Protocol for Hemgenix® (etranacogene dezaparvovec)

Approved April 2023

Background: *Hemophilia is a rare genetic bleeding disorder characterized by the inability of a person's blood to properly clot, resulting in spontaneous, prolonged, and excessive bleeding. Hemophilia B arises from missing or functionally defective levels of factor IX (FIX) due to a mutation in the F9 gene.*

Hemgenix *is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency).*

Criteria for approval:

1. Documented diagnosis of hemophilia B based on a factor assay to demonstrate deficiency of FIX; **AND**
    a. Documentation of moderate disease (FIX level 1-5 IU/dL, of 1%-5% of normal; OR
    b. Documentation of severe disease (FIX level < 1 IU/dL, or < 1% of normal
2. Current use of FIX prophylaxis for ≥ 2 months with greater than 150 previous exposure days of treatment with FIX protein; OR
    a. Have current or historical life-threatening hemorrhage; OR
    b. Have repeated, serious spontaneous bleeding episodes
3. Patient is male gender and is 18 years or older
4. Medication is prescribed by or in consultation with a hematologist
5. Approval is for one infusion per lifetime
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Exclusion Criteria:

Hemgenix will NOT be covered when ANY of the following are present:

1. Previous gene therapy

2. History of FIX inhibitors or a positive inhibitor screen conducted within the past 12 months (confirmed as 0.6 BU/ml [Bethesda Unit] or greater by chromogenic Bethesda Assay or other immunologic test)
3. Active hepatitis B infection, hepatitis C infection, or uncontrolled HIV infection
4. History of hepatitis B or C exposure currently controlled by antiviral therapy
5. Evidence of advanced liver fibrosis (screening laboratory values [i.e., ALT, AST, bilirubin, ALP, creatinine] > 2 times upper limit of normal

Approval Duration: One time dose

References

1. Hemgenix® (etranacogene dezaparvovec-drlb) [prescribing information]. King of Prussia, PA: CSL Behring. November 2022.
2. Soucie JM, Miller CH, Dupervil B, Le B, Buckner TW. Occurrence rates of haemophilia among males in the United States based on surveillance conducted in specialized haemophilia treatment centres. Haemophilia. 2020;26(3):487-493.
3. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically