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Prior Authorization Criteria Update: Biologics for Rare Diseases; Orphan Drug

Plain Language Summary:

- The Biologics for Rare Diseases class has been reviewed by the Pharmacy and Therapeutics committee four times since 2021 because the Food and Drug Administration (FDA) has approved new medicines and expanded the list of conditions for which older medicines can be prescribed.
- Only two patients in Medicaid fee-for-service (FFS) program have received more than one dose of a medicine in this class from July 2022 to June 2024.
- All medicines in this class are classified as orphan drugs. Medicines are classified as orphan drugs by the FDA when they are used to treat very uncommon diseases and conditions.
- We recommend using the Orphan drug policy to simplify management for most of these medicines. This will improve access when the FDA makes updates.
- Add recently approved orphan drug approvals to orphan drug policy.

Purpose of Update:

The purpose of this update is to evaluate utilization of medications in the "Biologics for Rare Diseases". Since 2021, the Medicaid fee-for-service (FFS) program has reviewed medications in the "Biologics for Rare Diseases" class 4 times due to new agent approvals and multiple expanded indications by the Food and Drug Administration (FDA). Since last review in February 2023, one new agent (crovalimab-akkz [PIASKY]) and two biosimilars (eculizumab-aeeb [BKEMV], eculizumab-aagh [EPYSQLI]) have been approved and two existing agents have received expanded indications. This class includes agents approved for rare conditions such as paroxysmal nocturnal hemoglobinuria and neuromyelitis optica spectrum disorder. All current medicines in this class are designated as "orphan" through the FDA. Orphan drugs are defined by the FDA as drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States or that affect more than 200,000 people but are not expected to recover the costs of developing and marketing a treatment.

From July 2022 to June 2024, only 2 patients in Medicaid FFS have received more than one dose from a medication in this class. Given infrequent use and frequent updates needed due to additional label indications, use of the orphan drug policy instead of drug specific criteria could improve alignment of the Medicaid FFS policy and FDA labeled indications. The orphan drug policy is used to improve bandwidth for topics at the Pharmacy and Therapeutics (P&T) meetings while supporting medically appropriate use based on the FDA package labeling. This will allow more rapid alignment with current FDA labeling as expanded indications are approved. Comparison of current, drug specific prior authorizations with the orphan drug policy show minimal differences in usage criteria for most drugs in this preferred drug list (PDL) class. One agent, efgartigimod alfa-fcab +/- hyaluronidase (VYVGART AND VYVGART HYTRULO), has step therapy requirements for treatment of general myasthenia gravis in the drug specific prior authorization criteria.

Newly approved orphan drugs to be added to orphan drug policy in **Table 1**.

Table 1. Updated Orphan Drugs

Author: Sara Fletcher, PharmD, MPH, BCPS

Date: December 2024

Generic Name	Brand Name
levacetylleucine	AQNEURSA
arimoclomol citrate	MIPLYFFA

Recommendation:

- Move inebilizumab-cdon (UPLIZNA), ravulizumab-cwvz (ULTOMIRIS), satralizumab-mwge (ENSPRYNG), eculizumab (SOLIRIS), eculizumab-aeeb (BKEMV), eculizumab-aagh (EPYSQLI), pegcetacoplan (EMPAVELI), and crovalimab-akkz (PIASKY) to the orphan drug class and apply the orphan drug policy. Remove PDL status. Retire drug specific prior authorizations linked to these drugs.
- Add newly approved orphan drugs (**Table 1**) to orphan drug policy.

Reference:

Food and Drug Administration. Drugs@FDA: FDA-approved drugs database. Center for Drug Evaluation and Research. Available at : https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm Accessed: Sept 6, 2024.

