Gaucher Disease Agents (Cerezyme®, Elelyso®, and VPRIV®)

Policy Number: MC/PC 015
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Table of Contents **Page**

Related Policies

- n/a

Coverage Rationale

<Please refer to Medical Benefit Plan Sponsor for preferred medications. Preferred products may be updated and therefore subject to change>.

Gaucher Disease

For coverage of Cerezyme (imiglucerase for injection) for the treatment of Type 1 Gaucher disease the following will be required:

- Diagnosis of Type 1 Gaucher disease **and**
- Patient has evidence of symptomatic disease (e.g.: moderate to severe anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly)

For coverage of Cerezyme (imiglucerase for injection) for the treatment of Type 3 Gaucher disease the following will be required:

- Diagnosis of Type 3 Gaucher disease **and**
- Presence of a non-central nervous system (CNS) manifestation (e.g., moderate to severe anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly)

For coverage of Elelyso (taliglucerase alfa for injection) for the treatment of Type 1 Gaucher disease the following will be required:

- Diagnosis of Type 1 Gaucher disease **and**
- Patient has evidence of symptomatic disease (e.g.: moderate to severe anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly) **and**

- Patient is 4 years of age or older

For coverage of VPRIV (velaglucerase alfa for injection) for the treatment of Type 1 Gaucher disease the following will be required:

- Diagnosis of Type 1 Gaucher disease **and**
- Patient has evidence of symptomatic disease (e.g.: moderate to severe anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly) **and**
- Patient is 4 years of age or older

Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

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

ICD-10 Code	Description
E75.22	Gaucher disease

Background

Gaucher disease is an inherited recessive lysosomal storage disorder caused by a deficiency of the lysosomal enzyme, glucocerebrosidase (also known as glucosylceramidase or acid beta-glucosidase), caused by mutations in the *GBA1* gene (Biegstraaten et al 2018, Hughes & Sidransky 2024, National Organization for Rare Disorders [NORD] 2020). The prevalence of Gaucher disease in the United States (U.S.) is approximately 6000 individuals and has been estimated to affect 1 in 40,000 to 60,000 individuals globally. Gaucher disease is most prevalent in the Ashkenazi Jewish population with an estimated incidence of 1 in every 450 live births (Biegstraaten et al 2018, Kaplan et al 2013, NORD 2020).

There are 3 principal types of Gaucher disease (Type 1 [GD1], Type 2 [GD2] and type Type [GD3]), characterized by different clinical manifestations (hematological, skeletal, visceral, neurological, and other organ systems), age of onset, progression, and life expectancies. The 3 types are thought to represent a continuum from severely affected babies to older adults with mild or no clinical manifestations (Biegstraaten et al 2018, Hughes & Sidransky 2024).

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 (non-neuronopathic). Type 2 Gaucher disease (rapid/acute neuronopathic) is characterized by severe early neurologic manifestations (acute neuronopathic) with death usually occurring before 2 years of age. Type 3 Gaucher disease (chronic/subacute neuronopathic) is characterized by subacute neurologic symptoms (chronic neuronopathic) and systemic manifestations.

Cerezyme (imiglucerase)

Type 1 Gaucher disease

The comparable efficacy of Ceredase (alglucerase; discontinued) 60 Units(U)/kg every 2 weeks (high dose, n = 15) vs. imiglucerase (n = 15) was evaluated in a 9 month, parallel-group (PG), double-blind (DB), randomized controlled trial (RCT) in patients 12 to 69 years of age with mild GD1 and intact, enlarged spleen (> 0.2% of body weight) and hemoglobin ≥ 1 g/dL less than the lower limit of normal (Grabowski et al 1995).

