

Corporate Medical Policy: Valoctocogene roxaparvovec-rvox (Roctavian®) “Notification”

POLICY EFFECTIVE APRIL 1, 2026

Restricted Product(s):

- valoctocogene roxaparvovec-rvox (Roctavian®) intravenous infusion for administration by a healthcare professional

FDA Approved Use:

- 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 (AAV5) detected by an FDA-approved test

Criteria for Medical Necessity:

The restricted product(s) may be considered medically necessary when the following criteria are met:

1. The patient is 18 years of age or older; **AND**
2. The patient is male; **AND**
3. The patient has a diagnosis of **hemophilia A** (also known as congenital factor VIII deficiency, or classic hemophilia) [**medical record documentation required**]; **AND**
4. The patient has severe disease, defined by factor VIII baseline residual level less than or equal to 1 IU/dL [**medical record documentation required, including lab test**]; **AND**
5. The patient does NOT have active inhibitors to factor VIII [**medical record documentation required**]; **AND**
6. The patient does NOT have a history of inhibitors to factor VIII [**medical record documentation required**]; **AND**
7. The patient has a Nijmegen modified Bethesda assay of less than 0.6 Bethesda Units (BU) on two consecutive occasions at least one week apart within the past 12 months [**medical record documentation required, including test results**]; **AND**
8. The patient is NOT on any bypassing agents (i.e., FEIBA, NovoSeven, SevenFact) [**medical record documentation required**]; **AND**
9. The patient is on prophylactic therapy with a factor VIII agent (e.g., Advate, Eloctate, Recombinate) AND has had a minimum of 150 exposure days [**medical record documentation required**]; **AND**
10. The patient does NOT have anti-AAV5 (adeno-associated virus serotype 5) antibodies [**medical record documentation required, including test results within the past 3 months**]; **AND**
11. ONE of the following:
 - a. The patient is NOT HIV positive [**medical record documentation required, including lab tests within the past 3 months**]; **OR**
 - b. The patient is HIV positive AND is well controlled (i.e., viral load within the past 12 months less than 1000 copies/mL) [**medical record documentation required, including lab results within the past 12 months**]; **AND**
12. The patient does NOT have another immunosuppressive disorder [**medical record documentation required**]; **AND**

13. The patient's hepatitis B surface antigen is negative **[medical record documentation required, including lab tests within the past 3 months]; AND**
14. ONE of the following:
 - a. The patient's hepatitis C virus (HCV) antibody is negative **[medical record documentation required, including lab tests within the past 3 months]; OR**
 - b. The patient's HCV antibody is positive AND the patient's HCV RNA is negative **[medical record documentation required, including lab tests within the past 3 months]; AND**
15. The patient does NOT have any other active infection **[medical record documentation required]; AND**
16. The patient does NOT have significant (i.e., stage 3 or 4) liver fibrosis or cirrhosis of any etiology **[medical record documentation required]; AND**
17. The patient does NOT have significant liver dysfunction as defined by abnormal elevation of any of the following **[medical record documentation required, including lab tests within the past 3 months]:**
 - a. ALT (alanine transaminase) 3 times the upper limit of normal; **OR**
 - b. Bilirubin above 3 times the upper limit of normal; **OR**
 - c. Alkaline phosphatase above 3 times the upper limit of normal; **OR**
 - d. INR (international normalized ratio) greater than or equal to 1.5; **AND**
18. The patient does NOT have creatinine greater than or equal to 1.4 mg/dL **[medical record documentation required, including lab tests within the past 3 months]; AND**
19. The patient does NOT have current evidence of active malignancy, except for non-melanoma skin cancer **[medical record documentation required]; AND**
20. The patient does NOT have a history of hepatic malignancy **[medical record documentation required]; AND**
21. The patient does NOT have evidence of any bleeding disorder that is not related to hemophilia A **[medical record documentation required]; AND**
22. The patient has NOT had any previous gene therapy, including the requested agent **[medical record documentation required]; AND**
23. The requested dose is within FDA labeled dosing for the requested indication, and the requested quantity does NOT exceed the maximum units allowed for the duration of approval (see table below) **[medical record documentation required].**

Duration of Approval: 180 days (one treatment course per lifetime)

****Please note, for certain identified gene and cellular therapies such as valoctocogene roxaparvovec-rvox (Roctavian[®]), when coverage is available and the individual meets medically necessary criteria, distribution from a specialty pharmacy provider due to cost (distribution channel restriction) may be required in order for coverage to be provided. **Please contact Blue Cross NC** to coordinate this therapy.**

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FDA Label Reference

Medication	Indication	Dosing	HCPCS	Maximum Units*
valoctocogene roxaparvovec-rvox (Roctavian®) intravenous (IV) infusion	Severe hemophilia A (congenital factor VIII deficiency with factor VIII activity < 1 IU/dL) in adults	IV: One-time, single-dose of 6 x 10 ¹³ vector genomes (vg) per kg of body weight	J1412	One-time, single-dose infusion per lifetime

***Maximum units allowed for duration of approval**

Other revenue codes that may be applicable to this policy: 0891, 0892

References: all information referenced is from FDA package insert unless otherwise noted below.

1. Mahlangu J, Kaczmarek R, von Drygalski A, et al. Two-year outcomes of valoctocogene roxaparvovec therapy for hemophilia A. *N Engl J Med.* 2023;388(8):694-705.
2. Ozelo MC, Mahlangu J, Pasi KJ, et al. Valoctocogene roxaparvovec gene therapy for hemophilia A. *N Engl J Med.* 2022;386(11):1013-1025.
3. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. *Haemophilia.* 2020;26(Suppl 6):1-158.

Policy Implementation/Update Information: Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q2 annually.

April 2026: Coding change: Added the following applicable revenue codes associated with policy HCPCS code(s): 0891 (Special Processed Drugs – FDA Approved Cell Therapy) and 0892 (Special Processed Drugs – FDA Approved Gene Therapy). **Policy notification given 2/1/2026 for effective date 4/1/2026.**

January 2026: Criteria update: Adjusted maximum units to remove unit value and specify one-time, single-dose infusion per lifetime for clarity.

September 2025: Criteria change: Adjusted maximum units according to coding unit definition (1 unit = per ml, containing nominal 2 x 10¹³ vg) for clarity.

January 2024: Coding change: Added HCPCS code J1412 to dosing reference table effective 1/1/2024; deleted C9399, J3490, and J3590
termed 12/31/2023.

July 2023: Original medical policy criteria issued.