HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Roctavian (valoctocogene roxaparvovec)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

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. Roctavian was approved by the FDA in June 2023 as the first gene therapy for hemophilia A. It provides a functional copy of a transgene encoding the B-domain deleted SQ form of human coagulation factor VIII (hFVIII-SQ) to replace the missing coagulation factor VIII needed for effective hemostasis.
Hemophilia is an X-linked disorder primarily affecting males that interferes with the normal coagulation process, which can cause bleeding into tissues, joints, and organs. Hemophilia A is more common than hemophilia B. It is caused by a faulty gene leading to a lack of clotting factor VIII. Standard treatment is by replacement of the missing factor or use of Hemlibra. Severe patients may require factor infusions 3 times per week. Successful gene therapy would obviate the need for prophylactic factor product use.
Approval was based on data from the Phase 3 GENEr8-1 study in which Roctavian substantially increased factor VIII activity and reduced the annualized rates of factor VIII use and bleeding as compared with factor VIII prophylaxis. Efficacy and safety are also supported by 5 years of data from a Phase 1/2 study.

Roctavian (valoctocogene roxaparvovec) will be considered for coverage when the following criteria are met:

Hemophilia A

For **initial** authorization *(DOCUMENTATION REQUIRED FOR ALL TEST RESULTS)*:

1. Member is a male at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a diagnosis of severe hemophilia A with factor VIII baseline residual level less than or equal to 1 IU/dL; AND
4. Member has tested negative for antibodies to adeno-associated virus serotype 5 (AAV5) within the past 3 months; AND
5. Member has tested negative for inhibitors to factor VIII (0.6 Bethesda units/mL) within the past 12 months; AND
6. Member meets one of the following:
   a) On prophylactic therapy with a factor VIII agent (e.g., Advate, Eloctate, Recombinate) AND has had a minimum of 150 exposure days OR
   b) The prescriber has determined that the patient requires improved protection than they are receiving from their current therapy (e.g., increased bleeding due to severely damaged joints, increased bleeding due to need for anticoagulation, elderly patients with risk for falls); AND
7. Member's liver function tests have been assessed in the last 3 months; AND
8. Member does NOT have any of the following:
   a) Acute or uncontrolled chronic active infections (e.g., HIV, hepatitis B, C)

b) Any immunosuppressive disorder
c) Significant (stage 3 or 4) hepatic fibrosis or cirrhosis
d) Active malignancy or history of hepatic malignancy
e) Any other bleeding disorder
f) History of thromboembolic event
g) Any prior gene therapy; AND

9. Member's current weight is provided for dose calculation.
10. **Dosage allowed/Quantity limit:** 6×10^{13} vector genomes (vg) per kg intravenously one time.

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:

1. Not applicable; Roctavian is a one-time single dose.

HAP CareSource considers Roctavian (valoctocogene roxaparvovec) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
08/02/2023	New policy for Roctavian created.
05/17/2024	Added guideline to reference list.

References:

1. Roctavian. [prescribing information]. BioMarin Pharmaceutical Inc.; 2023.
2. Ozelo MC, Mahlangu J, Pasi KJ, et al. Valoctocogene Roxaparvovec Gene Therapy for Hemophilia A. *N Engl J Med*. 2022;386(11):1013-1025. doi:10.1056/NEJMoa2113708
3. Pasi KJ, Laffan M, Rangarajan S, et al. Persistence of haemostatic response following gene therapy with valoctocogene roxaparvovec in severe haemophilia A. *Haemophilia*. 2021;27(6):947-956. doi:10.1111/hae.14391
4. Institute for Clinical and Economic Review (ICER). Gene Therapy for Hemophilia B and An Update on Gene Therapy for Hemophilia A: Final Policy Recommendations. December 2022.
5. IPD Analytics. Accessed August 2, 2023.
6. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition [published correction appears in Haemophilia. 2021 Jul;27(4):699]. *Haemophilia*. 2020;26 Suppl 6:1-158. doi:10.1111/hae.14046

Effective date: 01/01/2025
Revised date: 05/17/2024