

Pompe Disease Agents

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 provider administered claims)
- Avalglucosidase alfa (pharmacy and provider administered claims)
- Cipaglucosidase alfa (pharmacy and provider administered claims)
- Miglustat (OPFOLDA) (pharmacy and provider administered claims)

Covered Populations:

- Opfolda (miglustat): FFS and CCO patients beginning 1/1/26
- All others: FFS only

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 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 |
| Cipaglucosidase alfa* | Late Onset Pompe Disease (LOPD) | 18 years or older | <40 kg: <u>not indicated</u> ≥40 kg: 20 mg/kg IV once every 2 weeks -plus- Miglustat 260 mg orally (≥ 50 kg) -or- 195 mg orally (≥40 kg to <50 kg) (administer 1 hour before cipaglucosidase infusion) |

*must be administered with miglustat according to FDA labeled dosing parameters

Approval Criteria

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| 1. What diagnosis is being treated? | Record ICD10 code. |
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Approval Criteria

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| <p>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)</p> | <p>Yes: Document patient weight and go to #3. Weight: _____</p> | <p>No: Pass to RPh. Deny; medical appropriateness.</p> |
| <p>3. Is there documentation that the provider has assessed the patient for signs or susceptibility to the following?</p> <ul style="list-style-type: none"> • Fluid volume overload • Acute underlying respiratory illness • Compromised cardiac or respiratory function necessitating fluid restriction | <p>Yes: Go to #4</p> | <p>No: Pass to RPh. Deny; medical appropriateness</p> |
| <p>4. Is the request for continuation of therapy previously approved by FFS?</p> | <p>Yes: Go to Renewal Criteria</p> | <p>No: Go to #5</p> |
| <p>5. Is the treatment for the diagnosis of Pompe disease confirmed by either DNA testing or enzyme assay (e.g. acid alpha-glucosidase activity test)?</p> | <p>Yes: Go to #6</p> | <p>No: Pass to RPh. Deny; medical appropriateness</p> |
| <p>6. Is this request from a metabolic specialist, biochemical geneticist, or has provider documented experience in the treatment of Pompe disease?</p> | <p>Yes: Go to #7</p> | <p>No: Pass to RPh. Deny; medical appropriateness</p> |
| <p>7. Is the request for treatment of late-onset Pompe disease (LOPD)?</p> | <p>Yes: Go to #11</p> | <p>No: Go to #8</p> |
| <p>8. Has the provider documented a baseline value for ALL the following assessments?</p> <ul style="list-style-type: none"> • Muscle weakness/Motor function? (e.g. AIMS, PDMS-2, Pompe PEDI, etc) • Respiratory status (e.g. FEV, FVC, or other age-appropriate test of pulmonary function)? • Cardiac imaging (e.g. chest x-ray, echocardiography)? • CRIM status? | <p>Yes: Document baseline results and go to #9</p> | <p>No: Pass to RPh. Deny; medical appropriateness</p> |
| <p>9. Is the patient CRIM-negative?</p> | <p>Yes: Go to #10</p> | <p>No: Approve for 3 months</p> <p>If approved, a referral will be made to case management by the OHA.</p> |

Approval Criteria

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| 10. Is there documentation that concomitant immune tolerance induction (ITI) therapy will be initiated with enzyme replacement therapy (ERT)? | Yes: Approve for 3 months | No: Pass to RPh. Deny; medical appropriateness |
| 11. Is the request for cipaglucosidase alfa or miglustat for Pompe Disease? | Yes: Go to #12 | No: Go to #13 |
| 12. Does the provider plan to order combination treatment as outlined in Table 1? | Yes: Approve miglustat as combination treatment. Go to #14 | No: Pass to RPh. Deny; medical appropriateness |
| 13. Is the patient 5 years of age or older? | Yes: Go to #14 | No: Go to #15 |
| 14. Is there a baseline documentation for both of the following? <ul style="list-style-type: none"> Pulmonary function test (PFT) with spirometry including baseline percent predicted forced vital capacity (FVC) Demonstration of completed 6-minute walk test (6MWT) -OR- Muscle weakness in the lower extremities? | Yes: Approve for 6 months Document baseline results. If approved, a referral will be made to case management by the OHA. | No: Pass to RPh. Deny; medical appropriateness |
| 15. Has the provider documented a baseline value for both of the following assessments: <ul style="list-style-type: none"> Muscle weakness/Motor function? (e.g. AIMS, PDMS-2, Pompe PEDI, etc) Respiratory status (e.g. FEV, FVC, or other age-appropriate test of pulmonary function)? | Yes: Approve for 3 months Document baseline results. If approved, a referral will be made to case management by OHA. | No: Pass to RPh. Deny; medical appropriateness |

Renewal Criteria

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| 1. Is there documented evidence of adherence and tolerance to the approved infusion therapy regimen through claims history and/or provider assessment? | Yes: Go to #2 | No: Pass to RPh, Deny; medical appropriateness |
| 2. Is this a request for al glucosidase alfa? | Yes: Go to #3 | No: Go to #5 |

| Renewal Criteria | | |
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| 3. Is this the <u>first</u> renewal for al glucosidase alfa? | Yes: Go to #4 | No: Go to #5 |
| 4. Is there documentation that the patient has recently been tested* for IgG antibody formation? <i>* Patients should be monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter per manufacturer labeling.</i> | Yes: Go to #5 | No: Pass to RPh. Deny; medical appropriateness |
| 5. Compared to baseline measurements, is there documented evidence of improvement or stabilization in muscle, motor, and/or respiratory function? | Yes: Go to #6 | No: Pass to RPh. Deny; medical appropriateness |
| 6. Is patient under 5 years old? Note: Approve therapy per Table 1 (including miglustat if appropriate) | Yes: Approve for 3 months | No: Go to #7 |
| 7. Has the patient received the requested therapy for at least 6 months? Note: Approve therapy per Table 1 (including miglustat if appropriate) | Yes: Approve for 12 months | No: Approve for 3 months |

P&T/DUR Review: 12/25; 6/24 (DE); 2/22; 4/21
Implementation: 1/1/26; 7/1/24; 4/1/22; 5/1/21