

Gaucher Disease

Goal(s):

- Ensure medically appropriate use of drugs for Gaucher disease

Length of Authorization:

- Up to 12 months

Requires PA:

- Drugs for Gaucher disease (pharmacy and physician administered claims)

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. FDA-Approved Minimum Ages

Drug	Age
Eliglustat	18
Imiglucerase	2
Miglustat	18
Taliglucerase alfa	4
Velaglucerase alfa	4

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the request for continuation of therapy previously approved by FFS?	Yes: Go to Renewal Criteria	No: Go to #3
3. Is the request from a provider experienced in the treatment of Gaucher disease?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness
4. Is the request for treatment of Type 1 Gaucher Disease? Note: Type 1 disease is characterized predominately by bone involvement without CNS symptoms.	Yes: Go to #6	No: Go to #5

Approval Criteria

<p>5. Is the request for treatment of Type 3 Gaucher Disease?</p> <p>Note: Drugs are not FDA-approved for Type 2 or 3 Gaucher disease. Type 3 disease is characterized by both bone involvement and CNS symptoms.</p>	<p>Yes: Refer requests to the medical director for review. Provide relevant chart notes and literature documenting medical necessity.</p>	<p>No: Pass to RPh. Deny; medical appropriateness</p>
<p>6. Is the request for an FDA-approved age in Table 1?</p>	<p>Yes: Go to #7</p>	<p>No: Pass to RPh. Deny; medical appropriateness</p>
<p>7. Does the patient have current symptoms characteristic of bone involvement such as:</p> <ol style="list-style-type: none"> Low platelet count Low hemoglobin and hematocrit levels Radiologic bone disease, T-score less than -2.5 or bone pain Delayed growth in children (<10th percentile for age) OR Splenomegaly or hepatomegaly? 	<p>Yes: Go to #8</p> <p>Document baseline labs and symptoms</p>	<p>No: Pass to RPh. Deny; medical appropriateness</p>
<p>8. Is the request for combination treatment with more than one targeted therapy for Gaucher disease?</p>	<p>Yes: Pass to RPh. Deny; medical appropriateness</p>	<p>No: Go to #9</p>
<p>9. Is the request for enzyme replacement therapy?</p>	<p>Yes: Go to #10</p>	<p>No: Go to #11</p>
<p>10. Is the request for a non-preferred product and will the prescriber consider a change to a preferred product?</p> <p>Message: Preferred products are evidence-based reviewed for comparative effectiveness and safety by the Oregon Pharmacy & Therapeutics Committee.</p>	<p>Yes: Inform prescriber of covered alternatives in class. Approve preferred therapy for up to 6 months.</p>	<p>No: Approve for up to 6 months</p>
<p>11. Does the patient have a documented contraindication, intolerance, inadequate response, or inability to access or adhere to enzyme replacement therapy?</p>	<p>Yes: Go to #12</p>	<p>No: Pass to RPh. Deny; medical appropriateness</p>
<p>12. Is the request for eliglustat?</p>	<p>Yes: Go to #13</p>	<p>No: Approve for up to 6 months</p>

Approval Criteria		
13. Does the patient have cardiac disease, long-QT syndrome, or is currently taking a Class IA or Class III antiarrhythmic medication?	Yes: Pass to RPh. Deny; medical appropriateness	No: Go to #14
14. Does the patient have moderate to severe hepatic impairment?	Yes: Pass to RPh. Deny; medical appropriateness	No: Go to #15
15. Does testing for CYP2D6 metabolizer status indicate extensive, intermediate or poor CYP2D6 metabolism?	Yes: Go to #16	No: Pass to RPh. Deny; medical appropriateness
16. Is the dose consistent with FDA labeling based on CYP2D6 metabolism and use of concomitant CYP inhibitors (see FDA labeling for full details)?	Yes: Approve for up to 6 months	No: Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there documentation based on chart notes that the patient experienced a significant adverse reaction related to treatment for Gaucher disease?	Yes: Go to #2	No: Go to #3
2. Has the adverse event been reported to the FDA Adverse Event Reporting System?	Yes: Go to #3 Document provider attestation	No: Pass to RPh. Deny; medical appropriateness
3. Has the patient been adherent to current therapy?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness
4. Is there objective documentation of benefit based on improved labs or patient symptoms?	Yes: Approve for up to 12 months Document labs and patient symptoms	No: Pass to RPh. Deny; medical appropriateness