Elelyso (taliglucerase)

**Line(s) of Business:**
- HMO; PPO; QUEST Integration
- Medicare Advantage

**Original Effective Date:**
10/01/2015

**Current Effective Date:**
03/01/2018

**POLICY**

**A. INDICATIONS**
The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no contraindications or exclusions to the prescribed therapy.

**FDA-Approved Indication**
- Elelyso is indicated for long-term enzyme replacement therapy (ERT) for adult and pediatric patients with a confirmed diagnosis of type 1 Gaucher disease

**B. REQUIRED DOCUMENTATION**
The following information is necessary to initiate the prior authorization review:
- Enzyme assay or DNA testing confirming diagnosis of Gaucher disease

**C. EXCLUSIONS**
- Patients with type 2 Gaucher disease
- Concomitant therapy with Zavesca or Cerdelga

**D. CRITERIA FOR APPROVAL**

**Type 1 Gaucher Disease** (See Appendix A)
Authorization of 12 months may be granted to member with a diagnosis of Type 1 Gaucher disease which was confirmed by an enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by DNA testing who exhibit one or more documented complications of Type 1 Gaucher disease (See Appendix B).

**E. CONTINUATION OF THERAPY**

1. **No previous authorization/precertification:**
   - All members (including new members and members currently receiving treatment without prior authorization) must meet criteria for initial approval in section D.

2. **Reauthorization:**
   - Authorization of 12 months may be granted to members who are prescribed Elelyso for continuation of therapy if Elelyso was previously approved by HMSA/CVS and there is evidence of benefit from therapy.
F. DOSING AND ADMINISTRATION
Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

G. ADMINISTRATIVE GUIDELINES
Precertification is required. Please refer to the HMSA medical policy web site for the fax form.

H. APPENDICES
APPENDIX A. Diagnosis of Gaucher Disease
The variants (types 1, 2, and 3) are distinguished based on several factors, including neuronopathic involvement, molecular testing (specific mutations), age of onset, family history, ethnicity, and other clinical features (e.g., hepatosplenomegaly, bone involvement).

APPENDIX B. Complications of Gaucher Disease
Type 1 Gaucher Disease:
- Anemia
- Thrombocytopenia
- Bone disease (e.g., bone pain, bone crises, fractures, osteonecrosis, subchondral joint collapse)
- Hepatomegaly or splenomegaly

I. IMPORTANT REMINDER
The purpose of this Medical Policy is to provide a guide to coverage. This Medical Policy is not intended to dictate to providers how to practice medicine. Nothing in this Medical Policy is intended to discourage or prohibit providing other medical advice or treatment deemed appropriate by the treating physician.

Benefit determinations are subject to applicable member contract language. To the extent there are any conflicts between these guidelines and the contract language, the contract language will control.

This Medical Policy has been developed through consideration of the medical necessity criteria under Hawaii’s Patients’ Bill of Rights and Responsibilities Act (Hawaii Revised Statutes §432E-1.4), generally accepted standards of medical practice and review of medical literature and government approval status. HMSA has determined that services not covered under this Medical Policy will not be medically necessary under Hawaii law in most cases. If a treating physician disagrees with HMSA’s determination as to medical necessity in a given case, the physician may request that CVS/caremark reconsider the application of the medical necessity criteria to the case at issue in light of any supporting documentation.

J. REFERENCES
<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>10/01/2015</td>
<td>Original effective date</td>
</tr>
<tr>
<td>08/2016</td>
<td>Annual review</td>
</tr>
<tr>
<td>07/2017</td>
<td>Annual review</td>
</tr>
<tr>
<td>03/01/2018</td>
<td>Revision effective date</td>
</tr>
</tbody>
</table>