Reference number(s)
6063-A

Standard Medicare Part B 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 will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

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).

Prescriber Specialties

Treatment should be under the supervision of a physician experienced in the treatment of hemophilia and/or bleeding disorders.

Coverage Criteria

Hemophilia A[1]

Authorization of 3 months for one dose total may be granted for treatment of severe hemophilia A 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 Detect CDx™).
- Member meets both of the following:
  - Member does not have a history of Factor VIII inhibitors (e.g., ≥ 0.6 Bethesda units [BU]).
  - Member has a negative Factor VIII inhibitor test result within the past 30 days (e.g., < 0.6 Bethesda units [BU]).
- Member has not received Roctavian or any other gene therapy previously.

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Roctavian.
  - The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology

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After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Roctavian are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

References

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

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