# **Sickle Cell Disease, Gene Therapy**

## Goal(s):

 Approve Exagamglogene autotemcel (CASGEVY) and Lovotibeglogene autotemcel (LYFGENIA) for conditions supported by evidence of benefit

#### **Length of Authorization:**

Once in a lifetime dose.

### **Requires PA:**

- Exagamglogene autotemcel (billed as pharmacy or provider administered claim)
- Lovotibeglogene autotemcel (billed as pharmacy or provider administered claim)

Note: Any requests on behalf of a patient enrolled in a coordinated care organization (CCO) for an indication other than sickle cell disease should be sent to CCO.

#### **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 for Sickle Cell Disease			
What diagnosis is being treated?	<ul> <li>If for sickle cell disease, continue to question #2;</li> <li>If for beta thalassemia and patient is enrolled in fee-for-service, go to "Approval Criteria for Beta Thalassemia" section below;</li> <li>If for beta thalassemia and patient is enrolled in a coordinated care organization (CCO), request should be directed to that CCO.</li> </ul>		
2. Is this an FDA approved indication?	<b>Yes</b> : Go to #3	No: Pass to RPh. Deny; medical appropriateness	
3. Is there documentation that the patient has never received another gene therapy or hematopoietic stem cell transplant for any diagnosis?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness	
Is the medication being ordered by, or in consultation with, a hematologist?	Yes: Go to #5	No: Pass to RPh. Deny; medical appropriateness	

Approval Criteria for Sickle Cell Disease			
5. Does the patient have Sickle Cell Disease with recurrent vaso-occlusive crisis (VOC)?  Note: Recurrent VOC defined as at least 4 or moreVOC events in previous 24 months or receiving chronic transfusion therapy for recurrent VOC based on provider attestation. If lacking provider attestation, documentation of VOCs could include, but are not limited to, acute chest syndrome, priapism lasting > 2 hours and requiring visit to medical facility, acute pain event requiring visit to medical facility (with pain medications [e.g. opioids, injectable non-steroidal anti-inflammatory	Yes: Go to #6	No: Pass to RPh. Deny; medical appropriateness	
drugs] or red blood transfusion), acute splenic sequestration, or acute hepatic sequestration.			
6. Is the patient 12 years old or older?	<b>Yes</b> : Go to #7	No: Pass to RPh. Deny; medical appropriateness	
7. Does the prescriber attest that the patient's general health and comorbidities have been assessed and that the patient is expected to safely tolerate myeloablation?	Yes: Approve for one- time infusion treatment for lifetime of the patient. Approval is valid for 12 months and will be extended if needed to cover treatment journey. Notify DMAP of approval.	No: Pass to RPh. Deny; medical appropriateness  Notify DMAP of denial.	

Approval Criteria for Beta Thalassemia			
1. Is this an FDA approved indication?	Yes: Go to #2	No: Pass to RPh. Deny; medical appropriateness	
2. Is there documentation that the patient has never received another gene therapy or hematopoietic stem cell transplant for any diagnosis?	Yes: Go to #3	No: Pass to RPh. Deny; medical appropriateness	
Is the medication being ordered by, or in consultation with, a hematologist?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness	

Approval Criteria for Beta Thalassemia			
Does patient have confirmed beta thalassemia?	<b>Yes</b> : Go to #5	No: Pass to RPh. Deny; medical appropriateness	
5. Is the patient transfusion dependent, defined as requiring in each of the past 2 years:	<b>Yes</b> : Go to #8	No: Pass to RPh. Deny; medical appropriateness	
<ul> <li>100 mL/kg/year or more of packed red blood cells (any patient age) OR</li> <li>8 transfusions or more of packed red blood cells per year</li> </ul>			
6. Is the patient 12 years old or older?	<b>Yes</b> : Go to #7	No: Pass to RPh. Deny; medical appropriateness	
7. Is there documentation that the patient does not have cirrhosis or advanced liver disease?	<b>Yes</b> : Go to #8	No: Pass to RPh. Deny; medical appropriateness	
8. Is there documentation that the patient does not have HIV or active infections (acute or chronic) of either hepatitis B or hepatitis C?	<b>Yes</b> : Go to #9	No: Pass to RPh. Deny; medical appropriateness	
9. Does the prescriber attest that the patient's general health and comorbidities have been assessed and that the patient is expected to safely tolerate myeloablation?	<b>Yes</b> : Go to #10	No: Pass to RPh. Deny; medical appropriateness	
10. Is the patient of childbearing potential OR capable of fathering a child?	Yes: Go to #11	<b>No:</b> Go to #13	
11. Is the patient pregnant, actively trying to conceive, or trying to father a child?	Yes: Pass to RPh. Deny; medical appropriateness.	<b>No:</b> Go to #12	
12. Is there documentation that the provider and patient have discussed the teratogenic risks of the drug if the patient were to become pregnant or father a child during treatment and for at least 6 months after administration of the gene therapy?	<b>Yes:</b> Go to #13	No: Pass to RPh. Deny; medical appropriateness	

Approval Criteria for Beta Thalassemia		
13. Is there documentation that the provider and patient have discussed risks of myeloablative treatment on future fertility and options for fertility-preservation?	<b>Yes:</b> Approve for one-time infusion treatment for lifetime of the patient.	<b>No:</b> Pass to RPh. Deny; medical appropriateness
	Approval is valid for 12 months and will be extended if needed to	

cover treatment

journey.

P&T/DUR Review: 8/25; 6/24 (SF) Implementation: 9/15/25; 7/1/24