Plan refers to Boston Medical Center Health Plan, Inc. and its affiliates and subsidiaries offering health coverage plans to enrolled members. The Plan operates in Massachusetts under the trade name Boston Medical Center HealthNet Plan and in other states under the trade name Well Sense Health Plan.

Pharmacy Policy

CERDELGA®

Policy Number: 9.069
Version Number: 1.0
Version Effective Date: 05/02/2017

Product Applicability □ All Plan+ Products

Well Sense Health Plan
☒ New Hampshire Medicaid
☒ NH Health Protection Program
□ ____________________

Boston Medical Center HealthNet Plan
☒ MassHealth
☒ Qualified Health Plans/ConnectorCare/Employer Choice Direct
□ Senior Care Options
□ ____________________

Note: Disclaimer and audit information is located at the end of this document.

Policy Summary

The Plan will authorize coverage of Cerdela® when appropriate criteria are met.

Description of Item or Service

Cerdela® is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1) who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test. Patients who are CYP2D6 ultra-rapid metabolizers (URMs) may not achieve adequate concentrations of Cerdela® to achieve a therapeutic effect. A specific dosage cannot be recommended for those patients whose CYP2D6 genotype cannot be determined (indeterminate metabolizers).

Type 1 Gaucher disease is estimated to affect about 6,000 people in the United States. Gaucher disease occurs in people who do not produce enough of an enzyme called glucocerebrosidase. The enzyme deficiency causes fatty materials to collect in the spleen, liver and bone marrow. The major signs of Gaucher disease include liver and spleen enlargement, low red blood cell counts (anemia), low blood platelet counts and bone problems.

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Cerdelga® is a hard gelatin capsule containing eliglustat that is taken orally. In patients with Gaucher disease Type 1, the drug slows down the production of the fatty materials by inhibiting the metabolic process that forms them.

The safety and effectiveness of Cerdelga® were evaluated in two clinical trials with 199 participants with Type 1 Gaucher disease.

In one randomized, double-blind, placebo-controlled, multicenter clinical trial the safety and effectiveness of Cerdelga® were evaluated in 40 participants with Type 1 Gaucher’s disease who had not previously received enzyme replacement therapy. Subjects received the drug at a starting dose of 42 mg two times a day, with most receiving a dose of 84 mg two times a day after four weeks. Study participants continued the drug for nine months.

Compared to placebo, treatment with Cerdelga® resulted in a greater reduction in spleen volume from baseline to the end of the study (by the 39th week), the trial’s primary endpoint. Cerdelga® also resulted in greater improvement in liver volume, blood platelet count, and red blood cell (hemoglobin) level, compared to placebo.

The other trial sought to determine the safety and effectiveness of Cerdelga® compared to enzyme replacement therapy in 159 participants with Type 1 Gaucher disease previously treated and stabilized on enzyme replacement therapy. Subjects in the trial received either the enzyme replacement therapy drug imiglucerase or Cerdelga. The trial demonstrated that treatment with Cerdelga® resulted in similar stabilization of hemoglobin level, platelet count and spleen and liver volume as imiglucerase.

The most commonly observed side effects in the Cerdelga® clinical trials were fatigue, headache, nausea, diarrhea, back pain, pain in extremities, and upper abdominal pain.

**Policy**

The Plan may authorize coverage of Cerdelga® for members meeting the following criteria:

**Prior Authorization**

**Initial Approval Criteria** – *(Duration of Approval – 1 year)*

A prior authorization request will be required for all prescriptions for Cerdelga®. Requests will be approved when the following criteria are met:

Documentation of the following:

1. Member has been diagnosed with Type 1 Gaucher’s Disease and is symptomatic (i.e. radiologic evidence of skeletal disease, platelet count 2.5 times normal size, spleen > 15 times normal size); **AND**
2. Member is 18 years or older; **AND**
3. Member has been tested using an FDA cleared test to determine the patient’s CYP2D6 genotype and has been classified as an extensive metabolizers (EMs), intermediate metabolizers (IM) or poor metabolizers (PM); **AND**
4. Either one of the following:
   a. Member is a EM or IM and requested dose is 84mg twice daily; **or**
   b. Member is a PM and requested dose is 84 mg once daily; **AND**
5. Member has tried and failed enzyme replacement therapy for at least 6 months or patient is unable to tolerate enzyme replacement therapy (Cerezyme, Vpriv, Elelyso).

**Reauthorization Criteria** – *(Duration of Approval – 1 year)*

Documentation of the following:

1. Patient is responding to treatment (improved platelet count, decreased hepatomegaly and splenomegaly); **AND**
2. Patient is tolerating treatment

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Limitedations

The Plan will not approve coverage of Cerdelga® in the following instances:

- When the above criteria are not met.

Clinical Background Information and References

1. Cerdelga (eliglustat) [prescribing information]. Waterford, Ireland: Genzyme Ireland; August 2014.
3. 

Appendix A – Quantity Limitations for Cerdelga®

<table>
<thead>
<tr>
<th>Medication Name and Strength</th>
<th>Maximum Quantity</th>
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<tbody>
<tr>
<td>Cerdelga®</td>
<td>EMs and IMs: 56 capsules / 28 days PMs: 28 capsules /28 days</td>
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<table>
<thead>
<tr>
<th>Original Approval Date</th>
<th>Original Effective Date</th>
<th>Policy Owner</th>
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<tr>
<td>01/12/2017</td>
<td>05/03/2017</td>
<td>Pharmacy Services</td>
<td>Pharmacy &amp; Therapeutics (P&amp;T) Committee</td>
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</tbody>
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Next Review Date

01/11/2018

Other Applicable Policies

9.002 Mandatory Generic Substitution Policy
9.015 Quantity Limitation Program

Reference to Applicable Laws and Regulations, If Any

Disclaimer Information

Medical Policies are the Plan’s guidelines for determining the medical necessity of certain services or supplies for purposes of determining coverage. These Policies may also describe when a service or supply is considered experimental or investigational, or cosmetic. In making coverage decisions, the Plan uses these guidelines and other

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Plan Policies, as well as the Member’s benefit document, and when appropriate, coordinates with the Member’s health care Providers to consider the individual Member’s health care needs.

Plan Policies are developed in accordance with applicable state and federal laws and regulations, and accrediting organization standards (including NCQA). Medical Policies are also developed, as appropriate, with consideration of the medical necessity definitions in various Plan products, review of current literature, consultation with practicing Providers in the Plan’s service area who are medical experts in the particular field, and adherence to FDA and other government agency policies. Applicable state or federal mandates, as well as the Member’s benefit document, take precedence over these guidelines. Policies are reviewed and updated on an annual basis, or more frequently as needed. Treating providers are solely responsible for the medical advice and treatment of Members.

The use of this Policy is neither a guarantee of payment nor a final prediction of how a specific claim(s) will be adjudicated. Reimbursement is based on many factors, including member eligibility and benefits on the date of service; medical necessity; utilization management guidelines (when applicable); coordination of benefits; adherence with applicable Plan policies and procedures; clinical coding criteria; claim editing logic; and the applicable Plan – Provider agreement.

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Cerdelga®