- Note: Alglucerase is a macrophage-targeted placentally-derived human glucocerebrosidase, which became available in 1991 and has since been withdrawn from the U.S. market. Imiglucerase is its recombinant successor (available since 1994) (Drugs@FDA 2026, Kaplan et al 2013, Purple Book: Database of Licensed Biological Products 2026).
- Primary efficacy endpoints were improvement in hemoglobin concentrations and platelet count, plus hepatic and splenic volume changes.
- There were no significant differences between the 2 treatments in key clinical parameters ($p > 0.2$; analysis of variance).
- Treatment with alglucerase and imiglucerase significantly increased hemoglobin levels by 1.6 g/dL and 1.82 g/dL at month 6, and 2.28 g/dL and 2.54 g/dL at month 9, respectively, from a baseline hemoglobin level of 10.7 g/dL.
- Approximately half of the patients in each group had increases in platelet counts of 20% and 40% or more during the 6-and 9-month treatment periods, respectively, which did not differ between the 2 drugs.
- Visceral organ changes did not differ between the treatment groups. Overall reductions in hepatic volume were approximately 12.4% and 19% at 6 and 9 months, respectively, and overall reductions in splenic volume were 34.7% and 44.6% at months 6 and 9, respectively ($p > 0.2$ for both measures; analysis of variance).
- Anti-glucocerebrosidase antibodies developed in 3 patients receiving imiglucerase and in 6 patients receiving alglucerase over the course of the study. Patients in the imiglucerase group developed antibodies by 3 to 6 months.
- Bone x-rays showed improvements in cortical thickness and lucencies in 7 of 11 imiglucerase-treated patients (Cerezyme prescribing information 2025).
- While this comparative study demonstrated therapeutic similarity of imiglucerase and alglucerase, it was not powered to show equivalence and was not designed to demonstrate the effectiveness of ERT. Nonetheless, based on laboratory results, both imiglucerase and alglucerase may be considered equivalent and to exert a class effect (Connock et al 2006).
- In an extension study (RC 92-0501), 29 patients continued treatment for a total duration of 24 months. Patients were unblinded at 9 months and allowed to cross-over to imiglucerase treatment. At 24 months, mean increase in hemoglobin was 2.4 g/dL, mean increase in platelet count was $40^3 \times 10^3$/mL, and mean change in liver and spleen volume was -20% and -57%, respectively (Cerezyme prescribing information 2025).

The effect of long-term alglucerase/imiglucerase treatment on hematological, visceral, and bone manifestations of GD1 were analyzed in a large cohort of non-splenectomized (n = 557) and splenectomized (n = 200) patients with GD1 enrolled in the International Collaborative Gaucher Group (ICGG) Gaucher Registry with 10 years of follow-up (Weinreb et al 2013).

- At baseline, splenectomized vs. non-splenectomized patients had lower percentages of anemia and thrombocytopenia (26.0% vs 42.8% and 14.2% vs 76.3%, respectively), similar percentages of moderate or severe hepatomegaly (81.2% vs 80.0%, respectively), and higher percentages of bone pain and bone crises (88.9% vs 52.4% and 38.3% vs 16.0%, respectively).
- Ten years of imiglucerase treatment resulted in sustainable and significant ($p < 0.05$) improvements in all GD1 parameters in both splenectomized and non-splenectomized patients, including improvements in mean hemoglobin levels, platelet count, liver, and spleen (non-splenectomized) volumes, and bone crises.

An analysis of an international cohort of data from the ICGG Gaucher Registry e [illegible] with alglucerase (discontinued) or imiglucerase in 884 children over an 8-year period (Andersson et al 2008).

- Anemia was present in > 50% of patients at baseline and resolved for all patients after 8 years of treatment. At baseline, > 50% of patients had platelet counts of <100 000/mm3, and > 95% had platelet counts above this level after 8 years of treatment. Liver and spleen volumes also decreased over 8 years of treatment.
- At baseline, the median height z-score for the study population was -1.4; after 8 years of treatment, the median height approximated the median value for the normal population.
- The mean bone mineral density (BMD) z-score was -0.34 at baseline, and values normalized within 6.6 years of treatment.
- For bone crises, 17% of patients reported a bone crisis before treatment and in the first 2 years of treatment; no bone crises were reported after 2 years of ERT. A total of 2.5% of patients did not experience bone crises before ERT and had a crisis after treatment initiation.
- The authors concluded that within 8 years of ERT, most clinical parameters studied became normal or nearly normal.

