Etranacogene dezaparvovec

Goal(s):

- Approve Etranacogene dezaparvovec (HEMGENIX) for conditions supported by evidence of benefit
- Incorporate 2-step review process for drugs on the high-cost drug carve-out list.

Length of Authorization:

- Once in a lifetime dose.

Requires PA:

- Etranacogene dezaparvovec (billed as pharmacy or provider administered claim)

Covered Populations:

- FFS and CCO enrolled populations beginning 1/1/26

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 it the FDA approved indication?	**Yes**: Go to #3	**No:** Pass to RPh. Deny; medical appropriateness
3. Is there documentation that the patient has never received another gene therapy for any diagnosis?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical appropriateness
4. Does the patient require continuous routine factor IX prophylaxis?	**Yes**: Go to #7	**No:** Go to #5
5. Does the patient have a history of repeated, serious spontaneous bleeding OR current or historical life threatening hemorrhage?	**Yes**: Go to #6	**No:** Pass to RPh. Deny; medical appropriateness
6. Did these events occur during adherence to physician recommended and maximally adjusted factor IX therapy (including routine factor IX prophylaxis, if indicated) AND adherence to appropriate lifestyle precautions?	**Yes**: Go to #7	**No:** Pass to RPh. Deny; medical appropriateness. Refer to DMAP for secondary review.

Approval Criteria		
7. Does patient have congenital hemophilia B with: • Severe Factor IX deficiency (<1% plasma factor IX activity) OR • Moderately-Severe Factor IX deficiency (1 to 2% plasma factor IX activity) with a severe bleeding phenotype?	**Yes**: Go to #8	**No:** Pass to RPh. Deny; medical appropriateness. Send to Medical Director for review.
8. Is the patient 18 years or older?	**Yes**: Go to #9	**No:** Pass to RPh. Deny; medical appropriateness
9. Is there documentation that the patient does not have factor IX inhibitors by a test within the past 3 months? Note: If positive initial test, may retest, ideally within approximately 2 weeks of original test.	**Yes**: Go to #10 Test Date_____	**No:** Pass to RPh. Deny; medical appropriateness
10. Has this patient had a liver health assessment including all of the following: AST, ALT, ALP, total bilirubin, hepatic ultrasound, elastography, and recent (previous 3 months) screening for hepatitis B and C?	**Yes**: Go to #11	**No:** Pass to RPh. Deny; medical appropriateness
11. Were all hepatic enzymes and hepatic radiological tests normal AND were hepatitis B and C screenings negative? Note: Enzyme elevations which are transient and mild (less than twice the upper limit of normal) may answer “Yes” to this question.	**Yes**: Go to #13	**No:** Go to #12
12. Has the patient been evaluated and cleared for gene therapy treatment by a gastroenterologist or hepatologist?	**Yes**: Go to #13	**No:** Pass to RPh. Deny; medical appropriateness
13. Is there documentation that the patient is either: • HIV negative OR • HIV positive and controlled (CD4 count ≤ 200/μL)?	**Yes**: Go to #14	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
14. Has the provider discussed enrollment in a study to measure pre-existing anti-AAV5 neutralizing antibodies with patient? Note: study details and contact information in gene therapy package insert.[1]	**Yes:** Pass to RPh. Pend; Refer to DMAP for secondary review. Duration: Approvals cover one lifetime dose. Approval are valid for 12 months and will be extended if needed to cover treatment journey.	**No:** Pass to RPh. Deny; medical appropriateness

1. Hemgenix (etranacogene dezaparvovec-drlb) package insert.uniQure, Inc Lexington, MA: https://www.fda.gov/media/163467/download. November 2022.

P&T/DUR Review: 10/23 (SF)
Implementation: 11/1/23