CarePartners of Connecticut MEDICARE ADVANTAGE

Medical Necessity Guidelines

Medical Benefit Drugs

Roctavian™ (valoctocogene roxaparvovec-rvox)

Effective: January 1, 2025

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 is not established for this service. Point32Health covers Zynteglo in accordance with MassHealth coverage criteria.

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 the provider provides documentation of all the following and all the following clinical criteria

is met:

1. Diagnosis of severe hemophilia A (documented FVIII activity level ≤ 1% of normal); **and**
2. Prescriber is a hematologist or consult notes from a hematologist are provided; **and**
3. Appropriate dosing; **and**
4. Member weight; **and**
5. Member is ≥ 18 years of age on treatment date; **and**
6. Member is a biologic male/male assigned at birth; **and**
7. Member will be screened for acute infection prior to administration; **and**
8. Member has been assessed for their ability to receive corticosteroids and/or immunosuppressive therapy; **and**
9. Member currently uses **ONE** of the following:
    a. FVIII prophylaxis therapy; **or**
    b. Hemlibra® (emicizumab); **and**
10. **BOTH** of the following:
    a. Baseline ABR*; **and**
    b. FVIII activity level; **and**
11. Member does NOT have **ALL** of the following:
    a. Detectable pre-existing immunity to AAV5; **and**
    b. History of factor VIII inhibitor; **and**
    c. Hepatic fibrosis (stage 3 or 4 on the Batts Ludwig scale); **and**
    d. Cirrhosis; **and**
    e. History of thrombosis or thrombophilia; **and**
    f. Active malignancy

Limitations

- 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. MassHealth Drug List - Health and Human Services. Table 80: Anti-Hemophilia Agents. April 2024. Accessed June 7, 2024. https://mhdl.pharmacy.services.conduent.com/MHDL/pubtheradetail.do?id=2439&drugId=8653
6. 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

Subsequent endorsement date(s) and changes made:

- December 1, 2023: Reviewed and approved by UM Committee effective January 1, 2024
- May 15, 2024: Reviewed by MPAC, criteria update to align with MassHealth criteria effective July 1, 2024
- June 13, 2024: Reviewed and approved by UM Committee effective July 1, 2024
- November 21, 2024: Reviewed by MPAC, renewed without changes, effective, January 1, 2025

- December 13, 2024: Reviewed and approved by the UM Committee, effective January 1, 2025

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.