swissmedic

Summary report on authorisation dated 11 February 2025

Hemgenix® (active substance: etranacogene dezaparvovec)

Authorisation in Switzerland: 7 December 2023

Gene therapy for the treatment of male adults with haemophilia B to reduce the frequency of bleeding

About the medicinal product

Hemgenix contains the active substance etranacogene dezaparvovec. This medicinal product is a gene therapy that is authorised for the treatment of male patients with severe or moderate haemophilia B. Haemophilia B is a hereditary disease in which there is a lack of factor IX, an important blood clotting protein. This results in an increased tendency to bleed, both internally, such as in joints or muscles, and also externally, such as with cuts or injuries. Life-threatening bleeds or repeated severe spontaneous bleeding can occur.

Since haemophilia B is a rare and life-threatening disease, the medicine has been authorised as an orphan drug. "Orphan drug" is a designation given to medicinal products for rare diseases.

Mode of action

In patients with haemophilia B, the liver is unable to produce factor IX, which is necessary for blood clotting, as the gene responsible for this factor IX is defective. Hemgenix can correct this genetic defect. The active substance in Hemgenix is based on a virus that does not cause illness in humans. This virus has been modified so that it does not spread within the body, but can transport a functional copy of the factor IX gene into the liver cells. The liver cells are then able to produce factor IX. This helps to normalise blood clotting and reduce the risk of bleeding.

Use

Hemgenix is a prescription-only medicine.

It is available as a concentrate for solution for infusion. The recommended dosage is 2 × 10^{13} genome copies per kilogram of body weight. The diluted solution is administered once as a slow infusion into a vein. The patient must be monitored by a doctor during and for at least 3 hours after administration of Hemgenix. Infusion-related reactions can occur, including hypersensitivity reactions.

Efficacy

The efficacy of Hemgenix was investigated in a trial with 54 male patients with moderate or severe haemophilia B. The patients received one dose of etranacogene dezaparvovec into a vein and were monitored for at least 18 months afterwards. The treatment resulted in a significant increase in factor IX activity, meaning that the patients' bleeding rate was reduced considerably and the need for factor IX replacement therapy declined.

The trial also showed a 64% reduction in the annual bleeding rate, from an average of 4.19 bleeds in the period before the administration of Hemgenix to an average of 1.51 bleeds in months 7-18 following treatment.

Precautions, undesirable effects, & risks

Hemgenix must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects are increased alanine aminotransferase (a liver enzyme) (18%), headache (16%), flu-like illness (14%) and reactions in connection with the infusion (12%). All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

There are currently no treatment options for adult men with severe or moderate haemophilia B, who suffer life-threatening bleeds or recurring severe spontaneous bleeds, which can cure the disease. The clinical trials have shown that Hemgenix can be used to reduce bleeding episodes and the need for factor IX replacement therapies. The treatment of haemophilia B patients with Hemgenix showed a significant reduction in the bleeding rate and complications.

Based on the available data and taking all the risks (such as an increase in liver enzymes) and precautions into account, the benefits of Hemgenix outweigh the risks. Swissmedic has therefore authorised the medicinal product Hemgenix, with the active substance etranacogene dezaparvovec, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Hemgenix®

Information for patients (package leaflet): Information for patients Hemgenix®

Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.