Wellmark Advantage Health Plan

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Effective Date: 12/05/2022

Hemgenix® (etranacogene dezaparvovec-drlb)

HCPCS: J3590

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

A. Coverage of the requested drug is provided when all the following are met:
   a. FDA approved indication
   b. FDA approved age
   c. Must have severe disease defined as a factor IX levels less than 1% of normal
   d. Must currently be on factor IX therapy with greater than 150 prior exposure days to treatment
   e. Must not have a history of inhibitors to factor IX or a positive inhibitor screen defined as greater than or equal to 0.6 Bethesda units prior to administration of etranacogene dezaparvovec
   f. Must not have received prior treatment with any gene therapy for hemophilia B or are being considered for treatment with any other gene therapy for hemophilia B
   g. Trial and failure, intolerance, or a contraindication to the preferred products as specified in the Wellmark Advantage Health Plan medical utilization management drug list

B. Quantity Limitations, Authorization Period and Renewal Criteria
   a. Quantity Limit: Align with FDA recommended dosing
   b. Initial Authorization Period: 3 months
   c. Renewal Criteria: No renewal allowed, one infusion per lifetime

***Note: Coverage may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at http://www.cms.hhs.gov/. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- Hemophilia B is a rare genetic bleeding disorder in which affected individuals have insufficient levels of FIX due to a mutation on the F9 gene

- Hemophilia B is classified as mild, moderate or severe based upon the activity level of factor IX

    - Individuals with mild hemophilia have factor IX levels between 5 and 40% of normal

    - Those with moderate hemophilia have factor levels from 1 to 5% of normal

    - Patients with severe hemophilia have factor levels less than 1% of normal

- Symptoms may vary greatly from one person to another depending on severity

- Hemophilia B occurs in approximately 1 in 25,000 male births

- Although many hemophilia B carrier females do not have symptoms, an estimated 10-25% will develop mild symptoms and females have also been reported with moderate and severe symptoms

- Individuals with severe hemophilia B are usually diagnosed around birth or within the first 1-2 years of life; those with moderate hemophilia B, five to six years of age; and individuals with mild hemophilia B may not be diagnosed until later in life and even into adulthood

- Clotting factors are the treatment of choice for people with hemophilia as they are very safe and effective for treating and preventing bleeds

- The World Federation of Hemophilia (WFH) 2020 treatment guidelines do not express a preference for recombinant over plasma-derived clotting factor concentrates and state the choice between these classes of product must be made according to availability, cost, and patient preferences

- For treatment of hemophilia B, the WFH recommends a product containing only FIX is more appropriate than prothrombin complex concentrates which also contain other clotting factors such as factors II, VII, and X, that may become activated during manufacture and may predispose the patient to thromboembolism

- For patients with a severe phenotype, WFH strongly recommends patients be on prophylaxis sufficient to prevent bleeds at all times, but that prophylaxis should be individualized, taking into consideration patient bleeding phenotype, joint status, individual pharmacokinetics, and patient self-assessment and preference

- Recombinant activated factor VIIa, a bypassing agent, is recommended for the treatment and prevention of bleeding complications in patients with hemophilia B who develop FIX inhibitors

References:

1. CSL Behring. FDA accepts CSL Behring's biologics license application for etranacogene dezaparvovec for priority review. 2022 May 24. Available at: https://www.cslbehring.com/newsroom/2022/fda-bla-etranacogene-dezaparvovec. Accessed on August 15, 2022.
2. Figueiredo M. EtranaDez (etranacogene dezaparvovec) for hemophilia b. 2022 March 30. Available at: https://hemophilianewstoday.com/amt-061/. Accessed on August 15, 2022.
3. Clinicaltrials.gov. HOPE-B: Trial of AMT-061 in severe or moderately severe hemophilia b patients (NCT03569891). Available at: https://clinicaltrials.gov/ct2/show/NCT03569891. Accessed on Augusxt 15, 2022.
4. CSL Behring. uniQure and CSL Behring announce primary endpoint achieved in HOPE-B pivotal trial of etranacogene dezaparvovec gene therapy in patients with hemophilia B. 2021 Dec 9. Available at: https://www.cslbehring.com/newsroom/2021/hope-b-gene-therapy-for-hemophilia-b-topline-results. Accessed on August 15, 2022.

5. Shapiro AD. Hemophilia b. 2018. Available at: https://rarediseases.org/rare-diseases/hemophilia-b/. Accessed on August 15, 2022.
6. World Federation of Hemophilia. Guidelines for the management of hemophilia. Haemophilia. 2020 August 3. Available at: https://onlinelibrary.wiley.com/doi/epdf/10.1111/hae.14046. Accessed on: August 15, 2022.
7. Carcao M and Goudemand J. Inhibitors in hemophilia: a primer. 2018 Nov. Available at: https://www1.wfh.org/publication/files/pdf-1122.pdf. Accessed on August 15, 2022.

Policy History		
#	Date	Change Description
1.1	Effective Date: 12/05/2022	New Policy
1.0	Effective Date: 10/06/2022	Preliminary drug review

** The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or http://dailymed.nlm.nih.gov/dailymed/index.cfm.*