Effective Date.....**12/1/2024**
Coverage Policy Number.....**IP0441**

Eliglustat

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Related Coverage Resources

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment and have discretion in making individual coverage determinations. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

This policy supports medical necessity review for eliglustat (**Cerdelga**®).

Medical Necessity Criteria

Eliglustat (**Cerdelga**®) **is considered medically necessary when the following are met:**

1. **Gaucher Disease Type 1.** Individual meets **ALL** of the following criteria (A, B, C, and D):
   A. There is documentation of **EITHER** of the following (i or ii):
      i. Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts
      ii. Confirmation of molecular genetic test documenting biallelic pathogenic glucocerebrosidase *(GBA)* gene variants
   B. The individual is **ONE** of the following as detected by an approved test (i, ii, or iii):
      i. CYP2D6 extensive metabolizer (EM)
      ii. CYP2D6 intermediate metabolizer (IM)

iii. CYP2D6 poor metabolizer (PM)

C. 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.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Receipt of sample product does not satisfy any criteria requirements for coverage.

Reauthorization Criteria

Continuation of Eliglustat (Cerdelga) is considered medically necessary for Gaucher Disease Type 1 when the above medical necessity criteria are met AND there is documentation of beneficial response.

Authorization Duration

Initial and reauthorization-approval duration: 12 months

Conditions Not Covered

Any other use is considered experimental, investigational or unproven including the following (this list may not be all inclusive; criteria will be updated as new published data are available):

1. **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).

Background

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.

References

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

Revision Details

Type of Revision	Summary of Changes	Date
Selected Revision	**Gaucher Disease Type 1:**	12/1/2024

	Removed criterion “Individual is age 18 years or older” **Updated** criterion **from** “deficiency of glucosylceramidase [also known as acid β-glucosidase or glucocerebrosidase] in peripheral blood leukocytes or other nucleated cells” **to** “demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts.” **Updated** criterion **from** “Confirmation of biallelic pathogenic variants in the GBA gene” **to** “Confirmation of molecular genetic test documenting biallelic pathogenic glucocerebrosidase (GBA) gene variants.” **Updated** criterion **from** “Individual is ONE of the following: CYP2D6 extensive metabolizer (EM), CYP2D6 intermediate metabolizer (IM) or CYP2D6 poor metabolizer (PM)” **to** “Individual is ONE of the following as detected by an approved test: CYP2D6 extensive metabolizer (EM), CYP2D6 intermediate metabolizer (IM) or CYP2D6 poor metabolizer (PM)” **Reauthorization Criteria:** **Updated** criterion **from** “Eliglustat (Cerdelga) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response” **to** “Continuation of Eliglustat (Cerdelga) is considered medically necessary for Gaucher Disease Type 1 when the above medical necessity criteria are met AND there is documentation of beneficial response.” **Conditions Not Covered:** Concomitant use with other approved therapies for Gaucher disease was added.	

The policy effective date is in force until updated or retired.