MVP Health Care Medical Policy

Medicare Part B: Hemophilia Gene Therapy

Type of Policy: Drug Therapy
Prior Approval Date: 10/01/2024
Approval Date: 07/01/2025
Effective Date: 09/01/2025
Related Policies: Hemophilia Factor

Refer to the MVP Medicare website for the Medicare Part D formulary and Part D policies.

Refer to relevant CMS LCDs/NCDs/Policy Articles for most up to date Medicare Part B guidance if available

Drugs Requiring Prior Authorization under the medical benefit

J1411 Hemgenix (injection, etranacogene dezaparvovec-drlb)

J1412 Roctavian (injection, valoctocogene roxaparvovec)

Overview/Summary of Evidence

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 or have current/historical life-threatening hemorrhage or have repeated serious spontaneous bleeding episodes.
Hemgenix is designed to deliver a copy of a gene encoding the Padua variant of human coagulation Factor IX (hFIX-Padua). Hemgenix infusion results in cell transduction and increase in circulating Factor IX activity in patients with Hemophilia B.

Roctavian is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with **severe hemophilia A** (congenital factor VIII deficiency with factor VIII activity <1IU/dL) without pre-existing antibodies to adeno-associated virus

serotype 5 (AAV5). Roctavian is designed to introduce a functional copy of a transgene encoding the B-domain deleted SQ form of human coagulation factor VIII (hFVIII-SQ). Transcription of this transgene occurs within the liver, using the liver-specific promotor, which results in the expression of hFVIII-SQ. The expressed hFVIII-SQ replaces the missing coagulation factor VIII needed for effective hemostasis.

Indications/Criteria

A. Hemophilia A

Roctavian may be considered for coverage when **ALL** of the following criteria is met:

- Chart notes documenting that member has a confirmed diagnosis of severe hemophilia A (hereditary factor VIII deficiency with factor VIII activity <1IU/dL).
- Current chart notes documenting the **ALL** of the following tests:
    - No pre-existing antibodies to AAV5 as demonstrated using FDA approved companion diagnostic
    - Negative factor VIII inhibitor titer testing
    - Liver function tests [alanine aminotransferase (ALT), aspartate aminotransferase (AST), gamma-glutamyl transferase (GGT), alkaline phosphatase (ALP), total bilirubin and international normalized ration (INR)]
    - Ultrasound or laboratory assessments for liver fibrosis
        - See Exclusions section
- Provider attestation
    - Indicating evaluation for thrombosis and cardiovascular risk factors has been completed and will be monitored after Roctavian infusion.
    - For members with pre-existing risk factors for hepatocellular carcinogenicity (cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), advanced age), regular (annual) monitoring liver ultrasounds and alpha-fetoprotein testing following administration

Roctavian will be approved as a **one-time dose**. Requests for replacement due to lost or damaged product will not be covered. Coverage is contingent on eligibility at the time of infusion.

B. Hemophilia B

Hemgenix may be considered for coverage when **ALL** of the following criteria is met:

- Chart notes documenting that member has a confirmed diagnosis of moderately severe or severe hemophilia B (hereditary factor IX deficiency)
- Current chart notes documenting the **ALL** of the following tests:
    - Negative factor IX inhibitor titer testing
        - If initial test is positive, there must be documentation of a re-test within 2 weeks
    - Documentation of liver health assessments including:
        - Enzyme testing [alanine aminotransferase (ALT), aspartate aminotransferase (AST), alkaline phosphatase (ALP) and total bilirubin)
        - Hepatic ultrasounds and elastography
- Current chart notes documenting one of the following:
    - Current use of Factor IX prophylaxis **OR**
    - Member has a current or historical life-threatening hemorrhage **OR**
    - Member has had repeated, serious spontaneous bleeding episodes
- Provider attestation
    - For members with pre-existing risk factors for hepatocellular carcinogenicity (cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), advanced age), regular (annual) monitoring liver ultrasounds and alpha-fetoprotein testing following administration
    - Transaminase levels will be monitored once per week for 3 months after administration
    - Factor IX activity levels will be monitored regularly after Hemgenix administration

Hemgenix will be approved as a **one-time dose**. Requests for replacement due to lost or damaged product will not be covered. Coverage is contingent on eligibility at the time of infusion.

Exclusions

- Previous gene therapy treatment
- Member is biologically female
- Indication, age, dose, frequency of dosing, and/or duration of therapy outside of FDA approved package labeling

- Roctavian
    - Member has known significant hepatic fibrosis (stage 3 or stage 4 on the Batts-Ludwig scale or equivalent)
    - Member has mannitol hypersensitivity
    - Active or uncontrolled infection (including chronic active hepatitis B)
    - Positive test for antibodies to AAV5
    - Positive test for factor VIII inhibitors
- Hemgenix
    - Member has active hepatitis B or C infection
    - Member has uncontrolled HIV infection
    - Positive initial test and re-test results for human factor IX inhibitors

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References

1. U.S Food and Drug Administration. List of Cleared or Approved Companion Diagnostic Devices (In Vitro and Imaging Tools). Content current as of 08/03/2023. Accessed 08/03/2023. List of Cleared or Approved Companion Diagnostic Devices (In Vitro and Imaging Tools) | FDA

2. Roctavian (valotocogene roxaparvovec-rvox) suspension for intravenous infusion. BioMarin Pharmaceutical Inc. Novato CA. August 2023. 78bf2bcb-7068-4774-b962-a35c53704fc1_source__v.pdf (d34r3hkxgxjdtw.cloudfront.net)

3. Hemgenix (etranacogene dezaparvovec-drlb) suspension for intravenous infusion. CSL Behring LLC. King of Prussia, PA. November 2022. 2022-313 HEMGENIX.indd (cslbehring.com)

4. HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients CTG Labs - NCBI. (n.d.). Clinicaltrials.gov. Last updated: 2024-07-30