

Applicable Drugs: Roctavian (valoctocogene roxaparvovec-rvox)

Preferred: N/A

Non-preferred: N/A

Date of Origin: 2/26/2024

Date Last Reviewed / Revised: 10/20/2025

PRIOR AUTHORIZATION CRITERIA

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

- I. Natal male aged ≥ 18 years old with documented 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-adenovirus-associated viral vector serotype V5 (AAV5) antibody titers by FDA-approved test (AAV5 DetectCDx).
- VI. Treatment must be prescribed by or in consultation with a hematologist.
- VII. Treatment must be administered by a qualified healthcare professional.
- VIII. Request is for a medication with the appropriate FDA labeling, or its use is supported by current clinical practice guidelines.
- IX. Refer to the plan document for the list of preferred products. If the requested agent is not listed as a preferred product, must have a documented failure, intolerance, or contraindication to a preferred product(s).

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 per 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 for Body Weight (BW) = 70 kg
 - Dose in mL = 70 kg x 3 mL/kg = 210 mL
 - Number of vials = 210 mL/8 = 26.65 or 27 vials

APPROVAL LENGTH

- **Authorization:** Approve 1-time treatment course for a 6-month duration. Not to exceed 1 treatment course per lifetime.
- **Re-Authorization:** N/A

APPENDIX

N/A

REFERENCES

1. Roctavian. Prescribing Information. BioMarin Pharmaceutical Inc.; 2023. Accessed October 20, 2025.
<https://www.roctavian.com/en-us/wp-content/uploads/sites/5/2023/07/ROC-ROCTAVIAN-Prescribing-Information-PI-DIGITAL.pdf?v=0.24>
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: 1.1056/NEJMod2113708
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

4. Mahlangu J, Kaczmarek R, von Drygalski A, et al. Two-Year Outcomes of Valoctocogene Roxaparvovec Therapy for Hemophilia A. *N Engl J Med*. 2023;388(8):694-705. doi:10.1056/NEJMoa2211075
5. Madan B, Ozelo MC, Raheja P, et al. Three-year outcomes of valoctocogene roxaparvovec gene therapy for hemophilia A. *J Thromb Haemost*. 2024;22(7):1880-1893. doi:10.1016/j.jth.2024.04.001
6. Leavitt AD, Mahlangu J, Raheja P, et al. Efficacy, safety, and quality of life 4 years after valoctocogene roxaparvovec gene transfer for severe hemophilia A in the phase 3 GENEr8-1 trial. *Res Pract Thromb Haemost*. 2024;8(8):102615. Published 2024 Oct 30. doi:10.1016/j.rpth.2024.102615

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.