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## Prior Authorization Criteria Update: Orphan Drug

### Purpose of the Update:

This update identifies orphan drugs recently approved by the FDA to add to the orphan drug policy (**Table 1**).

**Table 1.** Updated orphan drugs

<u>Generic</u>	<u>Brand</u>	<u>High Cost Drug Carve-out</u>
doxycitine and doxribtimine	KYGEVVI	Yes (5/1/26)
linerixibat	LYNAVOY	No
lunsotogene parvec	OTARMENI	No
marnetegragene autotemcel	KRESLADI	Yes (5/1/26)
tividenofusp alfa	AVLAYAH	Yes (5/1/26)

### Recommendation:

- Modify PA criteria to include new orphan drugs and carve-out status.
- Update renewal criteria to accommodate longer approvals for chronic therapy.

## Appendix 1. Proposed Prior Authorization Criteria

### 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 provider 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

<b>Drug</b>	<b>Covered Population</b>
<a href="#">ADAMTS13, recombinant-krhn (ADZYNMA)</a>	FFS
<a href="#">Aficamten (MYQORZO)</a>	FFS
<a href="#">Allogeneic processed thymus tissue-agdc (RETHYMIC)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Apelisib (VIJOICE)</a>	FFS
<a href="#">Asfotase alfa (STRENSIQ)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Atidarsagene autotemcel (LENMELDY)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Avacopan (TAVNEOS)</a>	FFS
<a href="#">Axatilimab-csfr (NIKTIMVO)</a>	FFS
<a href="#">Belumosudil (REZUROCK)</a>	FFS
<a href="#">Burosumab-twza (CRYSVITA)</a>	FFS
<a href="#">Cerliponase alfa (BRINEURA)</a>	FFS
<a href="#">Chenodiol (CTEXLI)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Crinecefont (CRENESSITY)</a>	FFS
<a href="#">Crovalimab-akkz (PIASKY)</a>	FFS
<a href="#">Danicopan (VOYDEYA)</a>	FFS
<a href="#">doxycitine and doxribtimine (KYGEVVI)</a>	FFS and CCO populations beginning 5/1/26
<a href="#">Eculizumab (SOLIRIS)</a>	FFS
<a href="#">Eculizumab-aagh (EPYSQLI)</a>	FFS
<a href="#">Eculizumab-aeeb (BKEMV)</a>	FFS

<a href="#">Eladocagene exuparvovec-tneq (KEBILDI)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Elafibranor (IQIRVO)</a>	FFS
<a href="#">Elapegademase-lvr (REVCOVI)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Elivaldogene autotemcel (SKYSONA)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Elosulfase alfa (VIMIZIM)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Fosdenopterin (NULIBRY)</a>	FFS
<a href="#">Galsulfase (NAGLAZYME)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Givosiran (GIVLAARI)</a>	FFS
<a href="#">Idursulfase (ELAPRASE)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Inebilizumab-cdon (UPLIZNA)</a>	FFS
<a href="#">Iptacopan (FABHALTA)</a>	FFS
<a href="#">Leniolisib (JOENJA)</a>	FFS <u>and CCO populations beginning 5/1/26</u>
<a href="#">Levoketoconazole (RECORLEV)</a>	FFS
<a href="#">linerixibat (LYNAVOY)</a>	<u>FFS</u>
<a href="#">Lonafarnib (ZOKINVY)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Lumasiran (OXLUMO)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">lunsotogene parvec (OTARMENI)</a>	<u>FFS</u>
<a href="#">Luspatercept (REBLOZYL)</a>	FFS
<a href="#">Maralixibat (LIVMARLI)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">marnetegragene autotemcel (KRESLADI)</a>	<u>FFS and CCO populations beginning 5/1/26</u>
<a href="#">Mavacamten (CAMZYOS)</a>	FFS
<a href="#">Mavorixafor (XOLREMDI)</a>	FFS
<a href="#">Mirdametinib (GOMEKLI)</a>	FFS
<a href="#">Mitapivat (PYRUKYND)</a>	FFS
<a href="#">Nedosiran (RIVFLOZA)</a>	FFS
<a href="#">Nerandomilast (JASCAYD)</a>	FFS
<a href="#">Nipocalimab-aahu (IMAAVY)</a>	FFS
<a href="#">Odevixibat (BYLVAY)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Olipudase alfa-rpcp (XENPOZYME)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Palopegteriparatide (YORVIPATH)</a>	FFS
<a href="#">Paltusotine (PALSONIFY)</a>	FFS
<a href="#">Pegcetacoplan (EMPAVELI)</a>	FFS
<a href="#">Plasminogen, human-tvmh (RYPLAZIM)</a>	FFS
<a href="#">Pozelimab-bbfg (VEOPOZ)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Ravulizumab-cwvz (ULTOMIRIS)</a>	FFS
<a href="#">Remestemcel-L-rknd (RYONCIL)</a>	FFS
<a href="#">Rezafungin (REZZAYO)</a>	FFS
<a href="#">Rozanolixizumab-noli (RYSTIGGO)</a>	FFS

<a href="#">Satralizumab-mwge (ENSPRYNG)</a>	FFS
<a href="#">Seladelpar (LIVDELZI)</a>	FFS
<a href="#">Sodium thiosulfate (PEDMARK)</a>	FFS
<a href="#">Sutimlimab-jome (ENJAYMO)</a>	FFS
<a href="#">tvidenofusp alfa (AVLAYAH)</a>	FFS and CCO populations beginning 5/1/26
<a href="#">Tofersen (QALSODY)</a>	FFS
<a href="#">Trientine tetrahydrochloride (CUVRIOR)</a>	FFS
<a href="#">Velmanase alfa-tycv (LAMZEDE)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Vestronidase alfa-vjbc (MEPSEVII)</a>	FFS and CCO populations beginning 1/1/26
<a href="#">Zilucoplan (ZILBRYSQ)</a>	FFS

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	<b>Yes:</b> Go to #4	<b>No:</b> If not eligible for EPSDT review: Pass to RPh. Deny; not funded by the OHP  If eligible for EPSDT review: 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.

Approval Criteria		
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?  *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>	<b>Yes:</b> Go to #7	<b>No:</b> Pass to RPh. Deny; medical appropriateness.
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.
8. Have other therapies been tried and failed?  <u>Note: drugs carved-out of CCOs require secondary review by DMAP before approval.</u>	<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
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

Renewal Criteria		
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?  <u>Note: drugs carved-out of CCOs require secondary review by DMAP before approval.</u>	<b>Yes:</b> <u>1<sup>st</sup> renewal:</u> Approve for up to 6 months. <u>Subsequent renewals: Approve up to 12 months.</u>  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)?  <u>Note: drugs carved-out of CCOs require secondary review by DMAP before approval.</u>	<b>Yes:</b> <u>1<sup>st</sup> renewal:</u> Approve for up to 6 months. <u>Subsequent renewals: Approve up to 12 months.</u>  Document benefit and provider attestation	<b>No:</b> Pass to RPh. Deny; medical appropriateness

P&T/DUR Review: 2/26; 8/25; 6/25; 4/25; 2/25; 12/24; 10/24; 8/24; 4/24; 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: 3/1/26; 9/15/25; 5/12/25; 3/10/25; 1/1/25; 9/1/24; 5/1/24; 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