Type 3 Gaucher disease

The efficacy of imiglucerase for the treatment of non-CNS manifestations of Type 1 and Type 3 Gaucher disease was assessed in Study 3, an observational study, using data from the International Collaborative Gaucher Group (ICGG) Gaucher Disease Registry (NCT00358943). (Cerezyme prescribing information 2025).

- Study 3 included patients with Type 1 or Type 3 Gaucher disease who were treated with imiglucerase as initial therapy with an index clinical assessment and one or more follow-up clinical assessments. Study 3 was a baseline-controlled analysis in patients with Type 1 Gaucher disease (19 weeks to 87 years of age) and patients with Type 3 Gaucher disease (7 weeks to 54 years of age) who received imiglucerase intravenously as prescribed by their physicians (initiated treatment between 1992–2021).
- After approximately two years (1 to 3 years) of imiglucerase treatment in patients with Type 1 and 3 Gaucher disease, mean changes from baseline in the following measures showed improvement: hemoglobin, platelet count, liver volume, spleen volume, and height Z-score.
    - Among 1,052 Type 1 Gaucher disease patients, mean baseline hemoglobin was 11.8 g/dL and mean increase from baseline was 1.5 g/dL (95% CI: 1.4, 1.5).
    - Among 1,053 Type 1 Gaucher disease patients, mean baseline platelet count was 128×10^3 /mm^3 and mean increase from baseline was 64×10^3/mm^3 (95% CI: 59.6, 67.9).
    - Among 118 Type 3 Gaucher disease patients, mean baseline hemoglobin levels were 10 g/dL and mean increase from baseline was 1.8 g/dL (95% CI: 1.5, 2.1).
    - Among 116 Type 3 Gaucher disease patients, mean baseline platelet count was 149×10^3/mm^3 and mean increase from baseline was 105×10^3/mm^3 (95% CI: 87.4, 122.4).
- The 2-year summaries include measurements within 1 to 3 years after treatment initiation due to the lack of predefined data collection timepoints in the registry.

Elelyso (taliglucerase alfa)

Type 1 Gaucher disease

The efficacy and safety of taliglucerase alfa were assessed in a 9-month Phase 3, DB, multi-center (MC), PG, RCT in 31 treatment-naïve adults with GD1 and Gaucher disease-related enlarged spleens (> 8 times normal) and thrombocytopenia. Patients were randomized to treatment with taliglucerase alfa 30 U/kg body weight per infusion (n = 15) or 60 U/kg body weight per infusion (n = 16) every 2 weeks for a total of 20 intravenous (IV) infusions (no placebo arm was included due to ethical reasons, including the shortage of another Gaucher disease ERT) (Zimran et al 2011).

- The primary efficacy endpoint of reduction in spleen volume at month 9 was achieved in both groups. The taliglucerase alfa 30 U/kg arm achieved a reduction of 26.9% (95% confidence interval [CI], -31.9 to -21.8; p <

0.0001), and the 60 U/kg arm achieved a reduction of 38.0% (95% CI, -
statistically significant difference between treatment groups (95% CI, 1