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. Included orphan drugs

ADAMTS13, recombinant-krhn (ADZYNMA)
Allogeneic processed thymus tissue-agdc (RETHYMIC)
Alpelisib (VIJOICE)
arimoclomol citrate (MIPLYFFA)
Avacopan (TAVNEOS)
Belumosudil (REZUROCK)
Beremagene geperpavec-svdt (VYJUVEK)
Birch triterpenes (FILSUVEZ)
Burosumab-twza (CRYSVITA)
Cerliponase alfa (BRINEURA)
Crovalimab-akkz (PIASKY)
Danicopan (VOYDEYA)
Eculizumab (SOLIRIS)
Eculizumab-aagh (EPYSQLI)
Eculizumab-aeeb (BKEMV)
Elafibranor (IQIRVO)
Elapegademase-lvlr (REVCOVI)
Elivaldogene autotemcel (SKYSONA)
Fosdenopterin (NULIBRY)
Givosiran (GIVLAARI)
Inebilizumab-cdon (UPLIZNA)

Iptacopan (FABHALTA)
Leniolisib (JOENJA)
Levacetylleucine (AQNEURSA)
Levoketoconazole (RECORLEV)
Lonafarnib (ZOKINVY)
Lumasiran (OXLUMO)
Luspatercept (REBLOZYL)
Maralixibat (LIVMARLI)
Mavorixafor (XOLREMDI)
Mitapivat (PYRUKYND)
Nedosiran (RIVFLOZA)
Odevixibat (BYLVAY)
Olipudase alfa-rpcp (XENPOZYME)
Palovarotene (SOHONOS)
Palopegteriparatide (YORVIPATH)
Pegcetacoplan (EMPAVELI)
Plasminogen, human-tvmh (RYPLAZIM)
Pozelimab-bbfg (VEOPOZ)
Ravulizumab-cwvz (ULTOMIRIS)
Rozanolixizumab-noli (RYSTIGGO)
Satralizumab-mwge (ENSPRYNG)
Seladelpar (LIVDELZI)
Sodium thiosulfate (PEDMARK)
Sutimlimab-jome (ENJAYMO)
Tofersen (QALSODY)
Trientine tetrahydrochloride (CUVRIOR)
Velmanase alfa-tycv (LAMZEDE)
Zilucoplan (ZILBRYSQ)

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	**Yes:** Go to #4	**No:** For current age ≥ 21 years: Pass to RPh. Deny; not funded by the OHP For current age < 21 years: Go to #3

Approval Criteria		
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: 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: 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

Eculizumab (SOLIRIS) RETIRE

Goal(s):

- Restrict use to OHP-funded conditions and according to OHP guidelines for use.
- Promote use that is consistent with national clinical practice guidelines and medical evidence.
- Restrict use to FDA-approved indications.

Length of Authorization:

- Up to 12 months

Requires PA:

- Eculizumab (SOLIRIS) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is this an FDA approved indication?	**Yes:** Go to #3	**No:** Pass to RPh. Deny; medical appropriateness
3. Is the diagnosis funded by OHP?	**Yes:** Go to #5	**No:** For current age ≥ 21 years: Pass to RPh. Deny; not funded by the OHP For current age < 21 years: Go to #4

Approval Criteria		
4. 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 #5	**No:** Pass to RPh. Deny; medical necessity.
5. Is this request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to #6
6. Has the patient been vaccinated against *Streptococcus pneumoniae, Haemophilus influenzae* type B, and *Neisseria meningitidis serogroups A, C, W, and Y and serogroup B* according to current Advisory Committee on Immunization Practice (ACIP) recommendations for vaccination in patients with complement deficiencies*?* Note: Prescribing information recommends vaccination at least 2 weeks prior to starting therapy. If the risk of delaying therapy outweighs the risk of developing a serious infection, a 2-week course of antibiotic prophylaxis must be immediately initiated if vaccines are administered less than 2 weeks before starting complement therapy.	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness
7. Is the diagnosis one of the following: • Neuromyelitis Optica Spectrum Disorder in an adult who is anti-aquaporin-4 (AQP4) antibody positive, • Paroxysmal Nocturnal Hemoglobinuria (PNH), • atypical Hemolytic Uremic Syndrome (aHUS)? (Note: Eculizumab is not indicated for the treatment of patients with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS).	**Yes:** Go to #8	**No:** Go to #9
8. Does the requested dosing align with FDA-approved dosing (**Table 1**)?	**Yes:** Approve for 12 months	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
9. Is the request for a diagnosis of myasthenia gravis in an adult patient who is Acytelcholine Receptor (AChR) antibody-positive?	**Yes:** Go to # 10	**No:** Pass to RPh. Deny; medical appropriateness
10. Has the patient tried: • at least 2 or more immunosuppressant therapies (e.g., glucocorticoids in combination with azathioprine or mycophenolate mofetil or cyclosporine or tacrolimus or methotrexate or rituximab) for 12 months without symptom control OR • at least 1 or more nonsteroidal immunosuppressant with maintenance intravenous immunoglobulin once monthly or plasma exchange therapy (PLEX) over 12 months without symptom control?	**Yes:** Go to #11	**No:** Pass to RPh. Deny; medical appropriateness
11. Is the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score ≥ 6?	**Yes:** Approve for 12 months	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there objective documentation of treatment benefit from baseline? Appropriate measures will vary by indication (e.g., hemoglobin stabilization, decreased transfusions, symptom control or improvement, functional improvement, etc.).	**Yes:** Approve for 12 months Document baseline assessment and physician attestation received.	**No:** Pass to RPh. Deny; medical appropriateness

