HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Hemgenix (etranacogene dezaparvovec)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Hemgenix is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who currently use Factor IX (FIX) prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes. Hemgenix was approved by the FDA in November 2022 as the first gene therapy for hemophilia B. It is designed to deliver a copy of a gene encoding the Padua variant of human coagulation Factor IX, resulting in cell transduction and increase in circulating Factor IX activity.
Hemophilia is a disease that interferes with the normal coagulation process. Hemophilia B is less common than hemophilia A. It is caused by a faulty gene leading to a lack of clotting factor IX. Standard treatment is by replacement of the missing factor. Severe patients may require infusions 3 times per week. Successful gene therapy would obviate the need for prophylactic factor product use.
In a final analysis of the Phase 3 HOPE-B trial in 54 patients, Hemgenix reduced annualized bleed rate (ABR) by 64% and demonstrated superiority to factor prophylaxis in the lead-in period. Factor replacement use was significantly decreased from baseline and 96% were able to discontinue prophylactic infusions.

Hemgenix (etranacogene dezaparvovec) will be considered for coverage when the following criteria are met:

Hemophilia B

For **initial** authorization:

1. Member is a male at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of hemophilia B; AND
4. Member has severe or moderately severe disease as indicated by 2% or less of normal circulating factor IX (or <2 IU/dL); AND
5. Member meets one of the following
   a) Currently using Factor IX prophylaxis
   b) Has current or historical life-threatening hemorrhage
   c) Has repeated, serious spontaneous bleeding episodes; AND
6. Member's current weight is provided for dose calculation; AND
7. Member does NOT have any of the following:
   a) Factor IX inhibitors or history of inhibitors (0.6 or higher Bethesda units/mL)
   b) Active hepatitis B or C or advanced liver fibrosis
   c) HIV positive, uncontrolled on antiviral therapy
   d) Prior gene therapy.
8. **Dosage allowed/Quantity limit:** Single-use IV infusion of 2 x 10^{13} genome copies (gc) per kg

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:
1. Hemgenix is a one-time single infusion and will not be reauthorized.

HAP CareSource considers Hemgenix (etranacogene dezaparvovec) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
01/13/2023	New policy for Hemgenix created.
05/17/2024	Added guideline to reference list.

References:

1. Hemgenix [prescribing information]. uniQure, Inc.; 2022.
2. Miesbach W, Leebeek F, Recht M, et al. Final analysis from the pivotal phase 3 HOPE-B gene therapy trial: Stable steady-state efficacy and safety of etranacogene dezaparvovec in adults with severe or moderately severe hemophilia B. Haemophilia. 2022;28:25–126 (PO143).
3. Miesbach W, Meijer K, Coppens M, et al. Five Year Data Confirms Stable FIX Expression and Sustained Reductions in Bleeding and Factor IX Use Following AMT-060 Gene Therapy in Adults with Severe or Moderate-severe Hemophilia B [abstract]. *Res Pract Thromb Haemost*. 2021; 5 (Suppl 2).
4. 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. doi:10.1182/blood-2017-09-804419
5. Gomez E, Giermasz A, Castaman G, et al. Etranacogene Dezaparvovec (AAV5-Padua hFIX Variant, AMT-061), an Enhanced Vector for Gene Transfer in Adults with Severe or Moderate-severe Hemophilia B: 2.5 Year Data from a Phase 2b Trial [abstract]. *Res Pract Thromb Haemost*. 2021; 5 (Suppl 2).
6. 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 [published correction appears in Blood Adv. 2020 Aug 11;4(15):3668]. *Blood Adv*. 2019;3(21):3241-3247. doi:10.1182/bloodadvances.2019000811
7. Shah J, Kim H, Sivamurthy K, Monahan PE, Fries M. Comprehensive analysis and prediction of long-term durability of factor IX activity following etranacogene dezaparvovec gene therapy in the treatment of hemophilia B [published online ahead of print, 2022 Oct 25]. *Curr Med Res Opin*. 2022;1-11. doi:10.1080/03007995.2022.2133492
8. Institute for Clinical and Economic Review (ICER). Gene Therapy for Hemophilia B and An Update on Gene Therapy for Hemophilia A: Final Policy Recommendations. December 2022.
9. IPD Analytics. Accessed January 13, 2023.
10. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition [published correction appears in Haemophilia. 2021 Jul;27(4):699]. *Haemophilia*. 2020;26 Suppl 6:1-158. doi:10.1111/hae.14046

Effective date: 01/01/2025
Revised date: 05/17/2024