- Hemoglobin levels and liver volume demonstrated statistically significant improvement in both groups at 6 and 9 months.
    - Mean hemoglobin levels increased by 1.6 g/dL in the 30 U/kg group (95% CI, 0.3 to 3.5; p = 0.001) and 2.2 g/dL in the 60 U/kg group (95% CI, 0.6 to 3.8; p < 0.0001) at month 9, with no statistically significant differences observed between dose groups observed at 6 or 9 months.
    - For liver volume, statistically significant reductions were noted in both groups: 10.5% (95% CI, -17.0 to -4.0) in the 30 U/kg group (p = 0.004) and 11.1% (95% CI, -15.0 to -7.4) in the 60 U/kg group (p < 0.0001) at month 9. No statistically significant differences were observed between the dose groups at 6 or 9 months.
    - For platelet counts, improvements were seen in both dose groups at 6 and 9 months but only results from the 60 U/kg group achieved the pre-specified α-level of statistical significance at month 9 with a mean increase of 41,494/mm^3 (95% CI, 17,658 to 65,330; p = 0.003). There was a statistically significant difference in platelet response between groups at month 9 (p = 0.042).
- A total of 23 patients completed 36 months of treatment with taliglucerase alfa (Zimran et al 2016). Both taliglucerase alfa doses resulted in mean decreases in spleen and liver volumes with mean increases in hemoglobin concentrations and platelet counts.

A 12-month, Phase 3B, DB, MC RCT evaluated the safety and efficacy of taliglucerase alfa 30 U/kg (n = 6) or 60 U/kg (n = 5) administered every 2 weeks evaluated in 9 treatment-naïve pediatric patients 2 to < 18 years of age with GD1 (Zimran et al 2015). The dose was calculated by body weight and then rounded up to next full vial, with an average dose of 34.7 U/kg for the 30 U/kg group, and 63.7 U/kg for the 60 U/kg group.

- Median hemoglobin levels increased by 12.2% and 14.2%, with absolute changes of 1.4 g/dL and 1.6 g/dL, respectively.
- After 12 months, spleen and liver volumes were reduced from baseline in both dose groups.
- Platelet counts improved by 30.9% and 73.7% in the 30 U/kg and 60 U/kg dose groups.
- Most adverse events (AEs) were mild to moderate, transient, and not related to treatment. One patient experienced gastroenteritis requiring hospitalization for rehydration on the first infusion visit; however, the patient continued on treatment with intermittent antihistamine use.
- A MC extension study included 15 pediatric patients with GD1, with 10 treatment naïve patients who continued from the above study by Zimran et al 2015, and 5 patients who switched from imiglucerase (Zimran et al 2018). Patients received taliglucerase alfa 30 U/kg or 60 U/kg or the same dose previously treated with imiglucerase every other week.
    - In treatment-naïve patients, patients treated with taliglucerase alfa 30 and 60 U/kg, respectively, had reduced mean spleen volume (-18.6 multiples of normal [MN] and -26.0 MN), liver volume (-0.8 MN and 0.9 MN), and chitotriosidase activity (-72.7% and -84.4%), and increased mean hemoglobin (+2.0 g/dL and +2.3 g/dL) and mean platelet count (+38,200/mm^3 and +138,250/mm^3) from baseline through 36 total months of treatment.
    - In patients previously treated with imiglucerase, the spleen and liver volumes and hematological parameters remained stable through 33 months of taliglucerase alfa therapy.
    - AEs included cough, headache, upper respiratory tract infection, abdominal pain, Dengue fever, diarrhea, lymphedema, nasopharyngitis, and pain in extremity.

The efficacy and safety of switching from imiglucerase to taliglucerase alfa were evaluated in 31 patients (26 adult and 5 pediatric patients) with GD1 who were stable on imiglucerase and switched to taliglucerase alfa in a 9-month, Phase 3, MC, open-label (OL) study. Imiglucerase was discontinued, and taliglucerase alfa was administered every other week at the same number of units as each patient's previous imiglucerase dose, with dosage adjustment allowed during the study in order to maintain clinical stability (Pastores et al 2014).

