Pharmacy Management Drug Policy

SUBJECT: Hemophilia Gene Therapies
POLICY NUMBER: PHARMACY-109
EFFECTIVE DATE: 02/2023
LAST REVIEW DATE: 02/12/2026

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Hemophilia is an inherited, lifelong bleeding disorder caused by deficiency of coagulation factors. The blood fails to clot which can result in bleeding into soft tissue, joints, and internal organs. It can also cause severe bleeding and death in trauma from intracranial bleeding. Hemophilia is an X-linked recessive disease that presents almost exclusively in male children of female carriers. The two most common types of hemophilia are Hemophilia A, which is a lack of Factor VIII and Hemophilia B, which is a lack of Factor IX.

There are varying severities of both hemophilia A and B depending upon the level of factor produced by the patient. Patients with severe hemophilia frequently experience bleeding even in the absence of trauma. Patients with moderate hemophilia experience less bleeding, and mild hemophilia patients usually experience bleeding only after obvious trauma. The severity classification system is based on the patient's factor activity level:

Disease Severity	Clotting Factor Level
Severe	< 1 IU/dl or < 1% of normal
Moderate	1-5 IU/dl or 1-5% of normal
Mild	5-40 IU/dl or 5 to < 40% of normal

The current treatment for both hemophilia A and B is to replace the deficient coagulation factor either through episodic (on demand) treatment which is replacement factor given at the time of bleeding or through continuous prophylaxis which is replacement factor given to prevent bleeding. Hemgenix is the first gene therapy indicated for use in adults with hemophilia B. Factor IX replacement therapies, given intravenously, are used for the management of bleeding and include recombinant extended half-life products (i.e., Alprolix®, Idelvion®, Rebinyn®), recombinant standard half-life products (i.e., BeneFIX ®, Ixinity®, Rixubis®), and plasma-derived products (i.e., AlphaNine® SD, Mononine®, and Profilnine®). Such therapies may be given routinely as prophylaxis to prevent bleeds, on demand for the treatment of bleeding, or prior to medical or dental procedures associated with a high risk of bleeding. When used for routine prophylaxis, extended-half-life Factor IX products are usually administered once weekly or once every other week.

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

Roctavian (valoctocogene roxaparvovec-rvox) is an adeno-associated virus serotype 5 (AAV5) based gene therapy designed to provide a copy of the B-domain–deleted human coagulation Factor VIII gene. A single intravenous infusion of Roctavian results in cell transduction and an increase in circulating factor VIII activity in adults with severe hemophilia A.

POLICY:

Hemgenix (etranacogene dezaparvovec-drlb)) – Medical benefit
1. Must be prescribed by or in consultation with a hematologist at a Hemophilia Treatment Center (HTC) **AND** 2. The patient must be 18 years of age or older **AND** 3. The patient must have a diagnosis of congenital hemophilia B with factor IX deficiency ≤2% of normal circulating factor IX **AND** 4. There must be documentation that the patient has current or historical life-threatening hemorrhages, or have repeated, serious spontaneous bleeding episodes despite continuous routine prophylaxis with factor IX replacement therapy **AND** a. Patients on prophylactic factor IX therapy must have had a minimum of 150 exposure days **AND** b. Use of Hemgenix due to convenience will not be considered medically necessary and will not be authorized **AND** 5. Documentation must be submitted confirming that the patient was screened for neutralizing antibodies (NAbs) against adeno-associated viral vector serotype 5 (AAV5) **AND** 6. Documentation must be submitted confirming that the patient does not have active factor IX inhibitors, or a history of factor IX inhibitors **AND** 7. Retreatment with etranacogene dezaparvovec-drlb (Hemgenix) has not been proven to be safe and effective. Retreatment will be considered investigational when FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered **AND** 8. Patients with any of the following will not be eligible for coverage (documentation, including laboratory results [taken within the past 3 months], is required): a. An active Hepatitis B or C infection b. Uncontrolled human immunodeficiency virus (HIV) (defined as a CD4+ count ≤ 200/μL) c. Thrombocytopenia (defined as a platelet count below 50 × 109/L) d. Advanced liver disease (suggestive of or equal to METAVIR Stage 3 disease, e.g., a FibroScan™ score of ≥9 kPa is considered equivalent) e. A known coagulation disorder other than hemophilia B 9. Approval will be for 6 months to allow one-time administration. 10. See prescribing information for approved dosing.

