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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. New orphan drugs

<u>Generic Name</u>	<u>Brand Name</u>
Lonafarnib	ZOKINVY
Lumasiran	OXLUMO

Recommendation:

- Modify PA to include new, recently approved antineoplastic drugs.

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 physician 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. Indications for orphan drugs based on FDA labeling

Drug	Indication	Age	Dose	Recommended Monitoring
Burosumab-twza (CRYSVITA)	X-linked hypophosphatemia (XLH) FGF23-related hypophosphatemia in tumor-induced osteomalacia (TIO)	<u>XLH</u> ≥ 6 months <u>TIO</u> ≥ 2 years	<u>Pediatric <18 years:</u> Initial (administered SC every 2 weeks): XLH <ul style="list-style-type: none"> • <10 kg: 1mg/kg • ≥10 mg: 0.8 mg/kg <u>TIO</u> <ul style="list-style-type: none"> • 0.4 mg/kg Max dose of 2 mg/kg (not to exceed 90 mg for XLH or 180 for TIO) <u>Adult:</u> <u>XLH</u> 1 mg/kg monthly (rounded to nearest 10 mg; max 90 mg) TIO: 0.5 mg/kg monthly initially (Max	<u>Baseline and Ongoing Monitoring</u> <ul style="list-style-type: none"> • Use of active vitamin D analogues or oral phosphate within prior week; concurrent use is contraindicated • Fasting serum phosphorous: do not administer if serum phosphorous is within or above normal range • Renal function: use is contraindicated in ESRD or with severe renal impairment (CrCl <30 mL/min for adults or eGFR <30 mL/min/1.73m² for pediatric patients) • 25-hydroxy vitamin D levels: supplementation with vitamin D (cholecalciferol or ergocalciferol) is recommended as needed. <u>Additional baseline monitoring for TIO only:</u> <ul style="list-style-type: none"> • Documentation that tumor cannot be located or is unresectable • Elevated FGF-23 levels

			2 mg/kg or 180mg every 2 weeks)	<ul style="list-style-type: none"> Documentation indicating concurrent treatment for the underlying tumor is not planned (i.e., surgical or radiation)
Cerliponase alfa (BRINEURA)	To slow the loss of ambulation in symptomatic Batten Disease (late infantile neuronal ceroid lipofuscinosis type 2 or TPP1 deficiency)	3-17 years	300 mg every other week via intraventricular route	<u>Baseline Monitoring</u> <ul style="list-style-type: none"> Enzymatic or genetic testing to confirm tripeptidyl peptidase 1 deficiency or CLN2 gene mutation Baseline motor symptoms (e.g., ataxia, motor function, etc) ECG in patients with a history of bradycardia, conduction disorders or structural heart disease <u>Ongoing Monitoring</u> <ul style="list-style-type: none"> Disease stabilization or lack of decline in motor symptoms compared to natural history
elapegamase-ivlr (REVCovi)	adenosine deaminase severe combined immune deficiency (ADA-SCID)	N/A	Initial: 0.2mg/kg twice weekly; No max dose	<u>Baseline Monitoring</u> <ul style="list-style-type: none"> CBC or platelet count <u>Ongoing Monitoring</u> <ul style="list-style-type: none"> trough plasma ADA activity trough erythrocyte dAXP levels (twice yearly) total lymphocyte counts
Givosiran (GIVLAARI)	acute hepatic porphyria	≥ 18 years	2.5 mg/kg monthly	<u>Baseline and ongoing monitoring</u> <ul style="list-style-type: none"> Liver function tests
Lonafarnib (ZOKINVY)	To reduce risk of mortality in Hutchinson-Gilford Progeria Syndrome For treatment of processing-deficient Progeroid Laminopathies with either: <ul style="list-style-type: none"> Heterozygous LMNA mutation with progerin-like protein accumulation 	≥12 months AND ≥0.39 m ² body surface area	<ul style="list-style-type: none"> Initial 115 mg/m² twice daily Increase to 150 mg/m² twice daily after 4 months Round all doses to nearest 25 mg	<u>Baseline and ongoing monitoring</u> <ul style="list-style-type: none"> Contraindicated with strong or moderate CYP3A inducers, midazolam, lovastatin, simvastatin, or atorvastatin Comprehensive metabolic panel CBC Ophthalmological evaluation Blood pressure Pregnancy test (if childbearing potential)

	<ul style="list-style-type: none"> ○ Homozygous or compound heterozygous ZMPSTE24 mutations 			
Lumasiran (OXLUMO)	Treatment of primary hyperoxaluria type 1 to lower urinary oxalate levels	Adult and pediatric patients	<p><10 kg <u>Loading:</u> 6 mg/kg once/month for 3 doses <u>Maintenance:</u> 3 mg/kg once/month</p> <p>10 kg to <20 kg <u>Loading:</u> 6 mg/kg once/month for 3 doses <u>Maintenance:</u> 6 mg/kg once every 3 months</p> <p>≥ 20 kg <u>Loading:</u> 3 mg/kg once/month for 3 doses <u>Maintenance:</u> 3 mg/kg once every 3 months</p> <p>All maintenance dosing begins 1 month after last loading dose.</p>	
Luspatercept (REBLOZYL)	Anemia (Hg <11 g/dL) due to beta thalassemia in patients requiring regular red blood cell transfusions	≥ 18 years	<p>Initial: 1 mg/kg subcutaneously</p> <p>Max dose of 1.25 mg/kg every 3 weeks for beta thalassemia</p>	<p><u>Baseline Monitoring/Documentation</u></p> <ul style="list-style-type: none"> • Number of red blood cell transfusions in the prior 2 months; minimum of 2 RBC units over the prior 8 weeks in patients with myelodysplastic syndromes

	Anemia (Hg <11 g/dL) due to myelodysplastic syndromes with ring sideroblasts or myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis		Max dose of 1.75 mg/kg every 3 weeks for myelodysplastic syndromes	<ul style="list-style-type: none"> • Trial and failure of an erythropoiesis stimulating agent in patients with myelodysplastic syndromes • Hemoglobin level • Blood pressure <p><u>Ongoing Monitoring</u></p> <ul style="list-style-type: none"> • Discontinue if there is not a decrease in transfusion burden after 3 maximal doses (about 9-15 weeks) • Hemoglobin level • Blood pressure
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Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	Yes: Go to #3	No: Pass to RPh. Deny; not funded by the OHP.
3. Is the request for a drug FDA-approved for the indication, age, and dose as defined in Table 1 ?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness.
4. Is the request for continuation of therapy in a patient previously approved by FFS?	Yes: Go to Renewal Criteria	No: Go to #5
5. Is baseline monitoring recommended for efficacy or safety (e.g., labs, baseline symptoms, etc) AND has the provider submitted documentation of recommended monitoring parameters?	Yes: Go to #6	No: Pass to RPh. Deny; medical appropriateness.
6. Is this medication therapy being prescribed by, or in consultation with, an appropriate medical specialist?	Yes: Go to #7	No: Pass to RPh. Deny; medical appropriateness.

Approval Criteria		
7. 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: 2/21 (SF); 8/20 (SS); 6/20; 2/20
 Implementation: 3/1/21; 11/1/20; 9/1/20; 7/1/20