- The main efficacy outcome was clinical stability during treatment over [illegible]rs of platelet counts, hemoglobin concentration, spleen and liver volumes.
- Mean baseline values for the overall population were within normal range for all clinical parameters except for mean spleen volume, which was 822 mL or 1.1% of body weight or 5.5 MN volume.
- Most patients remained stable with regards to the main disease parameters, with the following exceptions: 4 of 28 patients experienced clinically significant deterioration in 1 of the 4 clinical parameters, with 1 patient who experienced spleen enlargement > 20%, 1 patient who experienced liver enlargement > 10%, and 2 patients who experienced decreases of > 20% in platelet count. One of the patients stabilized the platelet count after the taliglucerase alfa dose was increased from 9.5 U/kg to 19 U/kg.
- All 25 patients who had completed 9 months of treatment had stable hemoglobin and platelet counts within 15% of baseline value at the end of the study.
- The impact of immunogenicity on efficacy was not clear. There did not appear to be evidence of increased safety concerns in patients switched from imiglucerase to taliglucerase alfa.
- In an extension study of patients who had switched from imiglucerase, 18 patients received ≥ 1 dose of taliglucerase alfa and 10 patients completed 36 total months of therapy (Pastores et al 2016).
    - In patients who completed 36 months of taliglucerase alfa treatment, hemoglobin concentration and platelet counts were unchanged from baseline through 36 months of taliglucerase alfa therapy. Spleen and liver volumes remained unchanged or decreased through 36 months of therapy. A total of 4 patients had anti-taliglucerase alfa antibodies detected at ≥ 1 study visit.

A MC, OL, expanded-access study evaluated the safety and efficacy of taliglucerase alfa in 58 adults with GD1 for up to 33 months (Kuter et al 2020).

- A total of 51 previously-treated patients were treated with taliglucerase alfa for 9 months at a dose equivalent to their previous imiglucerase dose; these patients were offered treatment for up to 33 months. An amendment allowed for the addition of 7 treatment-naïve patients in the study; 36 patients completed the study.
- In previously treated patients, increases from baseline to last follow-up were observed for mean ± standard error of the mean (SE) hemoglobin levels (13.0 ± 0.3 g/dL to 13.4 ± 0.2 g/dL) and platelet counts (179,242 ± 15,344/mm^3 to 215,242 ± 17,867/mm^3); results were similar in treatment-naïve patients.
- Most AEs were mild or moderate; treatment-related AEs were mild and transient.

VPRIV (velaglucerase alfa)

Type 1 Gaucher disease

The safety and efficacy of velaglucerase alfa administered at 60 U/kg every other week were established in 3 pivotal clinical trials (Studies I, II, and III) and an open-label extension trial (Study IV), with a total of 99 patients with GD1.

Studies I and II were Phase 3, multinational, DB, PG, RCTs in treatment-naïve patients (ie, patients who had not previously received previous ERT [Study I] or had not received ERT within the previous 30 months [Study II]) (FDA Summary review [VPRIV] 2010).

Study I included 25 anemic patients ≥ 4 years of age (range, 4 to 62 years of age) who were randomized to receive velaglucerase alfa 45 U/kg (n = 13) or 60 U/kg (n = 12) every other week for 12 months (Gonzalez et al 2013).

- In the 60 U/kg treatment group, mean hemoglobin concentration increased from baseline by 2.43 g/dL (95% CI, 1.72 to 3.14; p < 0.001) and the mean percentage change of hemoglobin was 23.3% (95% CI, 15.8 to 30.7) at 12 months. With 45 U/kg, the mean hemoglobin concentration increased from baseline by 2.44 g/dL (95% CI, 1.49 to 3.39; p < 0.001) with a mean percentage change from baseline of 23.8% (95% CI, 13.7 to 33.9) at 12 months.
- At 12 months, the mean platelet counts increased from baseline in both treatment groups, with mean absolute changes from baseline of 50.9 × 10^9/L (95% CI, 24.0 to 77.8; p = 0.002) and 40.9 × 10^9/L (95% CI, 11.2 to 70.6; p =

0.01) in the 60 U/kg and 45 U/kg groups, respectively, and mean percer (95% CI, 28.7 to 103.2) and 66.4% (95% CI, 14.5 to 118.3) for the 60 U/kg an

