MEDICAL POLICY STATEMENT

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<tr>
<th>Effective Date</th>
<th>Next Annual Review Date</th>
<th>Last Review / Revision Date</th>
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<td>06/15/2011</td>
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Author
Shelley Jones RN, CCM, Wendy Null RPh

CSMG Medical Policy Statements are derived from literature based and supported clinical guidelines, nationally recognized utilization and technology assessment guidelines, other medical management industry standards, and published MCO clinical policy guidelines. Medically necessary services are those health care services or supplies which are proper and necessary for the diagnosis or treatment of disease, illness, or injury and without which the patient can be expected to suffer prolonged, increased or new morbidity, impairment of function, dysfunction of a body organ or part or significant pain and discomfort. These services meet the standards of good medical practice in the local area, are the lowest cost alternative and are not provided mainly for the convenience of the member or provider.

A. SUBJECT
Alglucerase (Ceredase) Infusion

B. BACKGROUND
Alglucerase (Ceredase) is a modified form of the enzyme, β-glucocerebrosidase (β-D-glucosyl-N-acylphosphosine glucohydrolase). Alglucerase (Ceredase) catalyzes the hydrolysis of the glycolipid, glucocerebroside, to glucose and ceramide as part of the normal degradation pathway for membrane lipids. Glucocerebroside is primarily derived from hematologic cell turnover. Gaucher disease is characterized by a functional deficiency in β-glucocerebrosidase enzymatic activity and the resultant accumulation of lipid glucocerebroside in tissue macrophages, which become engorged and are termed Gaucher cells.

The patient selection criteria outlined was derived from the FDA-approved prescribing information for alglucerase (Ceredase), the studies that were presented to the FDA in support of the pre-market approval application, and studies in the peer-reviewed published medical literature. The FDA label indication found in the manufacturer prescribing information and described below is type I Gaucher disease. Coverage decisions for conditions other than the above FDA approved indications will be reviewed on a case-by-case basis if proven effective through research documentation. The requesting provider will need to support his exception request with the appropriate literature.

C. POLICY
CareSource will approve the use of alglucerase (Ceredase) and consider its use as medically necessary when the following criteria have been met for:
- Type I Gaucher Disease
- Chronic neuropathic Type 3 Gaucher's disease who exhibit clinically significant non-neurological manifestations of the disease

All other uses of alglucerase (Ceredase) are considered experimental/investigational, and therefore, not covered such as replacement therapy in Type 2 disease, the neuronopathic form, as it does not alter the neurodegenerative course of the Type 2 disease and neuronopathic 3 Gaucher disease.
Type I Gaucher Disease

Alglucerase (Ceredase) is indicated for use as a long-term enzyme replacement therapy for children, adolescents and adult patients with a confirmed diagnosis of Type I Gaucher disease who exhibit signs and symptoms that are severe enough to result in one or more of the following conditions: moderate-to-severe anemia, thrombocytopenia with bleeding tendency, bone disease, significant hepatomegaly or splenomegaly.

Prior Authorization Criteria: (for adults, 18 years of age or older)

- Documented diagnosis of Type 1 Gaucher disease
- Exhibits one of the signs of skeletal progression confirmed by radiological assay
  - Joint deterioration
  - Pathological fracture
  - Avascular necrosis
  - Marrow infiltration
  - Documented Osteopenia

OR

- Exhibits two or more of the following:
  - Hemoglobin < 12.5 g/dl for males and < 11.5 g/dl for females
  - Platelet count < 120,000/mm
  - Hepatosplenomegaly

- Prescribed by an endocrinologist or under the recommendation of an endocrinologist

Prior Authorization Criteria: (for children, 2 years of age or older)

- Documented diagnosis of Type 1 Gaucher disease
- Exhibits one of the following:
  - Bone pain
  - Abdominal pain
  - Fatigue
  - Marrow infiltration
  - Cachexia
  - Weakness
  - Limited Exertion
  - Hepatomegaly > 2.5 times normal size
  - Splenomegaly > 15 times normal size

OR

- Exhibits one of the signs of skeletal progression confirmed by radiological assay
  - Avascular necrosis of bone
  - Destructive lesions of bone
  - Erlenmeyer Flask Deformity (EFD)

OR

- Thrombocytopenia (platelet count < 60,000/mm)
OR
- Hemoglobin < 2g/dl below the normal limit for age, weight and sex
- Growth failure confirmed by growth charts

- Prescribed by a pediatric endocrinologist or under the recommendation of a pediatric endocrinologist

**Chronic Neuropathic Type 3 Gaucher’s Disease**

Alglucerase (Ceredase) is indicated for use as a long-term enzyme replacement therapy for patients with a confirmed diagnosis of Chronic Neuropathic Type 3 Gaucher’s Disease who exhibit clinically significant non-neurological manifestations of the disease. Some individuals with Type 3 Gaucher disease never develop neurologic symptoms, and enzyme replacement therapy for these individuals is most beneficial.

