

RX.PA.083.MPC Hemgenix (etranacogene dezaparvovec)

PURPOSE

The purpose of this policy is to define the prior authorization process for Hemgenix (etranacogene dezaparvovec).

Hemgenix, an adeno-associated virus vector-based gene therapy, is indicated for the treatment of adults with hemophilia B (congenital Factor IX deficiency) who: 1) currently use Factor IX prophylaxis therapy; or 2) have current or historical life-threatening hemorrhage; or 3) have repeated, serious spontaneous bleeding episodes.

PROCEDURE

A. Initial Authorization Criteria:

**Please note that the provider must submit clinical documentation (chart notes, laboratory results and any other clinical support).*

Must meet all of the criteria listed under the respective diagnosis:

1. Hemophilia B. Approve one-time (lifetime) dose if the patient meets the following:

- Patient is male; AND
- Patient is greater than or equal to 18 years of age; AND
- Patient has moderately severe or severe hemophilia B as evidence by a baseline (without Factor IX replacement therapy) Factor IX level of $\leq 2\%$ of normal; AND
- Must meet ONE of the following:
 - Criteria 1 (A and B):
 - a)** Patient has been receiving routine prophylaxis with Factor IX therapy continuously for at least 2 months; AND
 - b)** According to the prescribing physician, the patient has a history of use of Factor IX therapy for at least 150 exposure days; OR
 - Criteria 2 (A and B):
 - a)** Patient has a history of life-threatening hemorrhage; AND
 - b)** On-demand use of Factor IX therapy was required for this life-threatening hemorrhage; OR
 - Criteria 3 (A and B):
 - a)** Patient has a history of repeated, serious spontaneous bleeding episodes; AND
 - b)** On-demand use of Factor IX therapy was required for these serious spontaneous bleeding episodes; AND
- Prescriber attests to all of the following:

- Patient will have a negative Factor IX inhibitor titer test result performed within 30 days before receipt of Hemgenix; AND
- Patients that have a positive Factor IX titer testing within the last 30 days must have a follow up negative test result obtained within 2 weeks of the original positive result; AND
- Patient does not currently have an inhibitor to Factor IX; AND
- Patient does not have a history of Factor IX inhibitors; AND
- Prescriber attests that prophylactic therapy with Factor IX will not be given after Hemgenix administration once adequate Factor IX levels have been achieved; AND
- Patient has not received Hemgenix or gene therapy in the past; AND
- Patient does not have an active infection with hepatitis B virus or hepatitis C virus; AND
- Patient is not currently receiving antiviral therapy for a prior hepatitis B virus or C virus exposure; AND
- Patient does not have uncontrolled human immunodeficiency virus; AND
- Patient has undergone a liver health assessment within the last 30 days and meets all of the following:
 - Alanine aminotransferase is ≤ 2 times the upper limit of normal; AND
 - Aspartate aminotransferase is ≤ 2 times the upper limit of normal; AND
 - Total bilirubin levels are ≤ 2 times the upper limit of normal; AND
 - Alkaline phosphatase levels are ≤ 2 times the upper limit of normal; AND
- Patient does not have evidence of advanced liver impairment and/or advanced fibrosis; AND
- Within the last 30 days, platelet counts were evaluated and were $\geq 50 \times 10^9/L$; AND
- Patient has adequate renal function as defined by meeting both of the following: an estimated creatinine clearance ≥ 30 mL/min and creatinine levels are ≤ 2 times the upper limit of normal; AND
- Prescriber attests that the member does not have another coagulation disorder, besides hemophilia B; AND
- Following Hemgenix infusion, liver enzyme testing to monitor for liver enzyme elevations will be done at least weekly for the first 3 months and periodically thereafter; AND
- Patient will undergo monitoring for Factor IX activity at least weekly for the first 3 months and periodically thereafter; AND
- Patient with preexisting risk factors (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age) for hepatocellular carcinoma will receive abdominal ultrasound screenings and be monitored at least annually for alpha fetoprotein elevations in the 5 years following receipt of Hemgenix; AND
- Must be administered at a Hemgenix approved administration center
- Must be prescribed by or in consultation with a hematologist

- B. Must be prescribed at a dose within the manufacturer’s dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.**
- C. Hemgenix will be considered investigational or experimental for any other use and will not be covered.**

Limitations:

| Length of Authorization (if above criteria met) | |
|---|--------|
| Initial Authorization | 1 dose |
| Reauthorization | N/A |

If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.

| APPLICABLE CODES: | |
|-------------------|------------------------|
| CODE | DESCRIPTION |
| J3590 | Unclassified biologics |

REFERENCES

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11. Von Drygalski A, Gomez E, Giermasz A, et al. Stable and durable factor IX levels in hemophilia B patients over 3 years post etranacogene dezaparvovec gene therapy. *Blood Adv*. 2022 Dec 9. [Online ahead of print].
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REVIEW HISTORY

| DESCRIPTION OF REVIEW / REVISION | DATE APPROVED |
|---|----------------------|
| <i>Annual Review</i> | <i>02/2026</i> |
| <i>Annual Review</i> | <i>02/2025</i> |
| <i>Annual review</i> | <i>02/2024</i> |
| <i>New Policy</i> | <i>07/2023</i> |