RX.PA.084.MPC Roctavian (valoctocogene roxaparvovec-rvox)

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

Roctavian® (valoctocogene roxaparvovec-rvox) 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 detected by an FDA-approved test.

PROCEDURE

A. Initial Authorization Criteria

1. Must meet ALL of the criteria listed below:

- Documented diagnosis of severe hemophilia A (congenital factor VIII deficiency):
  - Factor VIII activity level < 1 IU/dL (in absence of exogeneous factor VIII)
- Patient is 18 years of age or older
- Prescribed by or in consultation with a hematologist
- Documentation that shows patient has undetectable pre-existing antibodies to AAV5 capsid confirmed by a AAV5 total antibody assay prior to start of therapy
  - Note: labs obtained within 30 days of the authorization request
- Prescriber attests that patient will have a negative factor VIII titer test performed within 30 days of initiating the prior authorization request
- Documentation that confirms patient is currently being treated with a factor VIII product and has been adherent with therapy for at least 12 months
- Documentation that the patient has a history of serious and spontaneous bleeding episodes while being treated with factor VIII prophylaxis therapy
  - i.e. Gastrointestinal and intracranial bleeding events, etc.
- Prescriber attests that bleeding disorders that are not related to hemophilia A have been ruled out
- Documentation confirming that the patient does not have any of the following (lab work must be obtain within 60 days of starting treatment process):

  - Active infection
  - Hepatitis B and C
  - HIV
  - Liver fibrosis (stage 3 or 4)
  - Cirrhosis
  - Liver function test abnormalities
    a. (ALT/AST/GGT/ALP or total bilirubin > 1.25 ULN)

b. INR ≥ 1.4
- History of thrombosis or thrombophilia
- Hypersensitivity to mannitol
- Active malignancy
- Renal impairment
  a. Serum creatinine ≥ 1.4 mg/dL

- Prescriber attests that the patient will not receive concomitant live vaccines during treatment
- Prescriber attests to stopping factor VIII prophylaxis therapy* within 4 weeks after receiving Roctavian
  - *Note: active prior authorizations for prophylaxis therapy will be termed
- Following infusion, provider attests that liver enzyme testing to monitor for liver enzyme elevations will be done weekly for at least 26 weeks and periodically thereafter
- Prescriber attests that member will undergo monitoring for Factor VIII activity at least weekly for at least 26 weeks and periodically thereafter
- Dose does not exceed FDA approved indication
- Prescriber attests that the patient has not been treated and will not receive concomitant therapy with another adeno-associated virus vector-based gene therapy for the management of hemophilia A

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Roctavian will be considered investigational or experimental for any other use and will not be covered.

D. <u>Reauthorization Criteria:</u>

Roctavian is not eligible for reauthorization.

<u>Limitations:</u>

Length of Authorization (if above criteria met)	
Initial Authorization	1 month
Reauthorization	N/A

<u>Codes:</u>

Code	Description
J3590	Injection, Unclassified biologics

REFERENCES

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

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review	*02/2026*
Annual Review	*02/2025*
Annual Review	*02/2024*
New Policy	*08/2023*