Elelyso™ (taliglucerase alfa)
(Intravenous)

Document Number: IC-0105

Last Review Date: 09/01/2021
Date of Origin: 03/07/2013
Dates Reviewed: 12/2013, 02/2014, 09/2014, 07/2015, 07/2016, 08/2016, 08/2017, 07/2018, 07/2019, 07/2020, 09/2021

I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC unit]:

- Elelyso 200 unit powder for injection: 35 vials per 14 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- 700 billable units every 14 days

III. Initial Approval Criteria [1,9-13]

Coverage is provided in the following conditions:

- Patient is at least 4 years of age; **AND**

Universal Criteria

- Must be used as a single agent; **AND**

Type 1 Gaucher's Disease † Φ

- Patient has a documented diagnosis of Type 1 Gaucher Disease as confirmed by a beta-glucosidase leukocyte (BGL) test with significantly reduced or absent glucocerebrosidase enzyme activity; **AND**
- *Adults only (i.e., patients at least 18 years or older)*: Patient's disease results in one or more of the following:
  - Anemia [*i.e., hemoglobin less than or equal to 11 g/dL (women) or 12 g/dL (men)*] not attributed to iron, folic acid, or vitamin B12 deficiency; **OR**
  - Moderate to severe hepatomegaly (liver size 1.25 or more times normal) or splenomegaly (spleen size 5 or more times normal); **OR**

- Skeletal disease (e.g., lesions, remodeling defects and/or deformity of long bones, osteopenia/osteoporosis, etc.); **OR**
- Symptomatic disease (e.g., bone pain, fatigue dyspnea, angina, abdominal distension, diminished quality of life, etc.); **OR**
- Thrombocytopenia (platelet count less than or equal to 120,000/mm^3)

† FDA Approved Indication(s), Φ Orphan Drug

IV. Renewal Criteria [1,9-13]

Coverage can be renewed based on the following criteria:

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Disease response with treatment as defined by one or more of the following (compared to pre-treatment baseline):
  - Improvement in symptoms (e.g., bone pain, fatigue, dyspnea, angina, abdominal distension, diminished quality of life, etc.)
  - Reduction in size of liver or spleen
  - Improvement in hemoglobin/anemia
  - Improvement in skeletal disease (e.g., increase in lumbar spine and/or femoral neck BMD, no bone crises or bone fractures, etc.)
  - Improvement in platelet counts; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include the following: severe hypersensitivity reactions, etc.

V. Dosage/Administration[1]

Indication	Dose
Type 1 Gaucher Disease	Administer up to 60 units/kg every other week as an intravenous infusion

VI. Billing Code/Availability Information

HCPCS code:

- J3060 – Injection, taliglucerase alfa, 10 units; 1 billable unit = 10 units

NDC:

- Elelyso 200 unit powder for injection, single-use vial: 00069-0106-xx

VII. References

1. Elelyso [package insert]. New York, NY; Pfizer Inc; July 2021. Accessed August 2021.
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5. Baldellou A, Andria G, Campbell PE, et al. Paediatric non-neuronopathic Gaucher disease: recommendations for treatment and monitoring. Eur J Pediatr 2004; 163:67.
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11. Pastores G, Petakov M, Giraldo P, et al. A Phase 3, Multicenter, Open-Label, Switchover Trial to Assess the Safety and Efficacy of Taliglucerase Alfa, a Plant Cell-Expressed Recombinant Human Glucocerebrosidase, in Adult and Pediatric Patients With Gaucher Disease Previously Treated With Imiglucerase Blood Cells Mol Dis 2014 Dec;53(4):253-60. doi: 10.1016/j.bcmd.2014.05.004. Epub 2014 Jun 18.
12. Biegstraaten M, Cox TM, Belmatoug N, et al. Management goals for type 1 Gaucher disease: An expert consensus document from the European working group on Gaucher disease. Blood Cells, Molecules and Diseases 68 (2018) 203-208.
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Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E75.22	Gaucher disease

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Local Coverage Article may exist and compliance with these policies is required where applicable. They can be found at: http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC