PRIOR AUTHORIZATION POLICY

POLICY: Gaucher Disease Substrate Reduction Therapy – Cerdelga® (eliglustat capsules – Genzyme)

DATE REVIEWED: 05/06/2020

OVERVIEW

Cerdelga, a glucosylceramide synthase inhibitor, is indicated for the long-term treatment of adult patients with Gaucher Disease type 1 who are cytochrome P450(CYP)2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.[1] The Cerdelga prescribing information notes the following limitations of use: patients who are CYP2DE ultra-rapid metabolizers (URMs) may not achieve adequate concentrations of Cerdelga to achieve a therapeutic effect; and a specific dosage cannot be recommended for patients for whom the CYP2D6 genotype cannot be determined (indeterminate metabolizers).

DISEASE OVERVIEW

Gaucher disease is caused by a deficiency in the lysosomal enzyme β-glucocerebrosidase.[1] This enzyme is responsible for the breakdown of glucosylceramide into glucose and ceramide. In Gaucher disease, deficiency of the enzyme β-glucocerebrosidase results in the accumulation of glucosylceramide substrate in lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells." Cerdelga is a specific inhibitor of the enzyme glycosylceramide synthase, which is responsible for producing the substrate glucosylceramide; hence Cerdelga functions as a substrate reduction therapy.

POLICY STATEMENT

Prior authorization is recommended for prescription benefit coverage of Cerdelga. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Cerdelga as well as the monitoring required for adverse events and long-term efficacy, approval requires Cerdelga to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Cerdelga is recommended in those who meet the following criteria:

FDA-Approved Indications

1. **Gaucher Disease Type I.** Approve for 1 year if the patient meets the following criteria (A and B):
   - **A)** The patient is a cytochrome P450(CYP) 2D6 extensive metabolizer (EM), intermediate metabolizer (IM), or poor metabolizer (PM) as detected by an approved test; AND
   - **B)** Cerdelga is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of Gaucher Disease or related disorders.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Cerdelga has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Cerdelga™ capsules [prescribing information]. Waterford, Ireland: Genzyme; August 2018.