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Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Hematological Agents	**Original Policy Date:**	July 28, 2023
Subject:	Roctavian	**Page:**	1 of 4

Last Review Date: March 7, 2025

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

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 ≤ 1 IU/dL
2. Patient has been receiving prophylactic Factor VIII replacement therapy
3. Patient will receive baseline liver enzyme testing, including but not limited to ALT

4. Patient has received sufficient education and discussion regarding alcohol abstinence and use of certain concomitant medication (e.g., isotretinoin, efavirenz)
5. 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 ≥ 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

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
June 2024	Annual review
September 2024	Annual review
January 2025	Per Association, removed requirement to have at least 150 exposure days of treatment with factor VIII concentrates or cryoprecipitate. Also reworded requirements for receiving prophylactic FVIII therapy, baseline liver enzyme testing, and sufficient education about concomitant medications
March 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.