Roctavian™ (valoctocogene roxaparvovec-rvox)
(Intravenous)

Document Number: EOCCO-0718

Last Review Date: 06/04/2024
Date of Origin: 08/08/2023
Dates Reviewed: 08/2023, 06/2024

I. Length of Authorization

Coverage will be provided for one dose and may not be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Roctavian 2 x 10^{13} vg/mL single-dose vial: 44 vials one time only

B. Max Units (per dose and over time) [HCPCS Unit]:

- 352 billable units (352 mL) one time only

III. Initial Approval Criteria [1-15]

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax.

Coverage is provided in the following conditions:

Use for indications outside of FDA-approved labeled indications does NOT meet medical criteria for coverage and will be considered investigational, thus will NOT be covered.

Hemophilia A (Congenital Factor VIII Deficiency) † Φ

- Patient is at least 18 years of age; **AND**
- Patient has a diagnosis of severe hemophilia A (congenital factor VIII deficiency) as confirmed by a factor VIII activity level < 1 IU/dL; **AND**
- Evidence of any bleeding disorder NOT related to hemophilia A has been ruled out; **AND**
- Patient is on a stable dose of regularly administered exogenous factor VIII for the prevention and control of bleeding episodes; **AND**

- Patient does not have an active infection, either acute (such as acute respiratory infections or acute hepatitis) or uncontrolled chronic (such as chronic active hepatitis B); **AND**
- Patient is up to date with vaccinations prior to infusion and will avoid live vaccines while on immunosuppressive therapies; **AND**
- Patient does not have significant hepatic fibrosis (stage 3 or 4) or cirrhosis; **AND**
- Patient does not have a known hypersensitivity to mannitol: **AND**
- Patient has not received prior hemophilia AAV-vector–based gene therapy; **AND**
- Patient is adeno-associated virus serotype 5 (AAV5) antibody negative as determined by an FDA-approved or CLIA-compliant test❖; **AND**
- Patient has been tested and found negative for active factor VIII inhibitors *(i.e., results from a Bethesda assay or Bethesda assay with Nijmegen modification of less than 0.6 Bethesda Units (BU) on 2 consecutive occasions at least one week apart within the past 12 months)* and is not receiving a bypassing agent (e.g., Feiba, NovoSeven RT, SevenFact, etc.); **AND**
- Patient will have post-administration monitoring of serum ALT levels performed according to the monitoring schedule outlined in the product labeling with corticosteroids (or other immunosuppressive therapy) administered in response to elevations; **AND**
- Patients with preexisting risk factors for hepatocellular carcinoma [e.g., patients with hepatitis C or B, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age] will have regular (e.g., annually) liver ultrasounds performed and will be tested for alpha-fetoprotein (AFP) elevations following administration; **AND**

  - Provider attestation or documented in chart notes that the patient has been counseled or educated on ALL of the following:
    - Abstain from alcohol for at least a year; **AND**
    - Will not use any medications, herbal products, or supplements without first confirming with a health professional that they are not hepatotoxic; **AND**

- Patient will have factor VIII activity monitored according to the monitoring schedule outlined in the product labeling; **AND**
  - Patients with factor VIII activity levels >5 IU/dL will discontinue routine prophylactic exogenous factor VIII; **OR**
  - If the factor VIII activity levels decrease and/or if bleeding is not controlled, the patient will be assessed for the presence of factor VIII inhibitors and the need for hemostatic prophylaxis; **AND**

- Provider agrees to submit documentation or attestation, including but not limited to lab values or spontaneous or life-threatening bleeding events if Factor VIII is resumed and medically necessary for a patient following the administration of Roctavian

Notes:

- It may take several weeks after valoctocogene roxaparvovec infusion before valoctocogene roxaparvovec-derived factor VIII activity rises to a level sufficient for prevention of spontaneous bleeding episodes. Therefore, continued routine prophylaxis support with exogenous factor VIII or other hemostatic products used in the management of hemophilia A may be needed during the first few weeks after valoctocogene roxaparvovec infusion.
- Exogenous factor VIII or other hemostatic products may continue to be required in the case of surgery, invasive procedures, trauma, or bleeds in the event that valoctocogene roxaparvovec-derived factor VIII activity is deemed insufficient for adequate hemostasis in such situations.
- Use of exogenous factor VIII products before and after valoctocogene roxaparvovec administration may impede assessment of valoctocogene roxaparvovec-derived factor VIII activity.

❖ If confirmed using an immunotherapy assay-*http://www.fda.gov/companiondiagnostics*

✝ FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria [1]

Coverage cannot be renewed.

V. Dosage/Administration [1]

Indication	Dose
Hemophilia A (Congenital Factor VIII Deficiency)	The recommended dose of Roctavian is 6×10^{13} vector genomes per kilogram (vg/kg) body weight, administered as a single intravenous infusion. **Calculating Dose in Milliliters (mL) and Number of Vials Required** • Patient dose volume in mL: – Body weight in kg multiplied by 3 = dose in mL. – *The multiplication factor 3 represents the per kilogram dose (6×10^{13} vg/kg) divided by the amount of vector genomes per mL of suspension (2×10^{13} vg/mL).* • Number of vials to be thawed: – Patient dose volume (mL) divided by 8 = number of vials to be thawed (round up to next whole number of vials). – *The division factor 8 represents the minimum volume extractable from a vial (8 mL).*

- Roctavian is administered using an infusion pump at a rate of 1 mL/min, which can be increased every 30 minutes by 1 mL/min up to a maximum rate of 4 mL/min.
- Do not expose Roctavian to the light of an ultraviolet radiation disinfection lamp.
- Prepare using aseptic technique. Wear gloves and safety glasses during preparation and administration.
- Treat spills with a virucidal agent with proven activity against non-enveloped viruses and blot using absorbent materials.
- Dispose unused medicinal product and materials that may have come in contact with Roctavian in accordance with the local biosafety guidelines.
- Thaw at room temperature. Do not thaw or warm vials any other way. Thawing time is approximately 2 hours. Thawed suspension can be held at room temperature, up to 25°C (77°F), for a maximum of 10 hours including hold time in intact vial, preparation time into the syringes, and duration of infusion.
- DO NOT administer as an intravenous push or bolus.
- DO NOT infuse in the same intravenous line with any other products.
- DO NOT use a central line or port.

VI. Billing Code/Availability Information

HCPCS code:

- J1412 – Injection, valoctocogene roxaparvovec-rvox, per mL, containing nominal 2×10^{13} vector genomes; 1 billable unit = 1 mL, containing nominal 2×10^{13} vector genomes

NDC:

- Roctavian 2×10^{13} vector genomes (vg) per mL – 8 mL single dose vial: 68135-0927-xx

VII. References

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Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
D66	Hereditary factor VIII deficiency

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under

Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC