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 ≤ 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 ≥ 50 x 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].
12. Shah J, Kim H, Sivamurthy K, et al. Comprehensive analysis and prediction of long-term durability of factor IX activity following etranacogene dezaparvovec gene therapy in the treatment of hemophilia B. *Curr Med Res Opin*. 2022 Oct 25. [Online ahead of print].

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
New Policy	*07/2023*