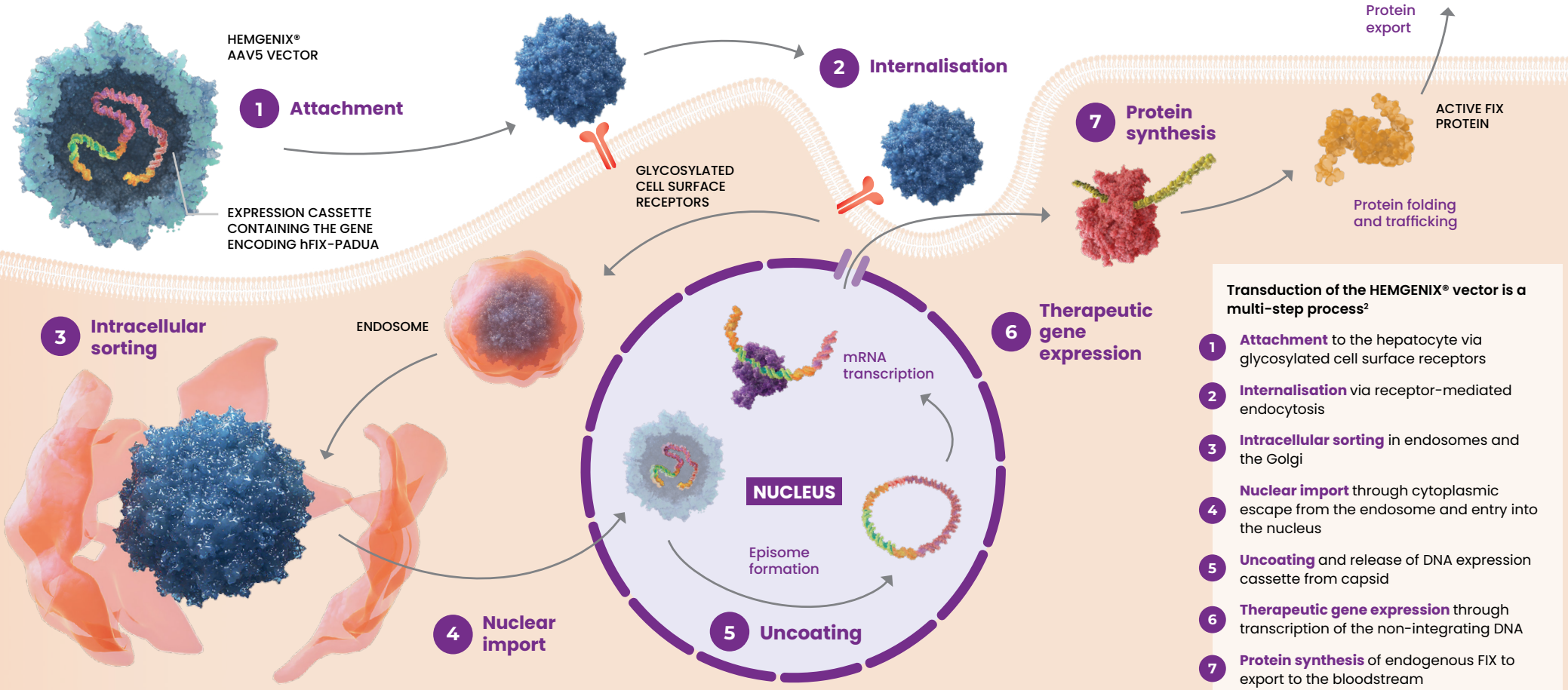
HEMGENIX®
etranacogene dezaparvovec

Mechanism of Action in Haemophilia B

HEMGENIX® is a gene therapy that uses a recombinant AAV5 vector to deliver a functional copy of the *F9* gene to the hepatocytes of eligible adults with severe or moderately severe haemophilia B. The vector is specifically designed to follow a multi-step process of *in vivo*, liver-directed gene transfer with the goal of achieving elevated and sustained endogenous expression of functional Factor IX through a single IV administration.[1,2]



Transduction of the HEMGENIX® vector is a multi-step process[2]

1. **Attachment** to the hepatocyte via glycosylated cell surface receptors
2. **Internalisation** via receptor-mediated endocytosis
3. **Intracellular sorting** in endosomes and the Golgi
4. **Nuclear import** through cytoplasmic escape from the endosome and entry into the nucleus
5. **Uncoating** and release of DNA expression cassette from capsid
6. **Therapeutic gene expression** through transcription of the non-integrating DNA
7. **Protein synthesis** of endogenous FIX to export to the bloodstream

HEMGENIX® (etranacogene dezaparvovec) is indicated for the treatment of severe and moderately severe haemophilia B (congenital Factor IX deficiency) in adult patients without a history of Factor IX inhibitors.[1]

Access the full HEMGENIX® prescribing information **here**. The adverse event reporting information can be found on the reverse.

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Prescribing Information

Click here for the HEMGENIX® prescribing information

Adverse events should be reported. Reporting forms and information can be found at www.mhra.gov.uk/yellowcard. Adverse events should also be reported to CSL Behring UK Ltd on 01444 447405.

AAV5, adeno-associated virus serotype 5; FIX, Factor IX; hFIX-Padua, Padua variant of human FIX; IV, intravenous.

REFERENCES

1. HEMGENIX® (etranacogene dezaparvovec). Summary of product characteristics.
2. Wang D *et al*. Adeno-associated virus vector as a platform for gene therapy delivery. *Nat Rev Drug Discov.* 2019;18(5):358–378.

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