- Mean spleen and liver volumes decreased from baseline to 12 months for both treatment groups.
    - For the 60 U/kg group, mean spleen volume decreased by 50.4% (95% CI, -62.1 to -38.6), from a median value of 14.0 MN to 5.8 MN (p = 0.003). For the 45 U/kg group, mean spleen volume decreased by 39.9% (95% CI, -51.9 to -27.9), from a median value of 14.5 MN to 9.5 MN (p = 0.009).
    - For the 60 U/kg group, mean liver volume decreased by 17.0% (95% CI, -27.0 to -7.0), from a median value of 1.5 MN to 1.2 MN (p = 0.03). For the 45 U/kg group, mean liver volume decreased by 6.2% (95% CI, -18.1 to 5.6), from a median value of 1.4 MN to 1.2 MN (p = 0.31). Based on the multiple testing strategy, the results for the 60 U/kg group were not statistically significant.
- The most common AEs, reported in ≥ 20% of patients, included headache, nasopharyngitis, traumatic injury (non-site specific), arthralgia, cough, pyrexia, dizziness, influenza, nasal congestion, vomiting, bone pain, and prolonged activated partial thromboplastin time.

Study II was a 9-month active-controlled (imiglucerase) trial in 34 patients ≥ 4 years of age (range, 3 to 73 years of age) with Gaucher disease-related anemia and either thrombocytopenia or organomegaly (Ben Turkia et al 2013). Patients were randomized to receive velaglucerase alfa 60 U/kg (n = 17) or imiglucerase 60 U/kg (n = 17) every other week.

- After 9 months, mean hemoglobin concentration increased from baseline by 1.6 g/dL in patients treated velaglucerase alfa. The treatment difference in mean change from baseline to 9 months (velaglucerase alfa - imiglucerase) was 0.135 g/dL ± 0.4. In the non-inferiority analysis, the lower bound of the 97.5% one-sided CI was -0.6 g/dL in the intention-to-treat population (n = 34); the predefined non-inferiority margin was -1 g/dL and therefore, non-inferiority was demonstrated (Ben Turkia et al 2013, FDA Summary review [VPRIV] 2010).
- There were no statistically significant differences in the secondary endpoints (i.e., included difference in changes from baseline between treatment groups in mean platelet count, and spleen and liver volumes) (Ben Turkia et al 2013)
- No patient treated with velaglucerase alfa developed antibodies to either drug, whereas 4 patients (23.5%) treated with imiglucerase developed immunoglobulin G (IgG) antibodies to imiglucerase, which were cross-reactive with velaglucerase alfa in 1 patient (Ben Turkia et al 2013).
- Most AEs were mild to moderate (Ben Turkia et al 2013).

In both Studies I and II, analysis of age and gender subgroups did not identify differences in response to velaglucerase alfa among these subgroups. The number of non-white patients in these studies was too small to adequately assess any difference in effects by race.

Study III was a 12-month, Phase 2/3, multinational, OL, single-arm trial in 40 patients ≥ 9 years of age (range, 9 to 71 years of age) who had been receiving imiglucerase therapy at doses ranging from 15 to 60 U/kg for a minimum of 30 consecutive months. Treatment with imiglucerase was discontinued, and velaglucerase alfa was administered every other week at the same number of units as the patient's previous imiglucerase dose. Adjustment of dosage was allowed by study criteria if needed in order to maintain clinical parameters (Elstein et al 2015).

- On average, hemoglobin concentrations and platelet counts remained stable throughout 12 months of velaglucerase alfa treatment. At 12 months, the median hemoglobin concentration was 13.5 g/dL (range, 10.8 to 16.1) vs the baseline of 13.8 g/dL (range, 10.4 to 16.5), and the median platelet count was 174 × 10^9/L (range, 24 to 408) vs the baseline of 162 × 10^9/L (range, 29 to 399).
- No patient required dosage adjustment during the 12-month treatment period.
- The most common drug-related AEs were fatigue and bone pain. Two patients, both treated with velaglucerase alfa 15 U/kg, discontinued the study after receiving ≥ full or partial dose of velaglucerase alfa: 1 due to an anaphylactic reaction and 1 due to lack of improvement at week 31 (FDA Medical review [VPRIV] 2010).

