Drugs

PEER-REVIEWED FEATURE

Etranacogene dezaparvovec : Adis Evaluation

Key Points

- An adeno-associated virus vector-based gene therapy being developed by uniQure and CSL Behring for the treatment of haemophilia B
- Received its first approval on 22 November 2022 in the USA
- Approved for the treatment of haemophilia B (congenital factor IX deficiency) in adults who are currently using FIX prophylaxis therapy, have current or historical life-threatening haemorrhage or have repeated, serious spontaneous bleeding episodes

Summary

Etranacogene dezaparvovec (etranacogene dezaparvovec-drlb; Hemgenix®) is an adeno-associated virus vector-based gene therapy being developed by uniQure and CSL Behring for the treatment of haemophilia B.

On 22 November 2022, etranacogene dezaparvovec was approved to be the first gene therapy available for the treatment of haemophilia B in the USA, where it is indicated in adults who are currently using factor IX (FIX) prophylaxis therapy, have current or historical life-threatening haemorrhage or have repeated, serious spontaneous bleeding episodes.

On 16 December 2022, etranacogene dezaparvovec received positive opinion in the EU for the treatment of moderate to severe haemophilia B in adults who do not have FIX inhibitors. It is currently undergoing clinical development for haemophilia B in the UK.

Y.-A. Heo Drugs. 2023. [https://doi.org/10.1007/s40265-023-01845-0]