

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 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:
 - o Has been receiving routine prophylaxis with Factor IX therapy continuously for at least 2 months and has a history of use of Factor IX therapy for at least 150 exposure days; OR
 - o Has a history of life-threatening hemorrhage and on-demand use of Factor IX therapy was required for this life-threatening hemorrhage; OR
 - o 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;
- Factor IX inhibitor titer testing has been performed within the last 30 days;
- Does not currently have an inhibitor to Factor IX;
- Does not have a history of Factor IX inhibitors;
- Prophylactic therapy with Factor IX will not be given after Hemgenix administration once adequate Factor IX levels have been achieved (*Note: Use of episodic Factor IX therapy is acceptable for the treatment of bleeds and for surgery/procedures if needed as determined by the hemophilia specialist physician*);
- Has not received Hemgenix in the past;

- 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 (*Note: A patient testing positive for human immunodeficiency virus can still qualify for Hemgenix if controlled on antiviral therapy with CD4+ counts $\geq 200/\mu\text{L}$ or by a viral load of ≤ 200 copies/mL*);
- Has undergone liver function testing within the last 30 days and meets all of the following:
 - Alanine aminotransferase level is $\leq 2 \times$ the upper limit of normal (ULN); AND
 - Aspartate aminotransferase level is $\leq 2 \times$ the ULN; AND
 - Total bilirubin level is $\leq 2 \times$ the ULN; AND
 - Alkaline phosphatase level is $\leq 2 \times$ the ULN ;
- Does not have evidence of advanced liver impairment and/or advanced fibrosis (*Note: for example, liver elastography [e.g., ≥ 9 kPA] suggestive of or equal to METAVIR Stage 3 disease*);
- The platelet count was $\geq 50 \times 10^9/\text{L}$ within the last 30 days
- Has an estimate creatinine clearance ≥ 30 mL/min within the last 30 days;
- Creatinine level is ≤ 2 times the upper limit of normal within the last 30 days;
- Does not have another coagulation disorder, besides hemophilia B;
- 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; 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, 2024.
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.