PDL NEW DRUG REVIEW

Proprietary Name: Elelyso®
Common Name: taliglucerase alfa
PDL Category: Agents for Gaucher Disease

Comparable Products	Preferred Drug List Status
Cerezyme®	Non-Preferred

Summary

Indications and Usage: For use as long-term enzyme replacement therapy (ERT) for adults with confirmed diagnosis of Type 1 Gaucher disease. This is a pregnancy category B medication. The safety and efficacy of use in children under the age of 18 have not been established.

Dosage Forms: Injection: lyophilized powder for reconstitution; 200U/single-use vial. Store at 2-8°C (36-46°F), and protect vials from light.

Recommended Dosage: 60U/kg administered once every 2 weeks as a 60-120 minute IV infusion. The dosage adjustments are to be based on achievement and maintenance of individual therapeutic goals. Dose ranges studied in clinical trials were 11U/kg-73U/kg every other week.

Common Adverse Drug Reactions: There was no placebo data available. There were 2 clinical studies that assessed adverse events and were reported by ≥10% of those treated with Elelyso® in both studies (thus a range). The most frequently reported adverse events includes infusion reaction (44-46%), upper respiratory tract infection/nasopharyngitis (18-22%), pharyngitis/throat infection (4-19%), headache (11-19%), arthralgia (11-13%), influenza/flu (4-13%), urinary tract infection/pyelonephritis (9-11%), back pain (3-11%), and extremity pain (0-11%). Other less commonly reported adverse events (>2%) included fatigue, pain, pruritus, diarrhea, dizziness, nausea, bone pain, abdominal pain, erythema, flushing, peripheral edema, muscle spasms, paresthesia, dyspnea, throat irritation, asthenia, chest discomfort, infusion site pain, insomnia, rash, and skin irritation.

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

Manufacturer: Pfizer Labs

Analysis: Taliglucerase alfa, the active ingredient of Elelyso®, is a hydrolytic lysosomal glucocerebroside-specific enzyme that is produced by recombinant DNA technology. It differs from native human glucocerebroside by 2 amino acids at the N terminal and up to 7 amino acids at the C terminal. It works by catalyzing the hydrolysis of glucocerebroside to glucose and ceramide. It was reported in clinical trials to reduce both spleen and liver size, as well as improve anemia and thrombocytopenia.

Efficacy was assessed in one 9-month double-blind, randomized trial (N=31) in those with Gaucher disease-related enlarged spleens (>8 times normal size) and thrombocytopenia (<120,000/mm3). Furthermore, some had enlarged livers and anemia at baseline. Subjects took either 60U/kg or 30U/kg. In those taking the 60U/kg, the mean change in spleen volume %BW (body weight) was -1.3% (-6.6 multiple of normal (MN) mean change). For the change in liver volume %BW, there was a -0.6% mean change (-0.3 MN mean change). There was a 2.2g/dL mean change in hemoglobin and 41,494/mm^3 mean change in platelet count. Results for those taking 30U/kg were also assessed in the study. For change in spleen volume %BW, there was a -0.9% mean change (-4.5 multiple of normal (MN) mean change). For the change in liver volume %BW, there was a -0.6% mean change (-0.2 MN mean change). There was a 1.6g/dL mean change in hemoglobin and 11,427/mm^3 mean change in platelet count.

Study 2 was an open-label single arm study that included those switching from imiglucerase to Elelyso® (N=25) and who had been receiving imiglucerase for at least 2 years. Spleen volume %BW at baseline vs end of study was 1.1% vs 1.0% (MN 5.5 vs 5.1). Liver volume %BW at baseline vs endpoint was 2.4% vs 2.3% (MN 1.0 vs 0.9). Hemoglobin at baseline vs endpoint was 13.6g/dL vs 13.4g/dL, and mean platelet count was 160,447 vs 165,654. Results suggest that taliglucerase alfa was as effective as imiglucerase in maintaining therapeutic response.

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

PDL Placement:

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

References

[1] Elelyso [package insert]. New York, NY: Pfizer Labs, a division of Pfizer Inc; 2012.