EC approves first gene therapy for Haemophilia B

The first gene therapy for haemophilia B has been given conditional marketing authorisation in Europe by the European Commission.

On February 20, 2023, A conditional marketing authorisation (CMA) has been granted by the European Commission (EC) for HEMGENIX® (etranacogene dezaparvovec), the first and only one-time gene therapy for haemophilia B (congenital Factor IX deficiency).

The treatment is indicated for severe and moderately severe forms of the condition in adults without a history of Factor IX inhibitors. HEMGENIX® is the first approved gene therapy for haemophilia B in the European Union (EU) and European Economic Area (EEA).

The EC's decision follows the CHMP's positive opinion in December 2022, based on findings from the pivotal HOPE-B trial, the largest gene therapy trial in haemophilia B to date.

According to the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP), there is an unmet medical need for new therapeutic approaches that might free patients from the burden of frequent infusions, or episodically at the time of a bleeding event.

HEMGENIX® is a single dose intravenous infusion that reduces the rate of abnormal bleeding by enabling the body to continuously produce Factor IX (FIX), a protein needed to form blood clots and stop bleeding. Specifically, HEMGENIX® utilizes a viral vector to deliver the genetic instructions for making Factor IX to target cells in the liver. Once delivered, the new genetic instructions remain in the target cells and generate factor IX proteins that are five to eight times more active than normal. Current Haemophilia B patients may require prophylactic intravenous infusions of factor IX up to several times per week. The approval of HEMGENIX® provides a one-time treatment alternative to the routine FIX injections.

The European Commission's decision follows the CHMP's positive opinion in December 2022, based on findings from the pivotal HOPE-B trial, the largest gene therapy trial in Haemophilia B to date.[4,5] These findings showed that Haemophilia B patients treated with HEMGENIX® demonstrated stable and durable increases in mean Factor IX activity levels (with a mean Factor IX activity of 36.9%) which led to an adjusted annualized bleed rate (ABR) reduction of 64%.[1] Following infusion of HEMGENIX®, 96% of patients discontinued routine Factor IX prophylaxis and mean Factor IX consumption was reduced by 97% at 18 months post-treatment, compared to the lead-in period.[1]

"Data from the HOPE-B study demonstrate the potential of HEMGENIX® to remove the need for routine prophylaxis, by providing durable Factor IX activity, as well as improved bleeding outcomes and quality of life," explained Professor Wolfgang Miesbach, Head of Coagulation Disorders at the Comprehensive Care Center, University Hospital of Frankfurt.

"This approval marks an important step forward in the treatment of haemophilia B, which could be transformative for people who are debilitated by bleeds into their muscles, joints and internal organs, alleviating the burden of lifelong intravenous infusions of Factor IX products," added Professor Miesbach.

The U.S. Food and Drug Administration (FDA) approved HEMGENIX® in November 2022.

References:
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3. European Medicines Agency. First Gene therapy to treat haemophilia B. Available at: https://www.ema.europa.eu/en/news/first-gene-therapy-treat-haemophilia-b. [Accessed February 2023].
4. European Medicines Agency. HEMGENIX Summary of Positive Opinion. Available at https://www.ema.europa.eu/en/medicines/human/summaries-opinion/HEMGENIX [Accessed February 2023].
5. ClinicalTrials.gov. HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Haemophilia B Patients. Available at: https://clinicaltrials.gov/ct2/show/NCT03569891. [Accessed February 2023].
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