Roctavian (valoctocogene roxaparvovec-rvox)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Roctavian (valoctocogene roxaparvovec-rvox) policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Roctavian is indicated for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity < 1 IU/dL) without antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test.

POLICY

Required Documentation

Submission of the following information is necessary to initiate the prior authorization review:

A. Chart notes, medical records, or lab results documenting all of the following:
    1. Severe factor VIII deficiency (factor VIII activity < 1 IU/dL)
    2. Absence of pre-existing antibodies to the adeno-associated virus serotype 5 (AAV5) capsid
    3. Absence of factor VIII inhibitor confirmed by a Bethesda assay (lab test results required).

Prescriber Specialty

This medication must be prescribed by or in consultation with a hematologist.

Criteria for Initial Approval

Hemophilia A

Authorization of 1 month for one dose total may be granted for treatment of severe hemophilia A when ALL of the following criteria are met:

A. Member is 18 years of age or older
B. Member was assigned male at birth
C. Member has been receiving factor VIII prophylaxis or emicizumab (Hemlibra) for at least 12 months
D. Member has severe hemophilia A as defined by a factor VIII (FVIII) activity level ≤1% (≤1 IU per deciliter)
E. Member does not have pre-existing antibodies to AAV5 confirmed by an FDA-approved test (e.g., AAV5 DetectCDx™)
F. Member 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).
G. Member has received a liver health assessment including enzyme testing [alanine aminotransferase (ALT), aspartate aminotransferase (AST), alkaline phosphatase (ALP), and total bilirubin] AND a hepatic ultrasound and elastography
H. Member has not received treatment with the requested medication previously
I. Medication is being prescribed by or in consultation with a hematologist or a prescriber who specializes in hemophilia A
J. Member is HIV negative
K. Member does not have an active hepatitis B and/or hepatitis C infection
L. Member does not have a history of thrombosis or thrombophilia
M. 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
N. Provider attestation of discontinuation of regular prophylactic therapy following appropriate time frame for FVIII levels to reach therapeutic levels after steady state, after member has received gene therapy

Continuation of Therapy

Repeat treatment of Roctavian for any indication is considered investigational, as the safety and efficacy beyond one dose has not been studied. Approval is limited to one treatment course per lifetime.

Roctavian is considered **not medically necessary** for members who do not meet the criteria set forth above.

The Evio platform is a provider portal that is used to capture clinical outcome information for patients on select high-cost treatments, such as gene and cellular therapies. If a patient meets medical necessity as defined by this policy and is approved for treatment, the requesting physician must attest to providing clinical outcome information within the Evio provider portal at the requested cadence.

Dosing and Administration

The recommended dose is 6×10^{13} vector genomes per kilogram of body weight.
Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits

Roctavian approvals will be limited to one treatment per lifetime.

Other Considerations

A. Where feasible, the member's vaccinations should be up to date with all age-appropriate vaccines before valoctocogene roxaparvovec-rvox administration.

B. Where feasible, the member should receive periodical monitoring for hepatotoxicity, hepatocellular carcinogenicity, and Factor VIII activity/inhibitors.
C. Where feasible, it is recommended that the prescriber consult with a hepatologist if there is significant concern regarding the member's liver function.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- J1412 – Injection, valoctocogene roxaparvovec-rvox, per ml, containing nominal 2 x 10^13 vector genomes (effective 1/1/24)
- C9399 – Unclassified drugs or biologicals
- J3590 – Unclassified biologicals

REFERENCES

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

POLICY HISTORY

Policy #: 05.05.12
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Reviewed: October 2025
Revised:
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