The long-term safety of VPRIV was assessed in Study IV (NCT00635427), an of 93 patients with Type 1 Gaucher disease ages 3 years and older. Patients who ha ble to participate in Study IV.

- In Studies I through IV, VPRIV was administered intravenously over 60 minutes at a maximum dose of 60 Units/kg every other week. Doses above 60 Units/kg were not studied in these trials. In patients treated with velaglucerase alfa, treatment-naïve patients (from Studies I and II) continued to show improvements in clinical parameters (hemoglobin concentration, platelet count, liver and spleen volumes), and patients previously treated with imiglucerase who switched to velaglucerase alfa (from Study III) maintained stability in clinical parameters (hemoglobin concentration, platelet count, liver and spleen volumes) vs baseline for up to 60 months of treatment with ERT (Clinicaltrials.gov Web site, VPRIV prescribing information 2024).

Clinical Guidelines:

Revised recommendations for the management of Gaucher disease in children (Kaplan et al 2013)

- According to the recommendations, every child and adolescent with symptomatic Gaucher disease should be treated with regular intravenous infusions of enzyme replacement therapy. There is no evidence that enzyme replacement therapy, even at high doses, can prevent or slow neurological progression in patients with Type 2 or Type 3 Gaucher disease.
- As enzyme replacement therapy is not recommended for Type 2 Gaucher disease, management should be focused on supportive care. For children with Type 3 Gaucher disease, enzyme replacement therapy is recommended to ameliorate the severe visceral manifestations

An update to The Paediatric Gaucher Disease in England: Guidelines for Assessment, Monitoring, and Enzyme Replacement Therapy was released in 2012 (Vellodi et al 2012).

- All children with Types I and III Gaucher disease should commence treatment with enzyme replacement therapy. Visceral disease in type III GD responds well, and so these children should be offered ERT. There is no evidence that the neurological features in patients with type II (neuronopathic Gaucher disease) show any response to ERT and therefore it should not be offered.

U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

VPRIV (velaglucerase alfa) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for patients with Type 1 Gaucher disease.

Cerezyme (imiglucerase) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for treatment of non-central nervous system (CNS) manifestations of Type 1 or Type 3 Gaucher disease in adults and pediatric patients.

Elelyso (taliglucerase alfa) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for treatment of patients 4 years of age and older with a confirmed diagnosis of Type 1 Gaucher disease.

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Policy History/Revision Information

Date	Summary of Changes
04/16/2025	Approved by OptumRx P&T Committee
03/18/2026	Annual Review. Updates made to include coverage for Cerezyme for Type 3 Gaucher. Updated background and references.

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, please check the member specific benefit plan document and any applicable federal or state mandates. The insurance reserves the right to modify its Policies and Guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

OptumRx may also use tools developed by third parties to assist us in administering health benefits. OptumRx Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.

Nondiscrimination & Language Access Policy

Discrimination is Against the Law. Aspirus Health Plan, Inc. complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability, or sex, (including sex characteristics, including intersex traits; pregnancy or related conditions; sexual orientation, gender identity and sex stereotypes), consistent with the scope of sex discrimination described at 45 CFR § 92.101(a)(2). Aspirus Health Plan, Inc. does not exclude people or treat them less favorably because of race, color, national origin, age, disability, or sex.

Aspirus Health Plan, Inc.:
Provides people with disabilities reasonable modifications and free appropriate auxiliary aids and services to communicate effectively with us, such as:

- Qualified sign language interpreters.
- Written information in other formats (large print, audio, accessible electronic formats, other formats).

Provides free language assistance services to people whose primary language is not English, which may include:

- Qualified interpreters.
- Information written in other languages.