Roctavian (valoctocogene roxaparvovec-rvox)-Medical
1. Must be prescribed by or in consultation with a hematologist at a Hemophilia Treatment Center (HTC) **AND** 2. The patient must be 18 years of age or older **AND** 3. The patient must have a diagnosis of severe hemophilia A (congenital factor VIII (FVIII) deficiency with FVIII activity less than 1 IU/dL **AND** 4. There must be documentation that the patient has current or historical life-threatening hemorrhages, or have repeated, serious spontaneous bleeding episodes despite continuous routine prophylaxis with factor VIII replacement therapy **AND** 5. Documentation must be submitted confirming that the patient does not have neutralizing antibodies (NAbs) against adeno-associated viral vector serotype 5 (AAV5) **AND** 6. Documentation must be submitted confirming that the patient does not have active factor VIII inhibitors, or a history of factor VIII inhibitors **AND** 7. Use of Roctavian due to convenience will not be considered medically necessary and will not be authorized **AND** 8. Retreatment with valoctocogene roxaparvovec-rvox (Roctavian) has not been proven to be safe and effective. Retreatment will be considered investigational when FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered **AND** 9. Patients with any of the following will not be eligible for coverage (documentation, including laboratory results [taken within the past 3 months], is required): a. An active Hepatitis B or C infection b. Uncontrolled human immunodeficiency virus (HIV) (defined as a CD4+ count ≤ 200/μL) c. Thrombocytopenia (defined as a platelet count below 50×10^9/L) d. Advanced liver disease (suggestive of or equal to METAVIR Stage 3 disease, e.g., a FibroScan™ score of ≥9 kPa is considered equivalent) e. A known coagulation disorder other than hemophilia A 10. Approval will be for 6 months to allow one-time administration. 11. See prescribing information for approved dosing.

POLICY GUIDELINES:

1. Prior-authorization is contract dependent.
2. Clinical documentation must be submitted for each request (initial and recertification) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments/treatment history, diagnostic testing, laboratory test results, genetic testing/biomarker results, and imaging.
    - Continued approval at time of recertification will require documentation that the drug is providing ongoing benefit to the patient in terms of improvement or stability in disease state or condition. Such documentation may include progress notes, imaging or laboratory findings, and other objective or subjective measures of benefit which support that continued use of the requested product is medically necessary. Also, ongoing use of the requested product must continue to reflect the current policy's preferred formulary.
3. All non-FDA approved indications for Hemgenix will be evaluated using off label policy criteria.
4. Dose and frequency should be in accordance with the FDA label or recognized compendia (for off-label uses). When services are performed in excess of established parameters, they may be subject to review for medical necessity.
5. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
6. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria

outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. Indications that have not been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan (which may include review per the Health Plan's Off-Label Use of FDA Approved Drugs policy). Step therapy requirements may be imposed in addition to LCD/NCD requirements.

7. Not all contracts cover all Medical Infusible drugs. Refer to specific contract/benefit plan language for exclusions of Injectable Medications.
8. All requests will be reviewed to ensure they are being used for an appropriate indication and may be subject to an off-label review in accordance with our Off-Label Use of FDA Approved Drugs Policy (Pharmacy-32).
9. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
10. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract. CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.

Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:

Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).

HCPCS: Hemgenix (J1411), Roctavian (J1412)

UPDATES:

Date	Revision
02/12/2026	P&T Committee Review & Approval
11/20/2025	Revised
11/19/2025	Revised
04/01/2025	Revised
03/06/2025	Revised
02/06/2025	P&T Committee Review & Approval
12/15/2024	Revised

09/13/2024	Revised
08/20/2024	Revised
06/20/2024	Revised
03/13/2024	Revised
02/08/2024	Reviewed / P&T Committee Approval
02/2023	Revised
02/2023	P&T Committee Approval

REFERENCES:

1. Hemgenix™. Package insert. CSL Behring;2022.
2. Institute for Clinical and Economic Review, 2022, Evidence Report – Hemophilia A & B
3. Roctavian™. Package insert. BioMarin;2023.