

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 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 $\leq 2\%$ 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 both of the following:
 - Factor IX inhibitor titer testing has been performed within the last 30 days;
 - 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 $\leq 2x$ the upper limit of normal (ULN); AND
 - Aspartate aminotransferase level is $\leq 2x$ the ULN; AND
 - Total bilirubin level is $\leq 2x$ the ULN; AND
 - Alkaline phosphatase level is $\leq 2x$ the ULN ;
- Does not have evidence of advanced liver impairment and/or advanced fibrosis;
- The platelet count was $\geq 50 \times 10^9/L$ within the last 30 days;
- Meets one of the following:
 - Has an estimate creatinine clearance ≥ 30 mL/min within the last 30 days; OR
 - Creatinine level is $\leq 2x$ 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 2×10^{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; April 2026.
2. Konkle BA, Nakaya Fletcher S. Hemophilia B. 2000 Oct 2 [Updated 2025 Aug 7]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026.
3. 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 8, 2026.
4. Chowdary P, Carcao M, Kenet G, Pipe SW. Haemophilia. *Lancet*. 2025;405:736-750.
5. Pipe SW, Leebeek FWG, Recht M, et al. Gene therapy with etranacogene dezaparvovec for hemophilia B. *N Engl J Med*. 2023;388:706-718.
6. Pipe SW, Miesbach W, Recht M, et al, for the HOPE-B study group investigators. Final analysis of the study of etranacogene dezaparvovec for hemophilia B. *N Engl J Med*. 2026;394:463-474.