DR.015.B ROCTAVIAN™ (valoctocogene roxaparvovec-rvox)

Original Implementation Date : 05/15/2024
Version [B] Date : 05/20/2026
PARP Approved Date: 04/15/2026
Last Reviewed Date: 5/20/2026

PRODUCT VARIATIONS

This policy applies to all Jefferson Health Plans/Health Partners Plans lines of business unless noted below.

Gene therapy is a benefit exclusion for Individual and Family (ACA) product lines and therefore, non-covered.

POLICY STATEMENT

The plan considers ROCTAVIAN™ (valoctocogene roxaparvovec-rvox) medically necessary for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity < 1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA-approved test when the prior authorization criteria listed in the policy are met. The plan considers the use of ROCTAVIAN™ experimental and investigational for all other indications.

FDA APPROVED INDICATIONS

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 < 1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5.

OFF-LABEL USE

N/A

PRIOR AUTHORIZATION CRITERIA

INITIAL CRITERIA

*AUTHORIZATION DURATION: IF **ALL CRITERIA MET**, APPROVE FOR 1 DOSE FOR 1 MONTH*

1. The patient is 18 years of age or older.
2. The patient was assigned male at birth.
3. Prescriber is or in consultation with a hematologist or a prescriber who specializes in hemophilia A.
4. The dose prescribed is consistent with U.S. Food and Drug Administration (FDA) approved package labeling, nationally recognized compendia, or peer-reviewed medical literature.
5. Patients have a diagnosis of severe hemophilia A as defined by a factor VIII levels less than 1% of normal factor VIII (< 0.01 IU/mL, < 1 IU/dL).
6. Patients have an FDA-approved test showing NO detectable pre-existing antibodies to adenoassociated virus 5 (AAV5).
7. Patient does not have prior or active factor VIII inhibitors (inhibitor titer must be less than 0.6 Bethesda Units {BU} using the Nijmegen-Bethesda assay).
8. Patients have received an ultrasound and elastography or laboratory assessments for liver fibrosis.
9. Patients have a history of Factor VIII therapy for at least 150 exposure days.
10. Patients have not received treatment with the requested medication previously.
11. Patients do not have a known hypersensitivity to mannitol.
12. Patients have documentation of a negative hepatitis B and hepatitis C infection.
13. Patients do not have a history of thrombosis or thrombophilia.
14. Provider attestation that member has been counseled regarding the risks of alcohol consumption and use of concomitant hepatotoxic medications after receiving Roctavian™ and member agrees to abstain from alcohol consumption for at least 1 year following infusion.

15. Provider attestation that member has been counseled that for a period of up to 6 months after administration of Roctavian™ a male of reproductive potential (and his female partner) should prevent or postpone pregnancy by utilizing an effective form of contraception; a male should not donate semen.

16. Provider attestation that after the gene therapy regular prophylactic therapy will be discontinued following an appropriate time for FVIII to reach therapeutic levels. (Use of episodic Factor VIII therapy is acceptable for the treatment of bleeds and for surgery/procedures if needed as determined by the hemophilia specialist physician).

RENEWAL CRITERIA

RENEWAL CRITERIA

The plan considers continued use of ROCTAVIAN™ experimental and investigational as the safety and efficacy beyond one dose has not been studied. Approval is limited to one treatment course per lifetime.

DOSAGE AND ADMINISTRATION

Per package labeling.

RISK FACTORS/SIDE EFFECTS

Per package labeling.

MONITORING

Per package labeling.

BLACK BOX WARNING

N/A

BACKGROUND

Hemophilia A is an X-linked recessive bleeding disorder caused by a deficiency of coagulation factor VIII (hemophilia A), factor IX (hemophilia B), or factor XI (hemophilia C). It is estimated about 400 babies per year are born with hemophilia A and affects 1 in 5,000 male births. Most are inherited cases, diagnosed at a young age, although some rare cases are developed later in life.

Clinical Manifestations Patients with hemophilia A who produce less than 1% of normal levels of factor VIII have the most severe form of the condition, accounting for roughly half of all hemophilia A patients (FDA 2022). Hemarthrosis, a hallmark of severe hemophilia, is a major cause of disability. Recurrent bleeds lead to joint contractures, and pseudotumors, resulting in chronic pain, disability, and a diminished quality of life.

Current standard of care: (Clotting Factor Replacement Therapy) remains the main treatment for severe Hemophilia A. These treatments are used during bleeding episodes or prophylactically to prevent bleeds.

Treatment History: Developed by BioMarin Pharmaceutical for hemophilia A, received orphan drug status from the FDA in 2016 and breakthrough therapy designation in 2017. It faced initial FDA rejection due to durability concerns but was approved in the EU in August 2022 and in the US in June 2023, targeting adults with severe hemophilia A lacking antibodies to AAV5 virus.

CODING

Note*:* The Current Procedural Terminology (CPT®), Healthcare Common Procedure Coding System (HCPCS), and the 10th revision of the International Statistical Classification of Diseases and Related Health Problems (ICD-10) codes that *may* be listed in this policy are for reference purposes only. Listing of a code in this policy does not imply that the service is covered and is not a guarantee of payment. Other policies and coverage guidelines may apply. When reporting services, providers/facilities should code to the highest level of specificity using the code that was in effect on the date the service was rendered. This list may not be all inclusive.

CPT® is a registered trademark of the American Medical Association.

CPT Code	Description
N/A	N/A

HCPCS Code	Description
J1412	Injection, valoctocogene roxaparvovec-rvox

ICD-10 Codes	Description
D66	• Classic Hemophilia • Deficiency Factor VIII • Hemophilia A • Hemophilia NOS • Hereditary Factor VIII deficiency

DISCLAIMER

Approval or denial of payment does not constitute medical advice and is neither intended to guide nor influence medical decision making. Policy Bulletins are developed to assist in administering plan benefits and constitute neither offers of coverage nor medical advice. This Policy Bulletin may be updated and therefore is subject to change.

For Health Partners Plans Medicaid and Health Partners Plans Chip products: Any requests for services that do not meet criteria set in PARP will be evaluated on a case-by-case basis.

POLICY HISTORY

This section provides a high-level summary of changes to the policy since the previous version.

Summary	Version	Version Date
2026 Annual Review. Revisions made to dosage and administration/risk factors and side effects/monitoring.	B	05/20/2026
2025 Annual Review. References updated.	A	05/15/2024
New Policy.	A	05/15/2024

REFERENCES

1. Centers for Disease Control and Prevention. Treatment of Hemophilia. 20243 Nov 13 https://www.cdc.gov/hemophilia/treatment/?CDC_AAref_Val=https://www.cdc.gov/ncbddd/hemophilia/treatment.html. Accessed March 14, 2024.

2. Roctavian™ [package insert]. Novato, CA; BioMarin Pharmaceutical, Inc: June 2023.

3. BioMarin. Is Roctavian™ Right for You? Roctavian™. Available from: https://www.roctavian.com/en-us/is-roctavian-right-for-you. Accessed March 14, 2024.

4. Centers of Disease Control and Prevention. Data & Statistics on Hemophilia. 2024 May15. Available from: https://www.cdc.gov/hemophilia/data-research/?CDC_AAref_Val=https://www.cdc.gov/ncbddd/hemophilia/data.htmlAccessed March 14, 2024.

5. National Library of Medicine. A Study to Evaluate the Efficacy and Safety of Valoctocogene Roxaparvovec, in Hemophilia A Patients (GenEr8-1). NCT02576795. https://clinicaltrials.gov/ct2/show/NCT02576795. Accessed March 14, 2024.