

# Pompe Disease

## Goal(s):

- Ensure medically appropriate use of approved agents for the treatment of Pompe disease

## Length of Authorization:

- Up to 12 months

## Requires PA:

- Alglucosidase alfa (pharmacy and physician administered claims)
- Avalglucosidase alfa (pharmacy and physician administered claims)

## Covered Alternatives:

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

**Table 1: FDA-approved Dosage and Administration**

Agent	Indication	Age Minimum	Dosing Regimen
Alglucosidase alfa	Early Onset Pompe Disease (EOPD) Late Onset Pompe Disease (LOPD)	None	20 mg/kg IV once every 2 weeks
Avalglucosidase alfa	Late Onset Pompe Disease (LOPD)	≥ 1 year	< 30 kg: 40 mg/kg IV once every 2 weeks ≥ 30 kg: 20 mg/kg IV once every 2 weeks

## Approval Criteria

1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the requested agent for an approved indication and dosed appropriately based on age and weight taken within the past month? (see Table 1)	<b>Yes:</b> Document patient weight and go to #3.  Weight: _____	<b>No:</b> Pass to RPh. Deny; medical appropriateness.
3. Is there documentation that the patient is switching enzyme replacement therapy (ERT) agents due to lack of benefit with prior therapy?	<b>Yes:</b> Pass to RPh. Deny; medical appropriateness	<b>No:</b> Go to #4
4. Is there documentation that the provider has assessed the patient for signs or susceptibility to the following? <ul style="list-style-type: none"> <li>• Fluid volume overload</li> <li>• Acute underlying respiratory illness</li> <li>• Compromised cardiac or respiratory function necessitating fluid restriction</li> </ul>	<b>Yes:</b> Go to #5	<b>No:</b> Pass to RPh. Deny; medical appropriateness
5. Is the request for continuation of therapy previously approved by FFS?	<b>Yes:</b> Go to <b>Renewal Criteria</b>	<b>No:</b> Go to #6

## Approval Criteria

6. Is the treatment for the diagnosis of Pompe disease confirmed by either DNA testing or enzyme assay (e.g. acid alpha-glucosidase activity test)?	<b>Yes:</b> Go to #7	<b>No:</b> Pass to RPh. Deny; medical appropriateness
7. Is this request from a metabolic specialist, biochemical geneticist, or has provider documented experience in the treatment of Pompe disease?	<b>Yes:</b> Go to #8	<b>No:</b> Pass to RPh. Deny; medical appropriateness
8. Is the request for treatment of late-onset Pompe disease (LOPD)?	<b>Yes:</b> Go to #12	<b>No:</b> Go to #9
9. Has the provider documented a baseline value for ALL the following assessments? <ul style="list-style-type: none"> <li>• Muscle weakness/Motor function? (e.g. AIMS, PDMS-2, Pompe PEDI, etc)</li> <li>• Respiratory status (e.g. FEV, FVC, or other age-appropriate test of pulmonary function)?</li> <li>• Cardiac imaging (e.g. chest x-ray, echocardiography)?</li> <li>• CRIM status?</li> </ul>	<b>Yes:</b> Document baseline results and go to #10	<b>No:</b> Pass to RPh. Deny; medical appropriateness
10. Is the patient CRIM-negative?	<b>Yes:</b> Go to #11	<b>No:</b> Approve for 3 months  If approved, a referral will be made to case management by the OHA.
11. Is there documentation that concomitant immune tolerance induction (ITI) therapy will be initiated with enzyme replacement therapy (ERT)?	<b>Yes:</b> Approve for 3 months	<b>No:</b> Pass to RPh. Deny; medical appropriateness
12. Is the patient 5 years of age or older?	<b>Yes:</b> Go to #13	<b>No:</b> Go to #14

## Approval Criteria

<p>13. Is there a baseline documentation for both of the following?</p> <ul style="list-style-type: none"> <li>• Pulmonary function test (PFT) with spirometry including baseline percent predicted forced vital capacity (FVC)</li> <li>• Demonstration of completed 6-minute walk test (6MWT)</li> </ul> <p>-OR-</p> <p>Muscle weakness in the lower extremities?</p>	<p><b>Yes:</b> Approve for 6 months</p> <p>Document baseline results.</p> <p>If approved, a referral will be made to case management by the OHA.</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness</p>
<p>14. Has the provider documented a baseline value for both of the following assessments:</p> <ul style="list-style-type: none"> <li>• Muscle weakness/Motor function? (e.g. AIMS, PDMS-2, Pompe PEDI, etc)</li> <li>• Respiratory status (e.g. FEV, FVC, or other age-appropriate test of pulmonary function)?</li> </ul>	<p><b>Yes:</b> Approve for 3 months</p> <p>Document baseline results.</p> <p>If approved, a referral will be made to case management by OHA.</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness</p>

## Renewal Criteria

<p>1. Is there documented evidence of adherence and tolerance to the approved infusion therapy regimen through claims history and/or provider assessment?</p>	<p><b>Yes:</b> Go to #2</p>	<p><b>No:</b> Pass to RPh, Deny; medical appropriateness</p>
<p>2. Is this a request for <b>al</b>glucosidase alfa?</p>	<p><b>Yes:</b> Go to #3</p>	<p><b>No:</b> Go to #5</p>
<p>3. Is this the <u>first</u> renewal for <b>al</b>glucosidase alfa?</p>	<p><b>Yes:</b> Go to #4</p>	<p><b>No:</b> Go to #5</p>
<p>4. Is there documentation that the patient has recently been tested* for IgG antibody formation?</p> <p><i>* Patients should be monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter per manufacturer labeling.</i></p>	<p><b>Yes:</b> Go to #5</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness</p>
<p>5. Compared to baseline measurements, is there documented evidence of improvement or stabilization in muscle, motor, and/or respiratory function?</p>	<p><b>Yes:</b> Go to #6</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness</p>

## Renewal Criteria

6. Is patient under 5 years old?	<b>Yes:</b> Approve for 3 months	<b>No:</b> Go to #7
7. Has the patient received the requested therapy for at least 6 months?	<b>Yes:</b> Approve for 12 months	<b>No:</b> Approve for 3 months

*P&T/DUR Review: 2/22 (DE); 4/21 (DE)  
Implementation: 4/1/22; 5/1/21*