Miglustat 100 mg capsule (Zavesca, Yargesa, generic)

Goal(s):

- Ensure medically appropriate use of miglustat for Niemann-Pick disease Type C and Gaucher disease.
- Incorporate 2-step review process for drugs on the high-cost drug carve-out list.

Length of Authorization:

- Up to 12 months

Requires PA:

- Miglustat (ZAVESCA, YARGESA, generic 100 mg capsules). For OPFOLDA see Pompe disease criteria.

Covered Populations: FFS and CCO patients beginning 1/1/26

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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the request for a patient with a prior FFS approval for the requested drug?	**Yes**: Go to **Renewal Criteria**	**No:** Go to #3
3. Is the drug prescribed by made or in consultation with an expert in metabolic or genetic disease or experienced in treating Niemann-Pick disease type C or Gaucher disease?	**Yes**: Go to #4	**No:** Pass to RPh. Deny; medical appropriateness
4. Is the drug being prescribed for Niemann-Pick disease type C?	**Yes**: Go to #5	**No:** Go to #7
5. Is that patient at least 2 years old?	**Yes**: Go to #10	**No:** Pass to RPh. Deny; medical appropriateness
6. Is the drug being prescribed for mild to moderate type 1 Gaucher disease in an adult (18 years or older)?	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
7. Does the patient have current symptoms characteristic of bone involvement such as: a. Low platelet count b. Low hemoglobin and hematocrit levels c. Radiologic bone disease, T-score less than -2.5 or bone pain d. Delayed growth in children (<10th percentile for age) OR e. Splenomegaly or hepatomegaly?	**Yes:** Go to #8 Document baseline labs and symptoms	**No:** Pass to RPh. Deny; medical appropriateness
8. Is the request for combination treatment with more than one targeted therapy for Gaucher disease?	**Yes:** Pass to RPh. Deny; medical appropriateness	**No:** Go to #9
9. Does the patient have a documented contraindication, intolerance, inadequate response, or inability to access or adhere to enzyme replacement therapy?	**Yes:** Go to #10	**No:** Pass to RPh. Deny; medical appropriateness
10. Is the request for a non-preferred product and will the prescriber consider a change to a preferred product?	**Yes**: Inform prescriber of covered alternatives in class. Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals for preferred therapy cover up to 6 months.	**No:** Go to #11
11. Does the patient have either: • A documented failure (either therapeutic or due to adverse events) with the preferred version of this product OR • Documentation of inability to access product to due to national/regional shortage?	**Yes**: Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover up to 6 months.	**No:** Pass to RPh. Deny; cost effectiveness.

Renewal Criteria		
1. Is there documentation based on chart notes that the patient experienced a significant adverse reaction related to miglustat?	**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:** Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover up to 12 months Document labs and patient symptoms	**No:** Pass to RPh. Deny; medical appropriateness

P&T/DUR Review: 12/25
Implementation: 1/1/26