Reference number(s)
6062-A

Specialty Guideline Management
Roctavian

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Roctavian	valoctocogene roxaparvovec-rvox

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

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.

All other indications are considered experimental/investigational and not medically necessary.

Documentation

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

Chart notes, medical records, or lab results documenting all of the following:

- Severe factor VIII deficiency (factor VIII activity levels less than or equal to 1 IU/dL).
- Absence of pre-existing antibodies to the adeno-associated virus serotype 5 (AAV5) capsid.

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- Absence of factor VIII inhibitor confirmed by a Bethesda assay (lab test results required).
- Baseline hematologic, hepatic, and renal assessments.

Prescriber Specialties

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

Coverage Criteria

Hemophilia A[1]

Authorization of 3 months for one dose total may be granted for treatment of severe hemophilia A (congenital factor VIII deficiency) when all of the following criteria are met:

- Member must be 18 years of age or older.
- Member has severe disease with factor VIII activity levels less than or equal to 1 IU/dL.
- Absence of pre-existing antibodies to AAV5 was confirmed by an FDA-approved test (e.g., AAV5 DetectCDx™).
- Member meets both of the following:
  - Member does not have a history of Factor VIII inhibitors (≥ 0.6 Bethesda units [BU]).
  - Member has a negative Factor VIII inhibitor test result within the past 30 days (< 0.6 Bethesda units [BU]).
- Member meets all of the following:
  - Member is currently using Factor VIII prophylaxis therapy (e.g., Advate, Adynovate, Eloctate).
  - Member has a history of prophylactic Factor VIII use for at least 150 exposure days.
  - Member has uncontrolled disease despite the use of prophylactic Factor VIII or has a clinical reason to avoid therapy with Factor VIII prophylaxis.
- Member has the following laboratory values at baseline:
  - Platelets ≥ 100,000 cells/microL.
  - Creatinine < 1.5 mg/dL.
- Member does not have alanine transaminase (ALT), aspartate aminotransferase (AST), alkaline phosphatase (ALP), gamma-glutamyl transferase (GGT) levels and total bilirubin (unless there is a diagnosis of Gilbert's Syndrome and member is otherwise stable) greater than 1.25 times the upper limit of normal (ULN).
- Member does not have an INR (international normalized ratio) ≥ 1.4.
- Member does not have stage 3 or 4 liver fibrosis.
- Member has undergone a hepatic ultrasound and/or elastography to rule out radiological liver abnormalities and/or sustained liver enzyme elevations.

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- Member meets both of the following:
    - Member does not have a chronic or active infection with hepatitis B virus or hepatitis C virus.
    - Member is not currently receiving antiviral therapy for a prior hepatitis B virus or hepatitis C virus exposure.
- Member does not have uncontrolled human immunodeficiency virus (HIV) infection.
- Member does not have an active infection or other immunosuppressive disorder.
- Member does not have an active malignancy.
- Member does not have a history of arterial or venous thromboembolic events (e.g., deep vein thrombosis, non-hemorrhagic stroke, pulmonary embolism, myocardial infarction, arterial embolism).
- Member does not have a known inherited or acquired thrombophilia, including conditions associated with increased thromboembolic risk (e.g., atrial fibrillation).
- Member has not received Roctavian or any other gene therapy previously.
- Prophylactic use of Factor VIII products will not be given after Roctavian administration once adequate Factor VIII levels have been achieved (note: Factor VIII therapy may be given in case of surgery, invasive procedures, trauma, or bleeds in the event that Roctavian-derived Factor VIII activity is deemed insufficient for adequate hemostasis).
- Provider attests that liver enzymes and Factor VIII activity will be followed per the protocol outlined in the prescribing information following receipt of Roctavian infusion.

Reference

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

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