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, 12/04/2024, 5/14/2025, 12/09/2025		

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

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:

Cerezyme: Cerezyme is a hydrolytic lysosomal glucocerebrosidase-specific enzyme indicated for the treatment of Type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly or splenomegaly. Study RC 91-0110 was a randomized, double-blind, active-controlled trial involving 30 patients (17 males and 13 females) aged 12 to 69 years, with an average age of 38 years in the Cerezyme group and 28 years in the alglucerase group. Participants, all diagnosed with Gaucher disease type 1 and with hemoglobin levels at least 1 g/dL below the lower limit for their age and sex, were randomized 1:1 to receive either Cerezyme (60 units/kg every other week) or alglucerase for 6 months. The primary efficacy endpoints included a minimum 1 g/dL increase in hemoglobin concentration, increased platelet count, and reduced spleen and liver volumes at the 6-month mark. Efficacy results indicated positive outcomes, including improvements in bone x-rays showing enhanced cortical thickness and reduced lucencies in 7 of 11 patients treated with Cerezyme. Common adverse events include back pain, chills, hypersensitivity reactions and pyrexia.

VPRIV: VPRIV is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy in patients with Type 1 Gaucher disease. The efficacy of Vpriv was evaluated in three clinical studies involving 99 patients with type 1 Gaucher disease, comprising 82 patients aged 4 years and older who received VPRIV and 17 patients aged 3 years and older who received imiglucerase. Studies I and II focused on patients not currently receiving Gaucher-specific therapy, while

Study III included patients transitioning from imiglucerase. Study I was a 12-month randomized, double-blind trial of 25 patients aged 4 and older with Gaucher disease-related anemia, thrombocytopenia, or organomegaly. Participants, primarily without prior therapy for at least 30 months, received either VPRIV at 45 Units/kg (N=13) or 60 Units/kg (N=12) every other week. At baseline, the mean hemoglobin was 10.6 g/dL, platelet count was 97 x 10^9/L, and liver and spleen volumes were measured via MRI. After 12 months, the observed changes in hemoglobin concentration were clinically meaningful. Study II was a 9-month randomized, double-blind, active-controlled trial involving 34 patients aged 3 years and older, with a mean age of 30 years. Patients received either 60 Units/kg of VPRIV (N=17) or imiglucerase (N=17) every other week. Baseline measures showed a mean hemoglobin concentration of 11.0 g/dL and a mean platelet count of 171 x 10^9/L. After 9 months, the mean increase in hemoglobin for the VPRIV group was 1.6 g/dL ± 0.2 (SE), with a treatment difference of 0.1 g/dL ± 0.4 (SE) compared to imiglucerase. There were no significant differences in response based on age or gender. Study III was a 12-month open-label study with 40 patients aged 9 years and older, all previously receiving imiglucerase for at least 30 consecutive months. Patients transitioned to VPRIV at equivalent doses to their previous imiglucerase therapy. Over 12 months, hemoglobin levels remained stable, with a median concentration of 13.5 g/dL compared to a baseline of 13.8 g/dL, and median platelet counts increased from 162 x 10^9/L to 174 x 10^9/L. No dosage adjustments were needed during this period. Overall, these studies support VPRIV's efficacy in managing Gaucher disease, maintaining hemoglobin levels and improving clinical parameters. Common adverse events include back and joint pain, pyrexia and abdominal pain.

Elelyso: Elelyso is hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy in patients with Type 1 Gaucher disease. In a 9-month trial assessing Elelyso for Type 1 Gaucher disease, 31 adult patients were evaluated for safety and efficacy. The study included patients with significantly enlarged spleens (over eight times normal) and thrombocytopenia (below 120,000/mm³), with 16 also having enlarged livers and 10 exhibiting anemia. All participants were treatment-naïve, and those with severe neurological symptoms were excluded. Patients were randomly assigned to receive either 30 Units/kg or 60 Units/kg of Elelyso. Baseline measurements revealed mean spleen volumes of 3.1% and 3.3% of body weight for the respective dose groups, with hemoglobin concentrations at 12.2 g/dL and 11.4 g/dL. After 9 months, spleen volume decreased significantly, while hemoglobin levels increased. A follow-up extension allowed 26 patients to continue treatment for a total of 24 months, showing consistent improvements in spleen size, hemoglobin, liver volume, and platelet counts. Additionally, in a separate 9-month study, 25 patients who switched from imiglucerase to Elelyso were assessed. All patients had stable imiglucerase therapy for at least 6 months before switching. Baseline metrics indicated spleen volumes at 1.1% BW and mean hemoglobin levels at 13.6 g/dL. After 9 months on Elelyso, spleen volumes and hemoglobin levels remained stable, confirming the treatment's effectiveness in maintaining clinical parameters. Common side effects include pyelonephritis, arthralgia, influenza, and pharyngitis.

Coverage Criteria:

- Member 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 ONLY requesting VPRIV (velaglucerase alfa) or Elelyso (taliglucerase alfa) they must have a documented failure, intolerance or contraindication to Cerezyme (imiglucerase); OR
- For Commercial members ONLY requesting VPRIV (velaglucerase alfa) they must have a documented failure, intolerance or contraindication to both Cerezyme (imiglucerase) and Elelyso (taliglucerase alfa); OR
- Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

Continuation of Therapy Criteria:

- The member meets all initial criteria; AND
- Member 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 Medicare 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) 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 Medicare 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.; January 2025. Accessed May 2025.
2. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2024. Accessed May 2025.
3. VPRIV [package insert]. Lexington, MA: Shire Human Genetic Therapies, Inc.; November 2024. Accessed May 2025.