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
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. Included orphan drugs

Drug	Covered Population
ADAMTS13, recombinant-krhn (ADZYNMA)	FFS
Aficamten (MYQORZO)	FFS
Allogeneic processed thymus tissue-agdc (RETHYMIC)	FFS and CCO populations beginning 1/1/26
Alpelisib (VIJOICE)	FFS
Asfotase alfa (STRENSIQ)	FFS and CCO populations beginning 1/1/26
Atidarsagene autotemcel (LENMELDY)	FFS and CCO populations beginning 1/1/26
Avacopan (TAVNEOS)	FFS
Axatilimab-csfr (NIKTIMVO)	FFS
Belumosudil (REZUROCK)	FFS
Burosumab-twza (CRYSVITA)	FFS
Cerliponase alfa (BRINEURA)	FFS
Chenodiol (CTEXLI)	FFS and CCO populations beginning 1/1/26
Crinecefont (CRENESSITY)	FFS
Crovalimab-akkz (PIASKY)	FFS
Danicopan (VOYDEYA)	FFS
Eculizumab (SOLIRIS)	FFS
Eculizumab-aagh (EPYSQLI)	FFS
Eculizumab-aeeb (BKEMV)	FFS
Eladocagene exuparvovec-tneq (KEBILDI)	FFS and CCO populations beginning 1/1/26
Elafibranor (IQIRVO)	FFS
Elapegademase-lvlr (REVCOVI)	FFS and CCO populations beginning 1/1/26
Elivaldogene autotemcel (SKYSONA)	FFS and CCO populations beginning 1/1/26
Elosulfase alfa (VIMIZIM)	FFS and CCO populations beginning 1/1/26
Fosdenopterin (NULIBRY)	FFS

Galsulfase (NAGLAZYME)	FFS and CCO populations beginning 1/1/26
Givosiran (GIVLAARI)	FFS
Idursulfase (ELAPRASE)	FFS and CCO populations beginning 1/1/26
Inebilizumab-cdon (UPLIZNA)	FFS
Iptacopan (FABHALTA)	FFS
Leniolisib (JOENJA)	FFS
Levoketoconazole (RECORLEV)	FFS
Lonafarnib (ZOKINVY)	FFS and CCO populations beginning 1/1/26
Lumasiran (OXLUMO)	FFS and CCO populations beginning 1/1/26
Luspatercept (REBLOZYL)	FFS
Maralixibat (LIVMARLI)	FFS and CCO populations beginning 1/1/26
Mavacamten (CAMZYOS)	FFS
Mavorixafor (XOLREMDI)	FFS
Mirdametinib (GOMEKLI)	FFS
Mitapivat (PYRUKYND)	FFS
Nedosiran (RIVFLOZA)	FFS
Nerandomilast (JASCAYD)	FFS
Nipocalimab-aahu (IMAAVY)	FFS
Odevixibat (BYLVAY)	FFS and CCO populations beginning 1/1/26
Olipudase alfa-rpcp (XENPOZYME)	FFS and CCO populations beginning 1/1/26
Palopegteriparatide (YORVIPATH)	FFS
Paltusotine (PALSONIFY)	FFS
Pegcetacoplan (EMPAVELI)	FFS
Plasminogen, human-tvmh (RYPLAZIM)	FFS
Pozelimab-bbfg (VEOPOZ)	FFS and CCO populations beginning 1/1/26
Ravulizumab-cwvz (ULTOMIRIS)	FFS
Remestemcel-L-rknd (RYONCIL)	FFS
Rezafungin (REZZAYO)	FFS
Rozanolixizumab-noli (RYSTIGGO)	FFS
Satralizumab-mwge (ENSPRYNG)	FFS
Seladelpar (LIVDELZI)	FFS
Sodium thiosulfate (PEDMARK)	FFS
Sutimlimab-jome (ENJAYMO)	FFS
Tofersen (QALSODY)	FFS
Trientine tetrahydrochloride (CUVRIOR)	FFS
Velmanase alfa-tycv (LAMZEDE)	FFS and CCO populations beginning 1/1/26
Vestronidase alfa-vjbk (MEPSEVII)	FFS and CCO populations beginning 1/1/26
Zilucoplan (ZILBRYSQ)	FFS

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	**Yes:** Go to #4	**No:** 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)?	**Yes:** Go to #4	**No:** 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 **Table 1)**? 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.	**Yes**: Go to #5	**No:** Pass to RPh. Deny; medical appropriateness.
5. Is the request for continuation of therapy in a patient previously approved by FFS?	**Yes:** Go to **Renewal Criteria**	**No:** 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: https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness.
7. Is this medication therapy being prescribed by, or in consultation with, an appropriate medical specialist?	**Yes:** Go to #8	**No:** Pass to RPh. Deny; medical appropriateness.

Approval Criteria		
8. Have other therapies been tried and failed?	**Yes:** Approve for up to 3 months (or length of treatment) whichever is less Document therapies which have been previously tried	**No:** 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?	**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. Is baseline efficacy monitoring available?	**Yes:** Go to #4	**No:** 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?	**Yes:** Approve for up to 6 months Document benefit	**No:** 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)?	**Yes**: Approve for up to 6 months Document benefit and provider attestation	**No:** 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