

5.85.051

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<b>Section:</b>	Prescription Drugs	<b>Effective Date:</b>	January 1, 2024
<b>Subsection:</b>	Hematological Agents	<b>Original Policy Date:</b>	July 28, 2023
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**Last Review Date:** December 8, 2023

## Roctavian

### Description

#### Roctavian (valoctocogene roxaparvovec-rvox)

#### Background

Roctavian (valoctocogene roxaparvovec-rvox) is an adeno-associated virus serotype 5 (AAV5) based gene therapy designed to introduce a functional copy of a transgene encoding the B-domain deleted SQ form of human coagulation factor VIII (hFVIII-SQ). Factor VIII activity levels increased over time post-Roctavian infusion (1).

#### Regulatory Status

FDA-approved indication: 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 detected by an FDA-approved test (1).

Roctavian is contraindicated in patients with active infections (either acute or uncontrolled chronic), known significant hepatic fibrosis (stage 3 or 4), cirrhosis, and known hypersensitivity to mannitol (1).

Roctavian can cause hepatotoxicity. Alanine aminotransferase (ALT) levels should be monitored weekly for at least 26 weeks after Roctavian infusion and corticosteroid treatment provided as clinically indicated. Patients with preexisting risk factors for hepatocellular carcinoma should

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receive abdominal ultrasound screenings and be monitored regularly for alpha-fetoprotein (AFP) following administration (1).

Factor VIII activity and factor VIII inhibitors should be regularly monitored (1).

The safety and effectiveness of Roctavian in pediatric patients less than 18 years of age have not been established (1).

## Related policies

### Policy

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Roctavian may be considered **medically necessary** if the conditions indicated below are met.

Roctavian may be considered **investigational** for all other indications.

## Prior-Approval Requirements

**Age** 18 years of age or older

**Gender assigned at birth** Male

### Diagnosis

Patient must have the following:

Hemophilia A

**AND ALL** of the following:

1. Severe or moderately severe hemophilia A defined as residual factor VIII levels  $\leq 1$  IU/dL
2. Patient has been receiving prophylactic Factor VIII replacement therapy for at least 12 months prior to administration
3. Patient has received  $\geq 150$  exposure days of treatment with factor VIII concentrates or cryoprecipitate

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4. Patient will receive pre-infusion and post-infusion monitoring of factor VIII and liver enzyme testing, including but not limited to ALT
5. Patient has received sufficient education and discussion regarding alcohol abstinence and use of certain concomitant medication (antiretrovirals, significant hepatic metabolizers, etc.)
6. Prescribed by or recommended by a hematologist or a prescriber who specializes in hemophilia A

**AND NONE** of the following:

1. History of factor VIII inhibitors
2. Positive factor VIII inhibitor screen results of  $\geq 0.6$  Bethesda Units (BU) using the Nijmegen-Bethesda assay
3. Detectable pre-existing antibodies to the AAV5 capsid
4. Positive HIV serological test
5. Active infection, including active or chronic hepatitis B or active hepatitis C infection
6. Prior gene therapy for hemophilia A or under consideration for treatment with another gene therapy for hemophilia A

## **Prior – Approval *Renewal* Requirements**

None

### [Policy Guidelines](#)

## **Pre - PA Allowance**

None

## **Prior - Approval Limits**

**Quantity** One infusion (only one PA approval for one infusion per lifetime)

## **Prior – Approval *Renewal* Limits**

None

### [Rationale](#)

## **Summary**

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Roctavian is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with severe Hemophilia A. The safety and effectiveness of Roctavian in pediatric patients less than 18 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Roctavian while maintaining optimal therapeutic outcomes.

### References

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

### Policy History

Date	Reason
July 2023	Addition to PA
September 2023	Annual review
December 2023	Annual review

### Keywords

**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 8, 2023 and is effective on January 1, 2024.**