# Hemgenix® (etranacogene dezaparvovec-drlb)

When requesting Hemgenix® (etranacogene dezaparvovec-drlb), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

## FDA-Approved Indication

Hemgenix is indicated for the 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.

## Coverage Guidelines

### Hemophilia B

The individual must meet all of the following criteria for authorization:

- Is male;
- Is 18 years of age or older;
- Has not received a gene therapy for hemophilia B in the past *(Note: If no claim for Hemgenix or Beqvez is present (or if claims history is not available), the prescribing physician confirms that the patient has not previously received Beqvez or Hemgenix)*;
- Has moderately severe or severe hemophilia B as evidenced by a baseline (without Factor IX replacement therapy) Factor IX level of ≤ 2% of normal;
- Meets one of the following:
    - Has a history of use of Factor IX therapy for at least 150 exposure days; OR
    - Has a history of life-threatening hemorrhage and on-demand use of Factor IX therapy was required for this life-threatening hemorrhage; OR
    - Has a history of repeated, serious spontaneous bleeding episodes and on-demand use of Factor IX therapy was required for these serious spontaneous bleeding episodes;
- Meets ALL of the following:
    - Factor IX inhibitor titer testing has been performed within the last 30 days;
    - Patient is negative for Factor IX inhibitors;
- Meets BOTH of the following:
    - Does not have an active infection with hepatitis B virus or hepatitis C virus;
    - Is not currently receiving antiviral therapy for a prior hepatitis B virus or C virus exposure;
- Does not have uncontrolled human immunodeficiency virus infection;

- Has undergone liver function testing within the last 30 days and meets all of the following:
    - Alanine aminotransferase level is ≤ 2x the upper limit of normal (ULN); AND
    - Aspartate aminotransferase level is ≤ 2x the ULN; AND
    - Total bilirubin level is ≤ 2x the ULN; AND
    - Alkaline phosphatase level is ≤ 2x the ULN ;
- Does not have evidence of advanced liver impairment and/or advanced fibrosis;
- The platelet count was ≥ 50 x $10^9$/L within the last 30 days
- Meets ONE of the following:
    - Has an estimate creatinine clearance ≥ 30 mL/min within the last 30 days;
    - Creatinine level is ≤ 2x the ULN within the last 30 days;
- The current body weight has been obtained within the last 30 days;
- Hemgenix is prescribed by a hemophilia specialist physician.

**Approval Duration:** One time (per lifetime)

## Dosing Recommendation

Hemgenix is for single-use intravenous infusion only.

The recommended dose of Hemgenix is 2 x $10^{13}$ genome copies per kilogram of body weight administered as an intravenous infusion.

## References


1. Hemgenix® intravenous infusion [prescribing information]. King of Prussia, PA; Kankakee, IL; and Lexington, MA: CSL Behring and uniQure; November 2022.
2. National Bleeding Disorders Foundation. Hemophilia B. An overview of symptoms, genetics, and treatments to help you understand hemophilia B. Available at: https://www.hemophilia.org/bleeding-disorders-a-z/types/hemophilia-b. Accessed on February 28, 2025.
3. Sidonio RF, Malec L. Hemophilia (Factor IX deficiency). *Hematol Oncol Clin N Am*. 2021;35:1143-1155.
4. Mancuso ME, Mahlangu JN, Pipe SW. The changing treatment landscape in haemophilia: from standard half-life clotting factor concentrates to gene editing. *Lancet*. 2021;397:630-640.
5. Croteau SE. Hemophilia A/B. *Hematol Oncol Clin N Am*. 2022;36:797-812.
6. Pipe SW, Leebeek FWG, Recht M, et al. Gene therapy with etranacogene dexaparvovec for hemophilia B. *N Engl J Med*. 2023;388:706-718.




  