Generic Name: valoctocogene roxaparvovec-rvox

Applicable Drugs: Roctavian

Preferred: N/A

Non-preferred: N/A

Date of Origin: 2/26/2024

Date Last Reviewed / Revised: 05/12/2024

PRIOR AUTHORIZATION CRITERIA

(May be considered medically necessary when criteria I through iX are met)

I. Natal male aged ≥ 18 years old with a diagnosis of hemophilia A and severe factor VIII (FVIII) deficiency.

II. Documented current use of routine prophylaxis or on-demand FVIII replacement for ≥ 12 months before the request.

III. Documented history of ≥ 150 previous exposure days of FVIII concentrates or cryoprecipitates.

IV. No history of previous gene therapy for hemophilia A.

V. Documentation of baseline labs/testing results (A through G):

   A. Liver function assessments, including liver enzyme testing (AST, ALT, ALP, GGT, and total bilirubin), hepatic ultrasound, and elastography to rule out significant hepatic fibrosis (stage 3 or 4) or cirrhosis.

   B. Hepatitis B serology to rule out active Hepatitis B infection.

   C. Hepatitis C serology to rule out active Hepatitis C infection.

   D. HIV screening and HIV-RNA levels (if applicable) to rule out active and virally unsuppressed HIV infection.

   E. FVIII activity level ≤ 1 IU/dL indicating severe FVIII deficiency.

   F. Negative FVIII inhibitor titer (< 0.6 Bethesda units).

   G. Negative anti-adeno-associated viral vector serotype V5 (AAV5) antibody titers by FDA-approved test (AAV5 DetectCDx).

VI. Must be prescribed by a board-certified hematologist.

VII. Must be administered by a qualified health care professional.

VIII. Indication, dose, and plan for monitoring are consistent with FDA labeling. The FDA-recommended dose of valoctocogene roxaparvovec-rvox is 6 × 1013 vector genomes (vg) per kg of body weight administered by IV infusion.

EXCLUSION CRITERIA

- Acute or uncontrolled chronic active infections.

- Currently receiving Hepatitis B or Hepatitis C antiviral therapy.
- Significant hepatic fibrosis (stage 3 or 4) or cirrhosis
- Known hypersensitivity to mannitol.

OTHER CRITERIA

- N/A

QUANTITY / DAYS SUPPLY RESTRICTIONS

- Number of vials/1 day supply
    - Number of vials = Patient dose in mL (BW in kg x 3 = dose in mL)/8 (rounded up to next whole number of vials).
    - Example: BW=70 kg.
        - Dose in mL= 70 kg x 3 mL/kg= 210 mL
        - Number or vials= 210 mL/8 = 26.65 or 27 vials

APPROVAL LENGTH

- **Authorization:** Approve for 1-time treatment course for a 6-month duration. Limit 1 treatment course per lifetime.
- **Re-Authorization:** N/A

APPENDIX

N/A

REFERENCES

1. Roctavian. Prescribing information. BioMarin Pharmaceutical Inc. 2023. Accessed December 3, 2023. https://d34r3hkxgxjdtw.cloudfront.net/6f836309-d95f-42af-b717-2efa058ad82d/78bf2bcb-7068-4774-b962-a35c53704fc1/78bf2bcb-7068-4774-b962-a35c53704fc1_source__v.pdf
2. Ozelo MC, Mahlangu J, Pasi KJ, et al; GENEr8-1 Trial Group. Valoctocogene roxaparvovec gene therapy for Hemophilia A. *N Engl J Med.* 2022;386(11):1013-1025. doi: 10.1056/NEJMoa2113708
3. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. *Haemophilia.* 2020: 26(Suppl 6): 1-158. doi.org/10.1111/hae.14046

DISCLAIMER: Medication Policies are developed to help ensure safe, effective and appropriate use of selected medications. They offer a guide to coverage and are not intended to dictate to providers how to practice medicine. Refer to Plan for individual adoption of specific Medication Policies. Providers are expected to exercise their medical judgement in providing the most appropriate care for their patients.