

## Orphan Drugs

### Goal(s):

- To support medically appropriate use of orphan drugs (as designated by the FDA) which are indicated for rare conditions
- To limit off-label use of orphan drugs

### Length of Authorization:

- Up to 6 months

### Requires PA:

- See Table 1 (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. Included orphan drugs

<a href="#">ADAMTS13, recombinant-krhn (ADZYNMA)</a>
<a href="#">Allogeneic processed thymus tissue-agdc (RETHYMIC)</a>
<a href="#">Apelisib (VIJOICE)</a>
<a href="#">Avacopan (TAVNEOS)</a>
<a href="#">Belumosudil (REZUROCK)</a>
<a href="#">Beremagene geperpavec-svdt (VYJUVEK)</a>
<a href="#">Birch triterpenes (FILSUEVZ)</a>
<a href="#">Burosumab-twza (CRYSVITA)</a>
<a href="#">Cerliponase alfa (BRINEURA)</a>
<a href="#">Elapegademase-lvr (REVCovi)</a>
<a href="#">Elivaldogene autotemcel (SKYSONA)</a>
<a href="#">Fosdenopterin (NULIBRY)</a>
<a href="#">Givosiran (GIVLAARI)</a>
<a href="#">Leniolisib (JOENJA)</a>
<a href="#">Levoketoconazole (RECORLEV)</a>
<a href="#">Lonafarnib (ZOKINVY)</a>
<a href="#">Lumasiran (OXLUMO)</a>
<a href="#">Luspatercept (REBLOZYL)</a>
<a href="#">Maralixibat (LIVMARLI)</a>
<a href="#">Mitapivat (PYRUKYND)</a>
<a href="#">Nedosiran (RIVFLOZA)</a>
<a href="#">Odevixibat (BYLVAY)</a>
<a href="#">Olipudase alfa-rpcp (XENPOZYME)</a>
<a href="#">Palovarotene (SOHONOS)</a>
<a href="#">Plasminogen, human-tvmh (RYPLAZIM)</a>
<a href="#">pozelimab-bbfq (VEOPOZ)</a>
<a href="#">Sodium thiosulfate (PEDMARK)</a>
<a href="#">Sutimlimab-jome (ENJAYMO)</a>
<a href="#">Trientine tetrahydrochloride (CUVRIOR)</a>
<a href="#">Velmanase alfa-tycv (LAMZEDE)</a>

### Approval Criteria

1. What diagnosis is being treated?

Record ICD10 code.

## Approval Criteria

2. Is the diagnosis funded by OHP?	<b>Yes:</b> Go to #4	<b>No:</b> For current age $\geq$ 21 years: Pass to RPh. Deny; not funded by the OHP  For current age < 21 years: Go to #3
3. Is there documentation that the condition is of sufficient severity that it impacts the patient's health (e.g., quality of life, function, growth, development, ability to participate in school, perform activities of daily living, etc)?	<b>Yes:</b> Go to #4	<b>No:</b> Pass to RPh. Deny; medical necessity.
4. Is the request for a drug FDA-approved for the indication, age, and dose as defined in the FDA label (see links in <b>Table 1</b> )?  Note: This includes all information required in the FDA-approved indication, including but not limited to, the following as applicable: diagnosis, disease severity, biomarkers, place in therapy, and use as monotherapy or combination therapy.	<b>Yes:</b> Go to #5	<b>No:</b> Pass to RPh. Deny; medical appropriateness.
5. Is the request for continuation of therapy in a patient previously approved by FFS?	<b>Yes:</b> Go to <b>Renewal Criteria</b>	<b>No:</b> Go to #6
6. Is baseline monitoring recommended for efficacy or safety (e.g., labs, baseline symptoms, etc) AND has the provider submitted documentation of recommended baseline and ongoing monitoring parameters described in the FDA label?*	<b>Yes:</b> Go to #7	<b>No:</b> Pass to RPh. Deny; medical appropriateness.
*FDA pages for drugs and biologics: <a href="https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm">https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm</a> <a href="https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products">https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products</a>		
7. Is this medication therapy being prescribed by, or in consultation with, an appropriate medical specialist?	<b>Yes:</b> Go to #8	<b>No:</b> Pass to RPh. Deny; medical appropriateness.

## Approval Criteria

8. Have other therapies been tried and failed?	<b>Yes:</b> Approve for up to 3 months (or length of treatment) whichever is less  Document therapies which have been previously tried	<b>No:</b> Approve for up to 3 months (or length of treatment) whichever is less  Document provider rationale for use as a first-line therapy
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## Renewal Criteria

1. Is there documentation based on chart notes that the patient experienced a significant adverse reaction related to treatment?	<b>Yes:</b> Go to #2	<b>No:</b> Go to #3
2. Has the adverse event been reported to the FDA Adverse Event Reporting System?	<b>Yes:</b> Go to #3  Document provider attestation	<b>No:</b> Pass to RPh. Deny; medical appropriateness
3. Is baseline efficacy monitoring available?	<b>Yes:</b> Go to #4	<b>No:</b> Go to #5
4. Is there objective documentation of improvement from baseline OR for chronic, progressive conditions, is there documentation of disease stabilization or lack of decline compared to the natural disease progression?	<b>Yes:</b> Approve for up to 6 months  Document benefit	<b>No:</b> Pass to RPh. Deny; medical appropriateness
5. Is there documentation of benefit from the therapy as assessed by the prescribing provider (e.g., improvement in symptoms or quality of life, or for progressive conditions, a lack of decline compared to the natural disease progression)?	<b>Yes:</b> Approve for up to 6 months  Document benefit and provider attestation	<b>No:</b> Pass to RPh. Deny; medical appropriateness

P&T/DUR Review: 12/23; 10/23; 6/23; 2/23; 12/22; 6/22; 4/22; 12/21; 10/21; 6/21; 2/21; 8/20; 6/20; 2/20

Implementation: 1/1/24; 11/1/23; 7/1/23; 4/1/23; 1/1/23; 7/1/22; 5/1/22; 1/1/2022; 7/1/2021; 3/1/21; 11/1/20; 9/1/20; 7/1/20