New Hampshire Medicaid Fee-for-Service Program Hemophilia B Gene Therapy Criteria

Approval Date: November 21, 2024

Medications

Brand Names	Generic Names	Indication
Hemgenix®	etranacogene dezaparvovec-drlb	indicated for treatment of adults with hemophilia B (congenital factor IX deficiency) who currently use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage or repeated, serious spontaneous bleeding episodes
Beqvez™	fidanacogene elaparvovec-dzkt	indicated for treatment of adults with moderate to severe hemophilia B (congenital factor IX deficiency) who currently use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage or repeated, serious spontaneous bleeding episodes **and** do not have neutralizing antibodies to adeno-associated virus serotype Rh74var capsid as detected by an FDA-approved test

Criteria for Approval

1. Patient is at least 18 years of age; **AND**
2. Prescribed by a hematologist and patient is managed by a hemophilia treatment center; **AND**
3. Patient has a diagnosis of moderately severe or severe congenital factor IX deficiency (e.g., factor IX ≤ 2%), as confirmed by blood coagulation testing; **AND**
4. Patient has one or more of the following:
    a. Currently uses factor IX prophylaxis therapy; **OR**
    b. Current or historical life-threatening hemorrhage; **OR**
    c. Repeated, serious spontaneous bleeding episodes; **AND**
5. Patient has been tested and found negative for factor IX inhibitor titers (Beqvez™ should not be administered if positive. For Hemgenix®, if test result positive, re-test within approximately 2 weeks. If re-test is also positive, Hemgenix® should not be given); **AND**
6. Patient factor IX (FIX) activity will be monitored periodically (frequency noted in package insert for Hemgenix® and Beqvez™); **AND**
7. Provider will monitor for presence of inhibitors if bleeding is not controlled (**note**: patient will continue to require exogenous factor IX until response occurs); **AND**

8. Patient will receive baseline liver function assessed prior to and after therapy weekly for at least 3 months with extended monitoring as noted in the package insert; **AND**
9. Patients with preexisting risk factors for hepatocellular carcinoma (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis C or B, non-alcoholic fatty liver disease [NAFLD], chronic alcohol consumption, non-alcoholic steatohepatitis [NASH], advanced age) will have abdominal ultrasound screenings and be monitored regularly (e.g., annually) for alpha-fetoprotein (AFP) elevations for 5 years following administration.

Limitation

A single dose per lifetime.

Criteria for Denial

1. Criteria for approval are not met.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	10/15/2024
Commissioner Designee	Approval	11/21/2024