**Corporate Medical Policy:** Etranacogene dezaparvovec-drlb (Hemgenix®) **"Notification"** **POLICY EFFECTIVE OCTOBER 1, 2024**

**Restricted Product(s):**

- etranacogene dezaparvovec-drlb (Hemgenix®) intravenous infusion for administration by a healthcare professional

**FDA Approved Use**:

- For treatment of adults with hemophilia B (congenital Factor IX deficiency) who:
  - Currently use Factor IX prophylaxis therapy, or
  - Have current or historical life-threatening hemorrhage, or
  - Have repeated, serious spontaneous bleeding episodes

**Criteria for Medical Necessity:**

**The restricted product(s) may be considered medically necessary when the following criteria are met:**

1. The patient is 18 years of age or older; **AND**
2. The patient is male; **AND**
3. The patient has a diagnosis of **congenital hemophilia B** (also known as Factor IX deficiency, Christmas disease) **[medical record documentation required]**; **AND**
4. The patient has moderately severe to severe disease, defined by Factor IX baseline residual level less than or equal to 2 IU/dL (≤ 2% of normal circulating Factor IX) **[medical record documentation required, including lab test]**; **AND**
5. The patient does NOT have a history of inhibitors to Factor IX **[medical record documentation required]**; **AND**
6. The patient does NOT have active inhibitors to Factor IX **[medical record documentation required]**; **AND**
7. ONE of the following **[medical record documentation required]**:
   a. The patient is on prophylactic therapy with a Factor IX agent (e.g., AlphaNine SD, Alprolix, BeneFIX, Idelvion, Ixinity, Mononine, Profilnine SD, Rebinyn, Rixubis) AND has had a minimum of 150 exposure days; **OR**
   b. The prescriber has determined that the patient requires improved protection more than is being received from current therapy (e.g., patients with increased bleeding due to severely damaged joints, patients with increased bleeding due to need for anticoagulation, elderly patients with risk for falls); **AND**
8. ONE of the following:
   a. The patient is NOT HIV positive **[medical record documentation required, including lab tests within the past 3 months]**; **OR**
   b. The patient is HIV positive AND is well controlled (i.e., viral load within the past 12 months less than 1000 copies/mL) **[medical record documentation required, including lab results within the past 12 months]**; **AND**

9. The patient's hepatitis B surface antigen is negative **[medical record documentation required, including lab tests within the past 3 months]**; **AND**
10. ONE of the following:
    a. The patient's hepatitis C virus (HCV) antibody is negative **[medical record documentation required, including lab tests within the past 3 months]**; **OR**
    b. The patient's HCV antibody is positive AND the patient's HCV RNA is negative **[medical record documentation required, including lab tests within the past 3 months]**; **AND**
11. The patient is NOT currently using antiviral therapy for hepatitis B or C **[medical record documentation required]**; **AND**
12. The patient does NOT have evidence of any bleeding disorder that is not related to hemophilia B **[medical record documentation required]**; **AND**
13. The patient does NOT have anti-AAV antibodies (e.g., AAV-5) titers that exceed labeling administration instructions **[medical record documentation required, including test results within the past 3 months]**; **AND**
14. The patient has NOT had any previous gene therapy, including the requested agent **[medical record documentation required]**; **AND**
15. The requested dose is within FDA labeled dosing for the requested indication, and the requested quantity does NOT exceed the maximum units allowed for the duration of approval (see table below) **[medical record documentation required]**.

**Duration of Approval:** 180 days (one treatment course per lifetime)

**Please note, for certain identified gene and cellular therapies such as etranacogene dezaparvovec-drlb (Hemgenix®), when coverage is available and the individual meets medically necessary criteria, distribution from a specialty pharmacy provider due to cost (distribution channel restriction) may be required in order for coverage to be provided. **Please contact BCBS NC** to coordinate this therapy.

| FDA Label Reference | | | | |
|---|---|---|---|---|
| **Medication** | **Indication** | **Dosing** | **HCPCS** | **Maximum Units*** |
| etranacogene dezaparvovec-drlb (Hemgenix®)<br><br>intravenous (IV) infusion | Hemophilia B (congenital Factor IX deficiency) in adults | IV: 2 x $10^{13}$ genome copies (gc) per kg of body weight | J1411 | **1** |

***Maximum units allowed for duration of approval**

**References**: all information referenced is from FDA package insert unless otherwise noted below.


1. Miesbach W, Meijer K, Coppens M, et al. Gene therapy with adeno-associated virus vector 5-human factor IX in adults with hemophilia B. Blood. 2018;131(9):1022-1031.
2. Von Drygalski A, Giermasz A, Castaman G, et al. Etranacogene dezaparvovec (AMT-061 phase 2b): normal/near normal FIX activity and bleed cessation in hemophilia B. Blood Adv. 2019;3(21):3241-3247.


**Policy Implementation/Update Information:** Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q2 annually.

October 2024: Criteria change: Updated moderately severe to severe disease criteria requirement to Factor IX baseline residual level less than or equal to 2 IU/dL (≤ 2% of normal circulating Factor IX). **Policy notification given 8/2/2024 for effective date 10/1/2024**.
April 2023: Coding update: Added HCPCS code J1411 to dosing reference table effective 4/1/2023; deleted C9399, J3490, and J3590 termed 3/31/2023.
December 2022: Original medical policy criteria issued.