Roctavian™ (valoctocogene roxaparvovec-rvox)

When requesting Roctavian™ (valoctocogene roxaparvovec-rvox), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-Approved Indication

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 antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test.

Coverage Guidelines

Hemophilia A

The individual must meet all of the following criteria for authorization:

- Is male;
- Is 18 years of age or older;
- Has severe hemophilia A as evidence by a baseline (without Factor VIII replacement therapy) Factor VIII level of < 1 IU/dL;
- Does not have detectable pre-existing antibodies to adeno-associated virus 5 (AAV5) by an FDA-approved test;
- Has a history of use of Factor VIII therapy for at least 150 exposure days;
- Performed Factor VIII inhibitor titer testing within the past 30 days before intended receipt of Roctavian;
- Does not currently have an inhibitor to Factor VIII;
- Does not have a history of Factor VIII inhibitors;
- Prophylactic therapy with Factor VIII will not be given after Roctavian administration once adequate Factor VIII levels have been achieved *(Note: use of episodic Factor VIII therapy is acceptable for the treatment of bleeds and for surgery/procedures if needed)*;
- Has not received Roctavian in the past;
- Does not have a known hypersensitivity to mannitol;
- Does not have chronic or active hepatitis B;
- Does not have active hepatitis C;
- Does not have evidence of significant hepatic fibrosis or cirrhosis;

- Has undergone liver function testing within the past 30 days and meets all of the following *(Note: if one or more of the laboratory values listed below is not at the value specified below, the individual has been evaluated and determined that the use of Roctavian is clinically appropriate by a hepatologist):*
    - Alanine aminotransferase levels are ≤ 1.25 x the upper limit of normal (ULN);
    - Aspartate aminotransferase levels are ≤ 1.25 x ULN;
    - Total bilirubin levels are ≤ 1.25 x ULN;
    - Alkaline phosphatase levels are ≤ 1.25 x ULN;
    - Gamma-glutamyl transferase levels are ≤ 1.25 x ULN;
    - International Normalization ratio is < 1.4
- The platelet count was ≥ 100 x 10^9/L within the past 30 days;
- The creatinine level was < 1.4 mg/dL within the past 30 days;
- Is not human immunodeficiency virus positive;
- Current body weight has been obtained within the past 30 days;
- Roctavian is prescribed by a hemophilia specialist physician.

Approval duration: One time (per lifetime)

Dosing Recommendation

Roctavian is for one-time single-dose intravenous use only.

The recommended dose of Roctavian is 6 x 10^{13} vector genomes (vg) per kg of body weight.

References

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7. National Hemophilia Foundation. MASAC (Medical and Scientific Advisory Council) recommendations concerning products licensed for the treatment of hemophilia and other selected disorders of the coagulation system (endorsed by the National Hemophilia Foundation Board of Directors on May 2, 2023). MASAC Document #276. Available at: https://www.hemophilia.org/sites/default/files/document/files/MASACTreatment.pdf. Accessed on August 13, 2023.
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9. Mahlangu J, Kaczmarek R, Von Drygalski A, et al, for the GENEr8-1 trial group. Two-year outcomes of valoctocogene roxaparvovec therapy for hemophilia A. *N Engl J Med*. 2023;388(8):694-705.
10. Madan B, Ozelo MC, Paheja P, et al. Three-year outcomes of valoctocogene roxaparvovec gene therapy for hemophilia A. *J Thromb Haemost*. 2024;22:1880-1893.

