PREFERRED SPECIALTY MANAGEMENT POLICY

POLICY: Gaucher Disease – Substrate Reduction Therapy Preferred Specialty Management Policy
- Cerdelga™ (eliglustat capsules – Genzyme)
- Zavesca® (miglustat capsules – Actelion Pharmaceuticals; generic)

REVIEW DATE: 08/12/2020

OVERVIEW

Gaucher disease is a rare autosomal recessive, inherited, lysosomal storage disorder caused by a deficiency of the lysosomal enzyme β-glucocerebrosidase.[1-3] Cerdelga and Zavesca/generic miglustat (AB-rated generic to Zavesca) are substrate reduction therapy agents indicated for long-term therapy in patients with a confirmed diagnosis of Type 1 Gaucher disease. Cerdelga is specifically indicated for the long-term treatment of adult patients with Gaucher disease type 1 who are cytochrome P450 2D6 extensive metabolizers, intermediate metabolizers, or poor metabolizers as detected by an FDA-cleared test.[1] Zavesca is indicated as monotherapy for the treatment of adult patients with mild to moderate Gaucher disease type 1 for whom enzyme replacement therapy is not a therapeutic option (e.g., due to allergy, hypersensitivity, or poor venous access).[2]

POLICY STATEMENT

This Preferred Specialty Management program has been developed to encourage the use of Preferred Products. For all medications (Preferred and Non-Preferred), the patient is required to meet the respective standard *Prior Authorization Policy* criteria. The program also directs the patient to try both of the Preferred Products (Cerdelga and generic miglustat) prior to the approval of the Non-Preferred Product (Zavesca). Requests for the Non-Preferred Product will also be reviewed using the exception criteria (below). Patients meeting the standard *Prior Authorization Policy* criteria for the Non-Preferred Product who have not tried the Preferred Products will be offered a review for one of the Preferred Products. All approvals for are provided for the duration noted below.

Documentation: Documentation is required for use of Cerdelga and generic miglustat as noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, prescription claims records, and prescription receipts.

Automation: None.

Preferred Product: Cerdelga, generic miglustat
Non-Preferred Product: Zavesca

RECOMMENDED EXCEPTION CRITERIA

Non-Preferred Product	Exception Criteria
Zavesca	**1. Gaucher Disease Type I.** **A)** Approve for 1 year if the patient meets the following criteria (i and ii): **i.** Patient meets the standard *Gaucher Disease Substrate Reduction Therapy – Miglustat (Zavesca) Prior Authorization* criteria; AND **ii.** Patient meets BOTH of the following criteria (a and b): **a)** Patient has tried BOTH Cerdelga (eliglustat capsules) **[documentation required]** and generic miglustat **[documentation required]**; AND **b)** Brand Zavesca is being requested due to a formulation difference in the inactive ingredient(s) [e.g., preservatives] between the Brand and the bioequivalent generic product, which, per the prescriber has or would result in a significant allergy or serious adverse reaction. **B)** For patients who have not tried Cerdelga and generic miglustat and do not meet the exception criteria (criteria 1Aii), offer to review for one of the Preferred Products using the standard *Gaucher Disease Substrate Reduction Therapy – Cerdelga Prior Authorization* criteria or *Miglustat (Zavesca) Prior Authorization* criteria. **2. Other Conditions.** Approve for 1 year if the patient meets the standard *Gaucher Disease Substrate Reduction Therapy – Miglustat (Zavesca) Prior Authorization* criteria.

REFERENCES

1. Cerdelga™ capsules [prescribing information]. Waterford, Ireland: Genzyme; August 2018.
2. Zavesca® [prescribing information]. South San Francisco, CA: Actelion Pharmaceuticals US Inc.; November 2017.