PRIOR AUTHORIZATION POLICY

POLICY: Gaucher Disease – Substrate Reduction Therapy – Cerdelga Prior Authorization Policy
- Cerdelga® (eliglustat capsules – Genzyme)

REVIEW DATE: 05/29/2024; selected revision 08/14/2024

OVERVIEW

Cerdelga, a glucosylceramide synthase inhibitor, is indicated for the long-term treatment of adults 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

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 the 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 Indication

Gaucher Disease Type 1. Approve for 1 year if the patient meets ALL of the following (A, B, and C):
Patient is a cytochrome P450 2D6 extensive metabolizer, intermediate metabolizer, or poor metabolizer as detected by an approved test; AND
The diagnosis is established by ONE of the following (i or ii):
Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts; OR
Molecular genetic test documenting biallelic pathogenic glucocerebrosidase (GBA) gene variants; AND
The medication is prescribed by or in consultation with a geneticist, endocrinologist, metabolic disorder subspecialist, or a physician who specializes in the treatment of Gaucher disease or related disorders.

Conditions Not Recommended for Approval
Coverage of Cerdelga is not recommended in the following situations:

Concomitant Use with Other Approved Therapies for Gaucher Disease. Concomitant use with other treatments approved for Gaucher disease has not been evaluated. Of note, examples of medications approved for Gaucher disease include Cerezyme (imiglucerase intravenous infusion), Elelyso (taliglucerase alfa intravenous infusion), Vpriv (velaglucerase alfa intravenous infusion), and Zavesca (miglustat capsules).

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

References
Cerdelga® capsules [prescribing information]. Waterford, Ireland: Genzyme; January 2024.