BENEFIT DESCRIPTION AND LIMITATIONS OF COVERAGE

ITEM: VPRIV™ (velaglucerase alfa for injection)

PRODUCT LINES: Advantage
                 Commercial HMO/PPO/CDHP

COVERED UNDER: Advantage: Medical
                HMO: Medical
                PPO/CDHP: Rx

DESCRIPTION: Catalyzes the hydrolysis of glucocerebrosidase, reducing the amount of accumulated glucocerebrosidase.¹

CPT/HCPCS Code: J3385

Company Supplying: Shire Human Genetic Therapies, Inc.

Setting: Intravenous (IV) injection

Coverage Criteria: Medical literature

Approval Period: 6 months

Recommended Authorization Criteria
Velaglucerase alfa should be prescribed by, or in consultation with, a physician who specializes in the treatment of inherited metabolic disorders.

FDA-Approved Indications

1. **Type 1 Gaucher’s disease.** Approve when the following criteria are met (a AND b AND c AND d):
   a. Patients aged 4 years and older¹-³ AND
   b. A diagnosis of type I Gaucher Disease¹-⁷ AND
   c. Dosage not to exceed the recommended dose of 60 units/kg¹-⁷ AND
   d. Documented failure of taliglucerase alfa therapy in patients 18 years or older
Continued approval should demonstrate evidence of clinical benefit (improvement or stability) with the following parameters as a guide:

**Table 1: Therapeutic goals for the treatment of type 1 Gaucher’s disease**

<table>
<thead>
<tr>
<th>Disease Effect</th>
<th>Within 12 months</th>
<th>12 – 24 months</th>
<th>&gt; 24 months</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Anemia</strong></td>
<td>N/A</td>
<td>Women and children: ≥ 11 mg/dL</td>
<td>Maintain hemoglobin values achieved in the first 12-24 months</td>
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<td>Men: &gt; 12 mg/dL</td>
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<tr>
<td><strong>Thrombocytopenia</strong></td>
<td>All patients: increase platelet count</td>
<td>Intact spleen and moderate baseline thrombocytopenia*: approaching low normal by year 2</td>
<td>All patients: maintenance of stable platelet counts</td>
</tr>
<tr>
<td></td>
<td>Splenectomy: normalization of platelet count</td>
<td>Intact spleen and severe baseline thrombocytopenia^: double from baseline</td>
<td>Intact spleen and severe baseline thrombocytopenia^: continued slight increase; not anticipated to normalize</td>
</tr>
<tr>
<td></td>
<td>Intact spleen and moderate baseline thrombocytopenia*: 1.5 – 2-fold increase</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Intact spleen and severe baseline thrombocytopenia^: 1.5-fold increase</td>
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<tr>
<td><strong>Splenomegaly</strong></td>
<td>Reduce spleen volume by 30%-50% from baseline</td>
<td>N/A</td>
<td>Reduce spleen volume by 50%-60% from baseline</td>
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<td></td>
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<td>Maintain spleen volume at &lt;2 to 8x normal</td>
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<tr>
<td><strong>Hepatomegaly</strong></td>
<td>N/A</td>
<td>Reduce liver volume by 20%-30% from baseline</td>
<td>Reduce liver volume by 30%-50% from baseline</td>
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<tr>
<td></td>
<td></td>
<td></td>
<td>Maintain liver volume at 1-1.5x normal</td>
</tr>
<tr>
<td><strong>Skeletal</strong></td>
<td>N/A</td>
<td>Lessen or eliminate bone pain</td>
<td>Adult patients: increase trabecular BMD</td>
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<td></td>
<td></td>
<td>Pediatric patients: increase cortical and trabecular BMD</td>
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</tr>
</tbody>
</table>

N/A: not applicable
BMD: bone mineral density
*Baseline platelet count < 120,000/µL and > 60,000/µL
^Baseline platelet count <60,000/µL

**Exclusions**

1. **Type 2 or Type 3 Gaucher’s disease.**
2. **Tay-Sachs disease.**
3. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.
References


APPROVAL:

ENDORSED BY: Pharmacy & Therapeutics Committee

APPROVED BY: Date:

Original Date: 5/15/2013