Enzyme replacement therapy has been shown to provide significant benefits in controlling visceral, bone and hematologic symptoms.

**Prior Authorization Criteria:**
- Documented diagnosis of Chronic Neuropathic Type 3 Gaucher’s disease
- Documented evaluation of neurological function
- Documented evaluation of visceral, bone and hematologic systems
- Prescribed by a endocrinologist or under the recommendation of a endocrinologist

**NOTE:** Safety not established in children younger than 2 years of age.

**NOTE:** Documented diagnosis must be confirmed by portions of the individual’s medical record, which will confirm the presence of disease and will need to be supplied with prior authorization request. These medical records may include, but not limited to, test reports, chart notes from provider’s office or hospital admission notes.

For Special Needs Plan members, reference the below link to search for Applicable National Coverage Descriptions (NCD) and Local Coverage Descriptions (LCD):

**For Medicare**

**NCD for alglucerase (Ceredase)**

Medicare does not have a National Coverage Determination (NCD) for aglucerase (Ceredase). In general, Medicare covers outpatient (Part B) drugs that are furnished “incident to” a physician’s service provided that the drugs are not usually self-administered by the patients who take them. Refer to the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, section 50 Drugs and Biologicals at:


Local Coverage Determinations (LCDs) for aglucerase (Ceredase) do not exist at this time. (Accessed April 6, 2011)
Safety
CareSource will only review requests for alglucerase (Ceredase) if the patient has none of the following contraindications:
- Hypersensitivity to alglucerase

Precautions
Ensure that patients receive antipyretics and/or antihistamines prior to infusion. If an infusion reaction occurs, regardless of pretreatment, decreasing the infusion rate, temporarily stopping the infusion, and/or administration of additional antipyretics and/or antihistamines may ameliorate the symptoms. If severe hypersensitivity or anaphylactic reactions occur, immediately discontinue the infusion of alglucerase (Ceredase) and initiate appropriate treatment. Life-threatening anaphylactic reactions have been observed in some patients during or up to 3 hours after alglucerase (Ceredase) infusions.

Pregnancy Risk Factor = C
Animal reproductive studies have not been conducted with alglucerase (Ceredase). It is also not known whether alglucerase (Ceredase) can cause fetal harm when administered to a pregnant woman, or can affect reproductive capacity. Alglucerase (Ceredase) should be given to a pregnant woman only if clearly needed.

Since alglucerase (Ceredase) may be excreted in human milk, caution should be exercised when alglucerase (Ceredase) is administered to a nursing woman.

Conditions of Coverage

| Quantity Limitations | Initial dose is determined for the individual patient and depends upon age at presentation, co-morbid conditions, the site(s) and extent of involvement.  
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<td>Initial dosage may be as little as 2.5 units/kg 3 times a week up to 60 units/kg as frequently as once a week or as infrequently as every 4 weeks. The minimum recommended starting dose for children with Type 1 Gaucher’s Disease is 30 units/kg</td>
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<td>J-Code</td>
<td>J0205</td>
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<tr>
<td>NDC</td>
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<td>Applicable ICD-9 Codes</td>
<td>272.7 Gaucher’s Disease</td>
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| Place Of Service | Office, Outpatient, Home  
**Preferred place of service is in the home.**  
**Note:** CareSource supports administering injectable medications in various setting, as long as those services are furnished in the most appropriate and cost-effective setting that are
supportive of the patient's medical condition and unique needs and condition.

The decision on the most appropriate setting for administration is based on the member’s current medical condition and any required monitoring or additional services that may coincide with the delivery of the specific medication.

| Authorization Period | Coverage may be approved for up to 12 weeks. Coverage may be approved for re-treatment if it meets initial diagnosis criteria and evidence of a beneficial response. |

D. REVIEW / REVISION HISTORY

6/15/2011

E. REFERENCES


The medical Policy Statement detailed above has received due consideration as defined in the Medical Policy Statement Policy and is approved.

May 31, 2011

Chief Medical Officer

Date

May 31, 2011

Senior Medical Director

Date