If *you* need reasonable modifications, appropriate auxiliary aids and services, or language assistance services, contact the Nondiscrimination Grievance Coordinator at the address, phone number, fax number, or email address below.

If *you* believe that Aspirus Health Plan, Inc. has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, *you* can file a *grievance* with:

Nondiscrimination Grievance Coordinator
Aspirus Health Plan, Inc.
PO Box 1890
Southampton, PA 18966-9998
Phone: 1-866-631-5404 (TTY: 711)
Fax: 763-847-4010
Email: customerservice@aspirushealthplan.com

You can file a *grievance* in person or by mail, fax, or email. If *you* need help filing a *grievance*, the Nondiscrimination Grievance Coordinator is available to help *you*.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1.800.368.1019, 800.537.7697 (TDD)

Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html. This notice is available at Aspirus Health Plan, Inc.'s website: https://aspirushealthplan.com/webdocs/70021-AHP-NonDiscrim_Lang-Assist-Notice.pdf.

Language Assistance Services

Albanian: KUJDES: Nëse flitni shqip, për ju ka në dispozicion shërbime të asistencës gjuhësore, pa pagesë.Telefononi në 1-800-332-6501 (TTY: 711).

Arabic تنبيه: إذا كنت تتحدث اللغة العربية، فإن خدمات المساعدة اللغوية متاحة لك مجاناً. اتصل بن اعلى رقم الهاتف1-800-332-6501(رقم هاتف الصم والبك : 711)

French: ATTENTION: Si vous parlez français, des services d'aide linguistique vous sont proposés gratuitement. Appelez le 1-800-332-6501 (ATS: 711).

German: ACHTUNG: Wenn Sie Deutsch sprechen, stehen Ihnen kostenlos sprachliche Hilfsdienstleistungen zurVerfügung. Rufnummer: 1-800-332-6501 (TTY: 711).

Hindi: _यान द_: य_द आप ि हंदी बोलते ह_ तो आपके ि लए मु_त म_ भाषा सहायता सेवाएं उपल_ध ह_।1-800-332-6501 (TTY: 711) पर कॉल कर_।

Hmong: LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1-800-332-6501 (TTY: 711).

Korean: 주의: 한국어를 사용하시는 경우, 언어 지원 서비스를 무료로 이용하실 수 있습니다.1-800-332-6501 (TTY: 711)번으로 전화해 주십시오.

Polish: UWAGA: Jeżeli mówisz po polsku, możesz skorzystać z bezpłatnej pomocy językowej. Zadzwoń pod numer1-800-332-6501 (TTY: 711).

Russian: ВНИМАНИЕ: Если вы говорите на русском языке, то вам доступны бесплатные услуги перевода.Звоните 1-800-332-6501 (телетайп: 711).

Spanish: ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al1-800-332-6501 (TTY: 711).

Tagalog: PAUNAWA: Kung nagsasalita ka ng Tagalog, maaari kang gumamit ng mga serbisyo ng tulong sa wika nangwalang bayad. Tumawag sa 1-800-332-6501 (TTY: 711).

Traditional Chinese: 注意：如果您使用繁體中文， 您可以免費獲得語言援助服務。請 致電 1-800-332-6501 (TTY: 711)

Vietnamese: CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1-800-332-6501 (TTY: 711).

Pennsylvania Dutch: Wann du Deitsch (Pennsylvania German / Dutch) schwetzscht, kannscht du mitaus Koschte ebbergricke, ass dihr helft mit die englisch Schprooch. Ruf selli Nummer uff: Call 1-800-332-6501 (TTY: 711).

Lao: ໂປດຊາບ: ຖ້າວ່າ ທ່ານເວົ້າພາສາ ລາວ, ການບໍລິການຊ່ວຍເຫຼືອດ້ານພາສາ,ໂດຍບໍ່ເສັງຄ່າ, ແມ່ນມີພ້ອມໃຫ້ທ່ານ. ໂທຣ 1-800-332-6501 (TTY: 711).