Table 1. FDA-Approved Indications and Dosing for Eculizumab[1]

	Eculizumab

FDA-approved Indications	• Neuromyelitis Optica Spectrum Disorder (NMOSD) in adult patients who are anti-AQP4-IgG-antibody • Reducing hemolysis in patients with paroxysmal nocturnal hemoglobinuria (PNH) • Inhibiting complement-mediated thrombotic microangiopathy in patients with atypical hemolytic uremic syndrome (aHUS) • Treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor antibody positive		
Recommended NMOSD dose in patients 18 yo and older	900 mg IV every week x 4 weeks, followed by 1200 mg IV for the fifth dose 1 week later, then 1200 mg IV every 2 weeks thereafter		
Recommended PNH dose in patients 18 yo and older	600 mg IV every week x 4 weeks, followed by 900 mg IV for the fifth dose 1 week later, then 900 mg IV every 2 weeks thereafter		
Recommended aHUS dose in patients less than 18 yo	**Body Weight** 5 kg to 9 kg 10 kg to 19 kg 20 kg to 29 kg 30 kg to 39 kg ≥ 40 kg	**Induction Dose** 300 mg weekly x 1 dose 600 mg weekly x 1 dose 600 mg weekly x 2 doses 600 mg weekly x 2 doses 900 mg weekly x 4 doses	**Maintenance Dose** 300 mg at week 2; then 300mg every 3 weeks 300 mg at week 2; then 300mg every 2 weeks 600 mg at week 3; then 600mg every 2 weeks 900 mg at week 3; then 900 mg every 2 weeks 1200 mg at week 5; then 1200 mg every 2 weeks
Recommended aHUS dose in patients 18 yo and older	900 mg IV every week x 4 weeks, followed by 1200 mg IV for the fifth dose 1 week later, then 1200 mg IV every 2 weeks thereafter		
Recommended gMG dose	900 mg IV every week x 4 weeks, followed by 1200 mg IV for the fifth dose 1 week later, then 1200 mg IV every 2 weeks thereafter		
Dose Adjustment in Case of Plasmapheresis, Plasma Exchange, or Fresh Frozen Plasma Infusion	Dependent on most recent eculizumab dose: refer to prescribing information for appropriate dosing (300 mg to 600 mg)		

1. SOLIRIS (eculizumab) Solution for Injection Prescribing Information. Boston, MA: Alexion Pharmaceuticals, Inc. 11/2020.

P&T/DUR Review: 2/23 (DM); 12/21; 4/21
Implementation: 5/1/21

Efgartigimod (VYVGART, VYVGART HYTRULO)

Goal(s):

- Restrict use to OHP-funded conditions.
- Promote use that is consistent with medical evidence.

Length of Authorization:

- Up to 12 months

Requires PA:

- Efgartigimod alfa-fcab (VYVGART) and efgartigimod alfa-hyaluronidase-qvfc (VYVGART HYTRULO) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	**Yes:** Go to #4	**No: No:** For current age ≥ 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)?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical necessity.
4. Is this an FDA approved indication?	**Yes**: Go to #5	**No:** Pass to RPh. Deny; medical appropriateness
5. Is this a request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to #6
6. Does the patient have an active infection?	**Yes:** Pass to RPh. Deny; medical appropriateness.	**No:** Go to #7

