# PDL NEW DRUG REVIEW

**Proprietary Name: Vpriv®**
**Common Name: velaglucerase alfa**
**PDL Category: Agents for Gaucher Disease**

| Comparable Products | Preferred Drug List Status |
|---|---|
| Cerezyme® | Non-Preferred |

## Summary


**Indications and Usage:** For long-term enzyme replacement therapy (ERT) for pediatric and adult patients with type 1 Gaucher disease. This is a pregnancy category B medication. The safety and efficacy of use in children under the age of 3 have not been established.

**Dosage Forms:** Injection: single-use preservative-free vials with lyophilized powder for reconstitution: 200U/vial and 400U/vial. Vials should be stored in a refrigerator at 2-8 °C (36-46°F).

**Recommended Dosage:** 60U/kg administered as a 60-minute IV infusion every other week. Doses ranging from 15-60U/kg once every other week have been evaluated in clinical trials. Those currently being treated with imiglucerase may be switched to Vpriv®.

**Common Adverse Drug Reactions:** There was no placebo data available. The % listed include those that were naïve to ERT vs those that switched from imiglucerase to Vpriv®. The most common adverse events reported include headache (35.2% vs 30%), dizziness (22.2% vs 7.5%), abdominal pain (18.5% vs 15%), nausea (5.6% vs 10%), back pain (16.7% vs 17.5%), joint knee pain (14.8% vs 7.5%), upper respiratory tract infection (31.5% vs 30%), activated partial thromboplastin time prolonged (11.1% vs 5%), infusion-related reaction (51.9% vs 22.5%), pyrexia (22.2% vs 12.5%), and asthenia/fatigue (13% vs 12.5%). Bone pain, tachycardia, rash, urticaria, flushing, hypertension, and hypotension were less commonly reported adverse events (>3% in treatment-naïve and >2% in those who switched).

**Contraindications:** There are currently no contraindications listed in the prescribing information.

**Manufacturer:** Shire Human Genetic Therapies, Inc

**Analysis:** Velaglucerase alfa, the active ingredient of VPRIV®, is a glycoprotein consisting of 497 amino acids, with the same amino acid sequence as the human enzyme glucocerebrosidase. Velaglucerase alfa catalyzes the hydrolysis of glucocerebroside to glucose and ceramide. The efficacy of velaglucerase alfa was established in a 2 studies with patients who were ERT naïve and in 1 study with patients who were receiving imiglucerase treatment. In study 1, 2 doses were given (both 45U/kg and 60U/kg) and mean changes were assessed from baseline. Results from study 1 suggest the spleen volume change (%body weight [BW]) with doses 45U/kg and 60U/kg were 1.9 and -1.9, respectively, while the liver volume change (%BW) was -0.3 and -0.84, respectively. The HbA1c mean change was 2.4g/dL and 2.4g/dL, respectively, while the platelet counts change (X $10^9$/L) was 41 and 51, respectively. Study 2 was a 9-month, double-blind, active-controlled (imiglucerase) study (N=34) with a primary endpoint of hemoglobin change. The mean treatment difference in change from baseline to 9 months (Vpriv® minus imiglucerase) was 0.1g/dL. This suggests that VPRIV® was as effective as imiglucerase. Study 3 was an open-label, single-arm, 12 month study (N=40). After 12 months, the median HbA1c level was 13.5g/dL vs the baseline of 13.8g/dL, while the median platelet count was 174 X$10^9$/L vs 162 X$10^9$/L for baseline. Results suggest that therapeutic response was maintained with 12 months of Vpriv® after switching from imiglucerase.

There is no evidence at this time to support that Vpriv is more efficacious or safer than other currently available products. It is recommended that Vpriv® remain non-preferred to confirm diagnosis.

**PDL Placement:**

- ☐ **Preferred**
- ☒ **Non-Preferred**
- ☐ **Preferred with Conditions**

## References

[1] VPRIV [package insert]. Cambridge, MA: Shire Human Genetic Therapies, Inc; 2012.