Policy Title:	Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), VPRIV (velaglucerase alfa) Intravenous		
		Department:	PHA
Effective Date:	01/01/2020		
Review Date:	04/19/2019, 9/18/2019, 12/18/2019, 1/29/2020, 2/04/2021, 1/27/2022, 1/19/2023, 2/16/2023, 12/07/2023, 01/10/2024		

Purpose: To support safe, effective and appropriate use of Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), and VPRIV (velaglucerase alfa) to treat Gaucher's disease.

Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

Policy Statement:

Medications to treat Gaucher's disease are covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure:

Coverage of Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), and VPRIV (velaglucerase alfa) will be reviewed prospectively via the prior authorization process based on criteria below.

Summary of Evidence:

Clinical trials evaluating the efficacy and safety of enzyme replacement therapies (ERTs) for Gaucher's disease have demonstrated significant benefits in reducing spleen and liver volumes, improving hematological parameters, reducing bone pain, and improving overall quality of life. ERTs provide exogenous replacement of the deficient lysosomal enzyme, glucocerebrosidase, thereby addressing the underlying metabolic defect in Gaucher's disease.

Coverage Criteria:

- Patient must have a confirmed diagnosis of type 1 Gaucher disease (GD1) when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing; AND
- For Medicaid members requesting Elelyso (taliglucerase alfa) or VPRIV (velaglucerase alfa) they must have a documented failure, intolerance or contraindication to Cerezyme (imiglucerase); OR
- For Commercial requesting Cerezyme (imiglucerase) or VPRIV (velaglucerase alfa) they must have a documented failure, intolerance or contraindication to Elelyso (taliglucerase alfa);OR
- For MMP members requesting VPRIV (velaglucerase alfa) they must have a documented failure, intolerance or contraindication to Elelyso (taliglucerase alfa) and Cerezyme (imiglucerase); OR

- Patients that are currently on treatment with Elelyso (taliglucerase alfa) or VPRIV (velaglucerase alfa) can remain on treatment; OR
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

Continuation of Therapy Criteria:

- The patient meets all initial criteria; AND
- Patient is tolerating and responding to medication (improvement in symptoms compared to pre-treatment baseline, such as e.g. bone pain, fatigue, dyspnea, angina, abdominal distension, diminished quality of life, etc.) and there continues to be a medical need for the medication.

Coverage duration: 6 months

Per §§ 42 CFR 422.101, this clinical medical policy only applies to INTEGRITY in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD)

Policy Rationale:
Cerezyme, Elelyso, and VPRIV Intravenous were reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Cerezyme, Elelyso, and VPRIV Intravenous according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.

Dosage/Administration:

Cerezyme:

Indication	**Dose**	**Maximum dose (1 billable unit = 10 units)**
Type 1 Gaucher Disease	Initial dosages range from 2.5 U/kg of body weight 3 times a week to 60 U/kg once every 2 weeks based on disease severity.	700 billable units every 14 days

Elelyso:

Indication	**Dose**	**Maximum dose (1 billable unit = 10 units)**

Type 1 Gaucher Disease	Up to 60 units/kg every other week as a 60-120-minute intravenous infusion	700 billable units every 14 days

VPRIV:

Indication	Dose	Maximum dose (1 billable unit = 10 units)
Type 1 Gaucher Disease	Up to 60 units/kg every other week as a 60-minute intravenous infusion	72 billable units every 14 days

Investigational use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Applicable Codes:
Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all-inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT codes are:

HCPCS/CPT Code	Description
J1786	Injection, imiglucerase, 10 units
J3060	Injection, taliglucerase alfa, 10 units
J3385	Injection, velaglucerase alfa, 100 units

References:

1. Elelyso [package insert]. New York, NY: Pfizer, Inc.; May 2023. Accessed November 2023.
2. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; April 2018. Accessed November 2023.
3. VPRIV [package insert]. Lexington, MA: Shire Human Genetic Therapies, Inc.; October 2022. Accessed November 2023