Approval Criteria		
7. Has the patient received, or have contraindications to, all routine immunizations recommended for their age? Note: Routine vaccinations for patients at least 2 years of age typically included hepatitis B, hepatitis A, diphtheria, tetanus, pertussis, pneumococcal conjugate, inactivated poliovirus, influenza, and at least 2 doses of measles, mumps, rubella, and varicella. Immunization with live vaccines is not recommended during efgartigimod treatment.	**Yes:** Go to #8. Document physician attestation of immunization history	**No:** Pass to RPh. Deny; medical appropriateness. Administer vaccines before initiation of a new treatment cycle of efgartigimod
8. Does the patient have a positive serological test for anti-acetylcholine receptor (AchR) antibodies?	**Yes:** Go to #9	**No:** Pass to RPh. Deny; medical appropriateness
9. Does the patient have a Myasthenia Gravis Foundation of America Clinical Classification of class II, III or IV?	**Yes:** Go to #10	**No:** Pass to RPh. Deny; medical appropriateness
10. Does the patient have a myasthenia gravis-specific activities of daily living scale (MG-ADL) total score of 5 points or more?	**Yes:** Go to #11 Record baseline MG-ADL score	**No:** Pass to RPh. Deny; medical appropriateness
11. Has the patient received or is currently receiving two immunosuppressant therapies (as monotherapy or in combination) for at least one year without adequate symptom control or do they have contraindications to these therapies? Example immunosuppressant therapies: - Azathioprine - Cyclosporine - Mycophenolate mofetil - Tacrolimus - Methotrexate - Cyclophosphamide	**Yes:** Go to #12	**No:** Pass to RPh. Deny; medical appropriateness. Recommend trial of immunosuppressant therapy

Approval Criteria		
12. Is the request for efgartigimod dosing that corresponds to FDA labeling? • 10 mg/kg once weekly for 4 weeks • For patients weighing 120 kg or more, the recommended dose is 1200 mg per infusion	**Yes:** Approve for up to two cycles. Each cycle is 1 dose/week for 4 weeks. The second cycle should not be administered sooner than 50 days from start of previous cycle.	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Has it been 50 days or more from the start of the previous efgartigimod treatment cycle?	**Yes**: Go to #2	**No:** Pass to RPh. Deny; medical appropriateness
2. Is this request for the first renewal of efgartigimod?	**Yes**: Go to #3	**No:** Go to #4
3. Has the patient experienced a reduction in symptoms of at least 2 points from MG-ADL total baseline score?	**Yes**: Approve for up to 5 cycles. Each cycle is 1 dose/week for 4 weeks. Additional cycles should not be administered sooner than 50 days from start of previous cycle. Record MG-ADL score	**No:** Pass to RPh. Deny; medical appropriateness
4. Has the patient maintained a stable MG-ADL score over the last 12 months of efgartigimod therapy?	**Yes**: Approve for up to 7 cycles. Each cycle is 1 dose/week for 4 weeks. Additional cycles should not be administered sooner than 50 days from start of previous cycle. Record MG-ADL score	**No:** Pass to RPh. Deny; medical appropriateness

Inebilizumab-cdon (UPLINZA) RETIRE

Goal(s):

- Restrict use to OHP funded conditions and according to OHP guidelines for use.
- Promote use that is consistent with national clinical practice guidelines and medical evidence.
- Restrict use to FDA-approved indications.

Length of Authorization:

- Up to 12 months

Requires PA:

- Inebilizumab-cdon (UPLINZA) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is this an FDA approved indication?	**Yes:** Go to #3	**No:** Pass to RPh. Deny; medical appropriateness
3. Is the diagnosis funded by OHP?	**Yes:** Go to #5	**No:** For current age ≥ 21 years: Pass to RPh. Deny; not funded by the OHP For current age < 21 years: Go to #4

Approval Criteria		
4. 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 #5	**No:** Pass to RPh. Deny; medical necessity.
5. Is this request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to # 6
6. Is the request for Neuromyelitis Optica Spectrum Disorder in an adult who is anti-aquaporin-4 (AQP4) antibody positive?	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness
7. Has the patient been screened for Hepatitis B and tuberculosis infection before starting treatment?	**Yes:** Go to #8	**No:** Pass to RPh. Deny; medical appropriateness
8. Does the patient have active Hepatitis B or untreated latent tuberculosis?	**Yes:** Pass to RPh. Deny; medical appropriateness	**No:** Approve for 12 months

