Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 308.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Roctavian (valoctocogene roxaparvovec-rvox)

Applicable line of business:

Commercial	X	Medicaid	X
Medicare	X	ACA	X
CHIP	X		

I. Policy:
Roctavian (valoctocogene roxaparvovec-rvox)

II. Purpose/Objective:
To provide a policy of coverage regarding Roctavian (valoctocogene roxaparvovec-rvox)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than the department requiring/authoring the policy.
3. Devised – the date the policy was implemented.
4. Revised – the date of every revision to the policy, including typographical and grammatical changes.
5. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:
a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:
Valoctocogene roxaparvovec is an adeno-associated virus serotype 5 (AAV5)–based gene therapy vector. It is designed to introduce a functional copy of a transgene encoding the B-domain deleted SQ form of human coagulation factor VIII (hFVIII-SQ). Transcription of this transgene occurs within the liver, using a liver-specific promoter, which results in hFVIII-SQ expression. The expressed hFVIII-SQ replaces the missing coagulation factor VIII required for effective hemostasis.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Roctavian (valoctocogene roxaparvovec-rvox) will be considered medically necessary for ALL lines of business when ALL of the following criteria are met:

- Medical record documentation that the patient is a male based on assigned sex at birth and age greater than or equal to 18 years **AND**
- Medical record documentation that the patient is diagnosed with severe hemophilia A* **AND**
- Medical record documentation that Roctavian is being dosed according to the Food and Drug Administration approved labeling for hemophilia A **AND**
- Medical record documentation that the member has not received any previous gene therapy for hemophilia A **AND**
- The prescription must be written with consultation from or by a Hematologist **AND**
- Medical record documentation showing lack of pre-existing antibodies to AAV5 using the FDA approved companion diagnostic **AND**
- Medical record documentation of factor VIII inhibitor titer testing showing lack of factor VIII inhibitor **AND**
- Medical record documentation whether a patient can receive corticosteroids and/or other immunosuppressive therapy that may be required for an extended period. **AND**
- Medical record documentation that the patient DOES NOT have active acute or uncontrolled chronic infections, known significant hepatic fibrosis (stage 3 or 4 on the Batts-Ludwig scale or equivalent) or cirrhosis, or mannitol hypersensitivity

***Note:** Severe hemophilia A is defined as Factor VIII activity <1% (<0.01 units/mL) with spontaneous bleeding into joints or muscles

AUTHORIZATION DURATION: One (1) time approval per lifetime. Requests for authorizations exceeding these limits will require the following medical record documentation of peer-reviewed literature citing well-designed clinical trials to indicate that the member's healthcare outcome will be improved by dosing beyond the FDA-approved treatment duration.

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

LINE OF BUSINESS:
Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Hemophilia. Centers for Disease Control and Prevention. Atlanta (GA). 2023 Jul 12. Available from: https://www.cdc.gov/ncbddd/hemophilia/index.html
2. DynaMed. Hemophilia A. EBSCO Industries 2023 [accessed 2023 Nov 8]. Available from https://www.dynamed.com/condition/hemophilia-a

3. Roctavian (Valoctocogene Roxaparvovec) prescribing information. Navato (CA): BioMarin Pharmaceutical, Inc; 2023 Jun. Available from: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=0dcf7185-8e1c-456a-9d4e-7cc316400118
4. Roctavian (Valoctocogene Roxaparvovec). In: Lexi-Drugs Online [database on the Internet]. Hudson (OH): Lexi-Comp, Inc.; 2023 [cited 2023 Nov 8]. Available from: http://online.lexi.com.
5. Ozelo MC, et al. Valoctocogene roxaparvovec gene therapy for hemophilia A. N Engl J Med. 2022;386(11):1013-1025. doi:10.1056/NEJMoa2113708
6. IPD Analytics. Roctavian for the Treatment of Adults with Hemophilia A. New Drug Approval Review. August 9, 2023. Accessed October 5, 2023. https://www.ipdanalytics.com

This policy will be revised as necessary and reviewed no less than annually.

Devised: 12/22/23

Revised: 12/2/24 (LOB table, taglines)

Reviewed:

MA UM Committee approval: 5/22/24

DHS PARP approval: 1/23/25