CarePartners of Connecticut MEDICARE ADVANTAGE

Medical Necessity Guidelines

Medical Benefit Drugs

Roctavian™ (valoctocogene roxaparvovec-rvox)

Effective: January 1, 2024

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

☒ CarePartners of Connecticut Medicare Advantage HMO plans, Fax 617-673-0956

☒ CarePartners of Connecticut Medicare Advantage PPO plans, Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Hemophilia is an X-linked recessive genetic disorder that primarily affects males. It is caused by mutations in the genes that encode coagulation factors. This causes bleeding into soft tissue, joints, and internal organs. There are two types of hemophilia: hemophilia A is caused by a deficiency in Factor VIII (FVIII), and hemophilia B is caused by a deficiency in coagulation factor IX (FIX).

According to the Centers for Disease Control and Prevention (CDC), there are between 30,000 and 33,000 males with hemophilia in the United States. Hemophilia A occurs in approximately 1 in 5617 live male births and is four times as common as hemophilia B.

Food and Drug Administration (FDA) Approved Indications:

- Roctavian is 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.

Roctavian consists of a viral vector carrying the F8 gene that encodes FVIII and is administered intravenously (IV) as a one-time dose.

Care Partners of Connecticut uses guidance from the Centers for Medicare and Medicaid Services (CMS) for its Medicare Advantage plan members. CMS National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs) and documentation included in the Medicare manuals are the basis for coverage determinations. When CMS does not provide guidance, the Plan's internally developed medical necessity guidelines are used. CMS coverage guidelines are not established for this service.

For the therapy Roctavian, evidence is sufficient for coverage. Roctavian was FDA approved in June 2023 based on the results of a 3-year open-label, single-group, multicenter, Phase 3 GENEr8-1 study. This study found that members annualized bleeding rate (ABR), or bleeds per year, dropped from an average of 5.4 bleeds per year before Roctavian to an average of 2.6 bleeds per year after Roctavian. Overall, 90.3% had no treated bleeds or fewer treated bleeds after infusion. Roctavian provides a one-time treatment option for specific members with hemophilia A, compared to the previous treatment alternative of Factor VIII.

The use of this criteria in the utilization management process will ensure access to evidence based clinically appropriate care. See References section below for all evidence accessed in the development of these criteria.

Clinical Guideline Coverage Criteria

The Plan may cover Roctavian when all the following clinical criteria is met:

1. The Member has been diagnosed with severe congenital Hemophilia A with Factor VIII (FVIII) activity <1% (1 IU/dL); **and**
2. The Member is 18 years of age or older; **and**
3. The Member does not have pre-existing antibodies to the adeno-associated virus serotype 5 (AAV5), as confirmed by an FDA-approved diagnostic test; **and**
4. The Member has received continuous FVIII prophylaxis for at least 1 year; **and**
5. The Member has had a minimum of 150 previous exposure days of treatment with FVIII replacement therapy within their lifetime; **and**
6. The Member does not have a history of FVIII inhibitors at screening (defined as greater than or equal to 0.6 Bethesda units on 2 consecutive occasions at least one week apart); **and**
7. The Member has adequate levels of **ALL** of the following:
    a. Blood count (eg platelet count); **and**
    b. Liver function (abnormal liver function is defined as alanine aminotransferase (ALT), aspartate aminotransferase (AST), gamma-glutamyl transferase (GGT), alkaline phosphatase [ALP], total bilirubin greater than 1.25 times the upper limit of normal (ULN) or international normalized ration (INR)] greater than or equal to 1.4 at screening); **and**
    c. Renal function; **and**
8. Member does not have **ANY** of the following:
    a. A known hypersensitivity to mannitol; **and**
    b. An active infection (e.g., viral, bacterial, fungal); **and**
    c. A history of hepatic malignancy or an active malignancy, except non-melanoma skin cancer; **and**
    d. Cirrhosis or significant hepatic fibrosis (stage 3 or 4); **and**
    e. A history of arterial or venous thromboembolic events or known risk factors for thrombosis; **and**
9. Member is not HIV positive OR Member is HIV positive and is virally suppressed with anti-viral therapy (<200 copies of HIV per mL); **and**
10. Members hepatitis B surface antigen is negative and is not on any hepatitis B antivirals; **and**
11. Member's hepatitis C is negative by antibody or HCV RNA test and not on any hepatitis C antivirals; **and**
12. The Member has been assessed for extended corticosteroid and/or immunosuppressive therapy; **and**
13. The Member has received education relating to alcohol abstinence and the use of hepatotoxic medications; **and**
14. Roctavian will be administered at a qualified Hemophilia Treatment Center authorized by BioMarin

Limitations

- Members who have received prior gene therapy for Hemophilia A, including therapies in clinical trial settings, will not be approved for Roctavian
- Roctavian will not be covered if the Member demonstrates clinical decompensation from time of authorization to time of infusion and no longer meets clinical coverage criteria.
- Any indications for Roctavian other than those outlined above are considered investigational and will not be covered
- Authorization of Roctavian is limited to one single dose treatment

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J1412	Injection, valoctocogene roxaparvovec-rvox, per ml, containing nominal 2 x 10^13 vector genomes

References:

1. Roctavian (valoctocogene roxaparvovec-rvox) [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; June 2023.
2. Ozelo MC, et al. Valoctocogene roxaparvovec gene therapy for hemophilia A. N Engl J Med. 2022;386(11):1013-1025.
3. Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients (BMN 270-301); NCT03370913. Accessed @ https://classic.clinicaltrials.gov/ct2/show/study/NCT03370913, accessed July 17, 2023.

4. Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at a Dose of 4E13 vg/kg (BMN270-302). Clinicaltrials.gov website https://clinicaltrials.gov/ct2/show/NCT03392974?term=bmn+270&draw=2&rank=2. Accessed July 17, 2023.
5. New Drug Review: Roctavian (valoctocogene roxaparvovec-rvox). IPD Analytics. August 2023.

Approval And Revision History

November 16, 2023: Reviewed by the Medical Policy Approval Committee (MPAC) effective January 1, 2024

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.