Renewal Criteria		
1. Is there objective documentation of treatment benefit from baseline? Appropriate measures will vary by indication (e.g., hemoglobin stabilization, decreased transfusions, symptom improvement, functional improvement, etc.).	**Yes:** Approve for 12 months Document baseline assessment and physician attestation received.	**No:** Pass to RPh. Deny; medical appropriateness

P&T/DUR Review: 2/23 (DM); 4/21
Implementation: 5/1/21

Pegcetacoplan (EMPAVELI) RETIRE

Goal(s):

- Restrict use to OHP-funded conditions and according to OHP guidelines for use.
- Promote use that is consistent with national clinical practice guidelines and medical evidence.
- Restrict use to FDA-approved indications.

Length of Authorization:

- Up to 12 months

Requires PA:

- EMPAVELI (pegcetacoplan) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is this an FDA approved indication?	**Yes:** Go to #3	**No:** Pass to RPh. Deny; medical appropriateness
3. Is the diagnosis funded by OHP?	**Yes:** Go to #4	**No:** For current age ≥ 21 years: Pass to RPh. Deny; not funded by the OHP For current age < 21 years: Go to #4
4. 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 #5	**No:** Pass to RPh. Deny; medical necessity.

Approval Criteria		
5. Is this request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to # 6
6. Has the patient been vaccinated against *Streptococcus pneumoniae, Haemophilus influenzae* type B, and *Neisseria meningitidis serogroups A, C, W, and Y and serogroup B* according to current Advisory Committee on Immunization Practice (ACIP) recommendations for vaccination in patients with complement deficiencies*?* Note: Prescribing information recommends vaccination at least 2 weeks prior to starting therapy. If the risk of delaying therapy outweighs the risk of developing a serious infection, a 2-week course of antibiotic prophylaxis must be immediately initiated if vaccines are administered less than 2 weeks before starting complement therapy.	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness
7. Is the diagnosis for an adult (age 18 years or older) with Paroxysmal Nocturnal Hemoglobinuria?	**Yes:** Approve for 12 months	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there objective documentation of treatment benefit from baseline? Appropriate measures will vary by indication (e.g., hemoglobin stabilization, decreased transfusions, symptom improvement, functional improvement, etc.).	**Yes:** Approve for 12 months Document baseline assessment and physician attestation received.	**No:** Pass to RPh. Deny; medical appropriateness

Ravulizumab (ULTOMIRIS) RETIRE

Goal(s):

- Restrict use to OHP-funded conditions and according to OHP guidelines for use.
- Promote use that is consistent with national clinical practice guidelines and medical evidence.
- Restrict use to FDA-approved indications.

Length of Authorization:

- Up to 12 months

Requires PA:

- Ravulizumab (ULTOMIRIS) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is this an FDA approved indication?	**Yes:** Go to #3	**No:** Pass to RPh. Deny; medical appropriateness.
3. Is the diagnosis funded by OHP?	**Yes:** Go to #5	**No:** For current age ≥ 21 years: Pass to RPh. Deny; not funded by the OHP For current age < 21 years: Go to #4

Approval Criteria		
4. 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 #5	**No:** Pass to RPh. Deny; medical necessity.
5. Is this request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to # 6
6. Has the patient been vaccinated against *Streptococcus pneumoniae, Haemophilus influenzae* type B, and *Neisseria meningitidis serogroups A, C, W, and Y and serogroup B* according to current Advisory Committee on Immunization Practice (ACIP) recommendations for vaccination in patients with complement deficiencies*?* Note: Prescribing information recommends vaccination at least 2 weeks prior to starting therapy. If the risk of delaying therapy outweighs the risk of developing a serious infection, a 2-week course of antibiotic prophylaxis must be immediately initiated if vaccines are administered less than 2 weeks before starting complement therapy.	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
7. Is the diagnosis for a patient with one of the following indications: • at least 1 month of age or older and weighs at least 5 kg with atypical Hemolytic Uremic Syndrome (aHUS) or Paroxysmal Nocturnal Hemoglobinuria (PNH) or • an adult with generalized myasthenia gravis (gMG) who is anti-acetylcholine receptor (AchR) antibody positive? Note: Ravulizumab is not indicated for the treatment of patients with Shiga toxin *E. coli* related hemolytic uremic syndrome (STEC-HUS).	**Yes:** Go to #8	**No:** Pass to RPh. Deny; medical appropriateness
8. Is the request for intravenous dosing?	**Yes:** Go to #9	**No:** Go to #10
9. Does the requested intravenous dosing align with the FDA-approved dosing (**Table 1**)?	**Yes:** Approve for 12 months	**No:** Pass to RPh. Deny; medical appropriateness
10. Is the request for subcutaneous (SC) administration of ravlizumab 490 mg SC once a week in an adult weighing 40 kg or greater with PNH or aHUS? Note: Subcutaneous administration of ravulizumab is not approved for use in pediatric patients.	**Yes:** Approve for 12 months	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there objective documentation of treatment benefit from baseline? Appropriate measures will vary by indication (e.g., hemoglobin stabilization, decreased transfusions, symptom improvement, functional improvement, etc.).	**Yes:** Approve for 12 months Document baseline assessment and physician attestation received.	**No:** Pass to RPh. Deny; medical appropriateness

Table 1. FDA-Approved Intravenous Weight-based Infusion Dosing for Ravulizumab in Adults and Pediatric Patients aged 1 month and older with PNH, aHUS, or gMG[1]

Body Weight	Indications	Loading Dose	Maintenance Dose (begins 2 weeks after loading dose)
5 to 9 kg	aHUS and PNH	600 mg	300 mg every 4 weeks
10 to 19 kg	aHUS and PNH	600 mg	600 mg every 4 weeks
20 to 29 kg	aHUS and PNH	900 mg	2,100 mg every 8 weeks
30 to 39 kg	aHUS and PNH	1,200 mg	2,700 mg every 8 weeks
40 to 59 kg	aHUS, gMG, and PNH	2,400 mg	3,000 mg every 8 weeks
60 to 99 kg	aHUS, gMG, and PNH	2,700 mg	3,300 mg every 8 weeks
100 kg or greater	aHUS, gMG, and PNH	3,000 mg	3,600 mg every 8 weeks
Abbreviations: aHUS = atypical hemolytic uremic syndrome; gMG = generalized myasthenia gravis; PNH = paroxysmal nocturnal hemoglobinuria			

1. ULTOMIRIS (Ravulizumab-cwvz) Solution for Intravenous Infusion Prescribing Information. Boston, MA: Alexion Pharmaceuticals Inc. 7/2022.

P&T/DUR Review 2/23 (DM); 12/21 (DM); 4/21 (DM)
Implementation: 4/1/23; 1/1/22; 5/1/21

Satralizumab-mwge (ENSPRYNG) RETIRE

Goal(s):

- Restrict use to OHP funded conditions and according to OHP guidelines for use.
- Promote use that is consistent with national clinical practice guidelines and medical evidence.
- Restrict use to FDA-approved indications.

Length of Authorization:

- Up to 12 months

Requires PA:

- Satralizumab-mwge (ENSPRYNG) 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/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the diagnosis funded by OHP?	**Yes:** Go to #4	**No:** For current age ≥ 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)?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical necessity.
4. Is this an FDA approved indication?	**Yes:** Go to #5	**No:** Pass to RPh. Deny; medical appropriateness
5. Is this request for continuation of therapy?	**Yes:** Go to **Renewal Criteria**	**No:** Go to # 6
6. Is the request for Neuromyelitis Optica Spectrum Disorder in an adult who is anti-aquaporin-4 (AQP4) antibody positive?	**Yes:** Go to #7	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
7. Has the patient been screened for Hepatitis B and tuberculosis infection prior to initiating treatment?	**Yes:** Go to #8	**No:** Pass to RPh. Deny; medical appropriateness
8. Does the patient have active Hepatitis B or untreated latent tuberculosis?	**Yes:** Pass to RPh. Deny; medical appropriateness	**No:** Approve for 12 months

Renewal Criteria		
1. Is there objective documentation of treatment benefit from baseline? Appropriate measures will vary by indication (e.g., hemoglobin stabilization, decreased transfusions, symptom improvement, functional improvement, etc.).	**Yes:** Approve for 12 months Document baseline assessment and physician attestation received.	**No:** Pass to RPh. Deny; medical appropriateness

P&T/DUR Review: 2/23 (DM); 4/21
Implementation: 5/1/21