New Hampshire Medicaid Fee-for-Service Program Roctavian™ (valoctocogene roxaparvovec-rvox) Criteria

Approval Date: January 22, 2024

Medications

Brand Name	Generic Name	Indication
Roctavian™	valoctocogene roxaparvovec-rovx	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

Criteria for Approval

1. Patient is ≥ 18 years of age; **AND**
2. Patient has severe hemophilia A (congenital factor VIII deficiency) diagnosed by factor VIII activity level < 1 IU/dL (in the absence of exogenous factor VIII); **AND**
3. Evidence of any bleeding disorder NOT related to hemophilia A has been ruled out; **AND**
4. Patient is on a stable dose of regularly administered exogenous factor VIII for the prevention and control of bleeding episodes; **AND**
5. Patient does not have an active infection, either acute (e.g., acute respiratory infection or acute hepatitis) or uncontrolled chronic (e.g., chronic active hepatitis B); **AND**
6. Must not be administered concurrently with live vaccines while on immunosuppressive therapies; **AND**
7. Patient does not have significant hepatic fibrosis (stage 3 or 4) or cirrhosis; **AND**
8. Patient does not have a known hypersensitivity to mannitol; **AND**
9. Patient has not received prior hemophilia adeno-associated virus (AAV)-vector-based gene therapy; **AND**
10. Patient is AAV serotype 5 (AAV5) antibody negative as determined by an FDA-approved or CLIA-compliant test; **AND**
11. Patient has been tested and found negative for active factor VIII inhibitors (e.g., results from a Bethesda assay or Bethesda assay with Nijmegen modification of < 0.6 [BU] on 2 consecutive occasions ≥ 1 week apart within the past 12 months) and is not receiving a bypass agent (e.g., Feiba); **AND**
12. Post administration monitoring of patient serum ALT levels will be performed according to the monitoring schedule outlined in the product labeling, with corticosteroids (or other immunosuppressive therapy) administered in response to elevations; **AND**

13. 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., annual) liver ultrasounds performed and will be tested for alpha-fetoprotein (AFP) elevations following administration; **AND**
14. Patient factor VIII activity will be monitored periodically; **AND**
    a. Patients with factor VIII activity levels > 5 IU/dL should discontinue routine prophylactic exogenous factor VIII; **OR**
    b. If Factor VIII activity levels decrease and/or if bleeding is not controlled, assess presence of factor VIII inhibitors and assess the need for hemostatic prophylaxis.

Limitation

A single dose per lifetime.

Criteria for Denial

Criteria for approval are not met.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	12/08/2023
Commissioner Designee	Approval	01/22/2024