

## Niemann-Pick Disease Type C Medications

### Goal(s):

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

### Length of Authorization:

- Up to 12 months

### Requires PA:

- Miplyffa™ (arimoclomol)
- Aqneursa™ (levacetylleucine)

**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](http://www.orpdl.org)
- Searchable site for Oregon FFS Drug Class listed at [www.orpdl.org/drugs/](http://www.orpdl.org/drugs/)

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. 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 #3
3. Is the request for arimoclomol or levacetylleucine in a patient already taking the other agent (i.e., combination therapy without documentation of planned therapeutic switch)?	<b>Yes:</b> Pass to RPh. Deny; medical appropriateness	<b>No:</b> Go to #4
4. Has the diagnosis of Niemann-Pick disease type C been confirmed by genetic testing or a filipin test?	<b>Yes:</b> Go to #5	<b>No:</b> Pass to RPh. Deny; medical appropriateness
5. Is the request being made by or in consultation with an expert in metabolic or genetic disease or experienced in treating Niemann-Pick disease type C?	<b>Yes:</b> Go to #6	<b>No:</b> Pass to RPh. Deny; medical appropriateness
6. Is there documentation that the patient has developed at least one neurological manifestation of disease?	<b>Yes:</b> Go to #7	<b>No:</b> Pass to RPh. Deny; medical appropriateness

## Approval Criteria

<p>7. Has baseline severity been documented using NPCCSS, SARA, or some other appropriate tool for assessing Niemann-Pick disease type C?</p> <p>Examples:</p> <ul style="list-style-type: none"> <li>Niemann-Pick Disease Type C Clinical Severity Scale (NPCCSS)</li> <li>Scale for the Assessment and Rating of Ataxia (SARA)</li> </ul>	<p><b>Yes:</b> Go to #8 Record tool and value:_____</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness</p>
<p>8. Is the patient of childbearing potential?</p>	<p><b>Yes:</b> Go to #9</p>	<p><b>No:</b> Go to #11</p>
<p>9. Is the patient pregnant or actively trying to conceive?</p>	<p><b>Yes:</b> Pass to RPh. Deny; medical appropriateness.</p>	<p><b>No:</b> Go to #10</p>
<p>10. Is there documentation that the provider and patient have discussed the teratogenic risks of the drug if the patient were to become pregnant?</p>	<p><b>Yes:</b> Go to #11</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness.</p>
<p>11. Has the provider documented patient-specific goals for this therapy over the next 6 to 12 months?</p> <p>Note: Goals of therapy can vary from intent to cure, disease burden reduction, disease stabilization and control of symptoms.</p>	<p><b>Yes:</b> Go to #12</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness.</p>
<p>12. Has the provider defined objective criteria to evaluate unsuccessful treatment or lack of response based on individual patient goals and current symptoms (i.e., when would the provider consider discontinuing therapy)?</p> <p>To qualify for treatment coverage, the patient and provider must have a documented discussion about when risks of the therapy outweigh the benefits and a knowledge of the realistic expectations of treatment efficacy. Care must always take place in the context of the patient's support systems, overall health, and core values.</p>	<p><b>Yes:</b> Go to #13</p>	<p><b>No:</b> Pass to RPh. Deny; medical appropriateness.</p>
<p>13. Is the request for arimoclomol in a patient who is at least 2 years old and ambulatory (with or without assistance)?</p>	<p><b>Yes:</b> Go to #14</p>	<p><b>No:</b> Go to #15</p>

Approval Criteria		
14. Is patient taking concomitant miglustat or starting miglustat therapy with arimoclomol initiation?	<b>Yes:</b> Pass to RPh. Pend; Refer to DMAP for secondary review.  Duration: Approvals cover 12 months	<b>No:</b> Pass to RPh. Deny; medical appropriateness  Arimoclomol is only approved for use in combination with miglustat.
15. Is the request for levacetylleucine in a patient weighing at least 15 kg?	<b>Yes:</b> Pass to RPh. Pend; Refer to DMAP for secondary review.  Duration: Approvals cover 6 months	<b>No:</b> Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Has the patient been adherent to current therapy?	<b>Yes:</b> Go to #2	<b>No:</b> Pass to RPh. Deny; medical appropriateness
2. Is there documentation that the patient's goals of therapy established prior to treatment have been met?	<b>Yes:</b> Pass to RPh. Pend; Refer to DMAP for secondary review.  Duration: Approvals cover 12 months	<b>No:</b> Go to #3
3. Is there documentation that pre-established criteria for unsuccessful treatment or lack of response have been met?	<b>Yes:</b> Pass to RPh. Deny; medical appropriateness	<b>No:</b> Go to #4
4. Have the patient and provider had a documented discussion about when benefits of the therapy outweigh the potential risks?	<b>Yes:</b> Pass to RPh. Pend; Refer to DMAP for secondary review.  Duration: Approvals cover 12 months	<b>No:</b> Pass to RPh. Deny; medical appropriateness