemia (hemoglobin ≤ 11.5 g/dL [adult women] or ≤ 12.5 g/dL [adult men] or ≤ 1.0 g/dL or more below the lower limit of normal for age and sex)
    b. Significant hepatomegaly (liver size 1.25 or more times normal [1,750 cc in adults]) or splenomegaly (spleen size 5 or more times normal [875 cc in adults])
    c. Skeletal disease beyond mild osteopenia and Erlenmeyer flask deformity
    d. Symptomatic disease, including abdominal or bone pain, fatigue, exertional limitation, weakness, or cachexia
    e. Thrombocytopenia (platelet count less than or equal to 120,000/mm3).
3. Age Limits:
    a. imiglucerase (Cerezyme®): Greater than or equal to (≥) 12 years of age
    b. taliglucerase (Elelyso®): Greater than or equal to (≥) 4 years of age
    c. velaglucerase alfa (VPRIV®): Greater than or equal to (≥) 4 years of age

Approve for 12 months

Criteria for Reauthorization:

Documentation of positive clinical response.

Approve for 12 months

For Type 3 Gaucher's disease:

1. Documented diagnosis of type 3 Gaucher's disease
2. Neurologic findings consistent with type 3 Gaucher's disease, including encephalopathy, opthalmoplegia, progressive myoclonic epilepsy, cerebellar ataxia, spasticity, or dementia
3. ANY of the following symptoms (a, b, c, d, or e):
    a. Moderate to severe anemia (hemoglobin ≤ 11.5 g/dL [adult women] or ≤ 12.5 g/dL [adult men] or ≤ 1.0 g/dL or more below the lower limit of normal for age and sex)
    b. Significant hepatomegaly (liver size 1.25 or more times normal [1,750 cc in adults]) or splenomegaly (spleen size 5 or more times normal [875 cc in adults])
    c. Skeletal disease beyond mild osteopenia and Erlenmeyer flask deformity
    d. Symptomatic disease, including abdominal or bone pain, fatigue, exertional limitation, weakness, or cachexia
    e. Thrombocytopenia (platelet count less than or equal to 120,000/mm3).

Approve for 12 months

Criteria for Reauthorization:

Documentation of positive clinical response.

Approve for 12 months

Dosing Recommendations
Doses up to, but not exceeding, 60 units/kg infused every 2 weeks.

SPECIAL CONSIDERATIONS

None

LIMITATIONS/EXCLUSIONS

Please refer to a product line's certificate of coverage for benefit limitations and exclusions for these services:

PRODUCT LINE	LINK TO CERTIFICATE OF COVERAGE
MEDICARE ADVANTAGE	http://healthfirst.chpw.org/for-members/resource-library/handbooks-and-guides
WASHINGTON APPLE HEALTH	http://chpw.org/our-plans/apple-health/
INTEGRATED MANAGED CARE	http://chpw.org/our-plans/apple-health/

Citations & References

CFR		
WAC	WAC 284-43-2050	
RCW		
Contract Citation	☐ WAH	
	☐ IMC	
	☐ MA	
Other Requirements	WA HCA Agents for Gaucher's Disease Medical policy no. 82.70.00-1 Last Updated 02/21/2018	
NCQA Elements		

References	1. AHFS Drug Information® with AHFSfirstReleases®. (www.statref.com) American Society Of Health-System Pharmacists®, Bethesda, MD. Updated periodically. 2. DRUGDEX® System [Internet database]. Greenwood Village, CO: Thomson Micromedex. Updated periodically. 3. Drug Facts and Comparisons online. (www.drugfacts.com), Wolters Kluwer Health, St. Louis, MO. Updated periodically. 4. PDR® Electronic Library™ [Internet database]. Greenwood Village, CO: Thomson Micromedex. Updated periodically. 5. Cerezyme [prescribing information]. Cambridge, MA: Genzyme Corp.; March 2003. 6. Elelyso [prescribing information]. NY, NY: Pfizer, Inc.; June 2016. 7. VPRIV [prescribing information]. Lexington, MA: Shire Human Genetic Therapies, Inc.; April 2015. 8. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. International Collaborative Gaucher Group U.S. Regional Coordinators. *Genet Med*. 2005;7(2):105. 9. Weinreb NJ, Aggio MC, Andersson HC, et al. Gaucher disease type 1: revised recommendations on evaluations and monitoring for adult patients. International Collaborative Gaucher Group (ICGG). *Semin Hematol*. 2004;41(4 Suppl 5):15. 10. Vellodi A, Tylki-Szymanska A, Davies EH, et al. Management of neuronopathic Gaucher disease: revised recommendations. European Working Group on Gaucher Disease. *J Inherit Metab Dis*. 2009;32(5):660. 11. Washington health Care Authority Agents for Gaucher's Disease Medical Policy 82.70.00-1

Revision History

Revision Date	Revision Description	Revision Made By
07/30/2018	New policy	Jennifer Farley, Pharm.D.
11/14/2018	Approval	UM Pharmacy Subcommittee