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Section:	Prescription Drugs	**Effective Date:**	January 1, 2024
Subsection:	Hematological Agents	**Original Policy Date:**	December 30, 2022
Subject:	Hemgenix	**Page:**	1 of 4

Last Review Date: December 8, 2023

Hemgenix

Description

Hemgenix (etranacogene dezaparvovec-drlb)

Background
Hemgenix (etranacogene dezaparvovec-drlb) is an adeno-associated virus serotype 5 (AAV5) based gene therapy designed to deliver a copy of a gene encoding the Padua variant of human coagulation Factor IX (hFIX-Padua). Single intravenous infusion of Hemgenix results in cell transduction and increase in circulating factor IX activity in patients with Hemophilia B (1).

Regulatory Status
FDA-approved indication: 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 prophylaxis therapy, have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes (1).

Hemgenix can cause hepatotoxicity. Transaminase levels should be closely monitored weekly for 3 months after Hemgenix administration. Patients with preexisting risk factors for hepatocellular carcinoma should receive abdominal ultrasound screenings and be monitored regularly for alpha-fetoprotein (AFP) evaluations in the 5 years following administration (1).

While on Hemgenix therapy, factor IX activity and factor IX inhibitors should be regularly monitored (1).

The safety and effectiveness of Hemgenix in pediatric patients less than 18 years of age have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Hemgenix may be considered **medically necessary** if the conditions indicated below are met.

Hemgenix may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age or older

Gender assigned at birth Male

Diagnosis

Patient must have the following:

Hemophilia B

AND ALL of the following:

1. Known severe or moderately severe factor IX deficiency (≤ 2% normal circulating factor IX)
2. Patient is currently receiving factor IX prophylaxis
3. Patient has **ONE** of the following:
    a. Current or historical life-threatening hemorrhage
    b. Repeated, serious spontaneous bleeding episodes
4. Patient has received a liver health assessment including enzyme testing (ALT, AST, ALP, and total bilirubin) **AND** a hepatic ultrasound and elastography
5. Prescribed by or recommended by a hematologist or a prescriber who specializes in hemophilia B

AND NONE of the following:

1. History of factor IX inhibitors
2. Positive factor IX inhibitor screen results of ≥ 0.6 Bethesda Units (BU) using the Nijmegen-Bethesda assay
3. Positive HIV serological test not controlled with anti-viral therapy
4. Active hepatitis B and/or hepatitis C infection
5. Prior gene therapy for hemophilia B or under consideration for treatment with another gene therapy for hemophilia B

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity One infusion (only one PA approval for one infusion per lifetime)

Prior – Approval *Renewal* Limits

None

Rationale

Summary

Hemgenix is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B who currently use factor IX prophylaxis therapy, have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes. The safety and effectiveness of Hemgenix in pediatric patients less than 18 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Hemgenix while maintaining optimal therapeutic outcomes.

References

1. Hemgenix [package insert]. Lexington, MA: uniQure, Inc.; November 2022.

Policy History

Date	Reason
December 2022	Addition to PA
January 2023	Revised to align with association policy. Added options for patient to be receiving Factor IX prophylaxis, have a life-threatening hemorrhage, or bleeding episodes. Added requirement for a liver health assessment including enzyme testing. Added requirement for a consultation with a hepatologist in certain scenarios. Added that the medication must be prescribed by or recommended by a hematologist or prescriber who specializes in hemophilia B
March 2023	Annual review
April 2023	Revised to align with association policy. Added male gender requirement. Rearranged requirements so all patients must be receiving factor IX prophylaxis. Removed requirement for patient to receive >150 exposure days of treatment with factor IX protein. Require all patients to have a hepatic ultrasound and elastography. Removed requirement for a hepatologist consultation
June 2023	Annual review
December 2023	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 8, 2023 and is effective on January 1, 2024.