

EVH Clinical Guideline 5083.CC for Roctavian (valoctocogene roxaparvovec-rvox)

Guideline Number: EVH_CG_5083.CC	<u>Applicable Codes</u>	
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STATEMENT

General Information

- *It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity and approval by the Medical Policy Committee.*
- *If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.*

Purpose

The purpose of this guideline is to define the prior authorization process for the following drug: Roctavian (valoctocogene roxaparvovec-rvox).

Scope

This guideline applies to all practitioners who are involved in providing the requested drug. This guideline is specific to the Health Plan's medical benefit.

INITIAL REVIEW CRITERIA

The request must meet all of the criteria listed below.

- Must be age 18 years or older
- Must be male sex assigned at birth
- Must be prescribed by, or in consultation with, a hematologist
- Must have a diagnosis of severe hemophilia A (congenital factor VIII deficiency), confirmed by a factor VIII activity level of < 1 IU/dL (in absence of exogenous factor VIII)
- Must have documentation of BOTH the following:
 - The member has been receiving prophylactic FVIII replacement therapy over the last 12 months prior to the request AND
 - The member has been exposed to FVIII therapy for a minimum of 150 exposure days (i.e., has received a dose of FVIII therapy on at least 150 days in their lifetime)
- Must have documentation of ALL the following testing results, **collected within the last 3 months:**
 - Liver function tests <1.25 upper limit of normal
 - ALT/AST
 - Bilirubin

- Alkaline phosphatase
- INR <1.4
- Negative Hepatitis B surface antigen*
- Negative Hepatitis C virus antibody **OR** HCV antibody positive AND HCV RNA negative*
- Negative HIV test **OR** HIV viral load <200 copies/mL and the member is on anti-retroviral therapy
- Platelet count >100 x 10⁹/L
- Serum creatinine <1.5mg/dL
- *If the member has a history of HBV or HCV exposure, the member must NOT be currently using antiviral therapy to treat
- Must have documentation of ALL the following results of testing/screening:
 - Negative for pre-existing antibodies to adeno-associated virus serotype 5 (AAV5) capsid, as determined by an FDA-approved or CLIA-compliant test
 - Negative for factor VIII inhibitors [must have a Bethesda titer of <0.6 Bethesda units (BU) for FVIII]
 - Liver ultrasound/biopsy confirming no fibrosis stage 3 or 4 and no cirrhosis
- Must have documentation or an attestation from the prescriber on all the following:
 - The member does not have a history of thrombosis or thrombophilia
 - The member does not have a hypersensitivity to mannitol
 - The member does not have active malignancy
 - The member does not have an active infection
 - The member will not receive live vaccines during treatment
 - The member has not previously been treated with Roctavian or another adeno-associated virus (AAV5)-vector-based gene therapy for the management of hemophilia A
 - Following infusion, the member's liver enzymes and factor VIII activity will be monitored weekly for at least 26 weeks and periodically thereafter
- Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc.) listed in the FDA approved labeling

REAUTHORIZATION CRITERIA

Roctavian is not eligible for reauthorization as only a single course is allowed per member's lifetime.

APPROVAL DURATIONS

Initial Authorization	1 time approval (up to 6 months)
Reauthorization	N/A

CODING AND STANDARDS

Codes

Code	Brand	Description
J1412	Roctavian	Injection, valoctocogene roxaparvovec-rvox, per ml, containing nominal 2×10^{13} vector genomes

Applicable Lines of Business

☐	CHIP (Children's Health Insurance Program)
☐	Commercial
☐	Exchange/Marketplace
☒	Medicaid
☐	Medicare Advantage

BACKGROUND

The purpose of this policy is to define the prior authorization process for Roctavian (valoctocogene roxaparvovec-rvox) 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.

Definitions

Exposure Day (ED) – A unit of time (1 day) in which replacement therapy is given to a patient. In patients with severe hemophilia A, inhibitors develop after a median FVIII treatment time of 10–15 days. During this period, regular screening for inhibitor development is recommended. After 50–75 EDs, the cumulative incidence of inhibitors reaches a plateau. The incidence rate of inhibitor development in patients with hemophilia A who have been previously treated for at least 150 EDs has been estimated to be approximately 2–5 per 1000 patient-years¹

POLICY HISTORY

Date	Summary
July 2026	Updated approval duration to 6 months
April 2024	New guideline

LEGAL AND COMPLIANCE

Guideline Approval

Committee

Reviewed / Approved by Evolent Administrative Services Medical Policy Committee

Disclaimer

Evolent Clinical Guidelines do not constitute medical advice. Treating health care professionals are solely responsible for diagnosis, treatment, and medical advice. Evolent uses Clinical Guidelines in accordance with its contractual obligations to provide utilization management. Coverage for services varies for individual members according to the terms of their health care coverage or government program. Individual members' health care coverage may not utilize some Evolent Clinical Guidelines. Evolent clinical guidelines contain guidance that requires prior authorization and service limitations. A list of procedure codes, services or drugs may not be all inclusive and does not imply that a service or drug is a covered or non-covered service or drug. Evolent reserves the right to review and update this Clinical Guideline in its sole discretion. Notice of any changes shall be provided as required by applicable provider agreements and laws or regulations. Members should contact their Plan customer service representative for specific coverage information.

REFERENCES

1. Hermans C, Astermark J, de Moerloose P. Exposure to factor VIII and prediction of inhibitor development: exposure days vs. danger days, or both? J Thromb Haemost 2012; 10: 2194–6.
2. Ozelo MC, Mahlangu J, Pasi KJ, et al; GENEr8-1 Trial Group. Valoctocogene roxaparvovec gene therapy for hemophilia A. N Engl J Med. 2022;386(11):1013-1025. doi:10.1056/NEJMoa2113708 [PubMed [35294811](#)]
3. Roctavian (valoctocogene roxaparvovec) [prescribing information]. Novato, CA: BioMarin Pharmaceuticals Inc; June 2023.