Medicines & Healthcare products Regulatory Agency

MHRA

Public Assessment Report

National Procedure

Hemgenix 1 x 10^{13} genome copies/mL concentrate for solution for infusion (etranacogene dezaparvovec)

PLGB 15036/0160

CSL Behring GmbH

LAY SUMMARY

Hemgenix 1 x 10^{13} genome copies/mL concentrate for solution for infusion (etranacogene dezaparvovec)

This is a summary of the Public Assessment Report (PAR) for Hemgenix 1 x 1013 genome copies/mL concentrate for solution for infusion. It explains how this product was assessed and its authorisation recommended, as well as its conditions of use. It is not intended to provide practical advice on how to use this product.

This product will be referred to as Hemgenix in this lay summary for ease of reading.

This product has been authorised by MHRA for Great Britain (consisting of England, Scotland and Wales). In coming to its decision, MHRA has relied on a European Commission (EC) decision on 20 February 2023 (EMEA/H/C/004827), in accordance with the advice from the Committee for Medicinal Products for Human Use (CHMP). This is known as the EC Decision Reliance Procedure.

This application was approved under Regulation 50 of the Human Medicines Regulation 2012, as amended (previously Article 8.3 of Directive 2001/83/EC, as amended).

For practical information about using Hemgenix, patients should read the Patient Information Leaflet (PIL) or contact their doctor or pharmacist.

What is Hemgenix and what is it used for?

Hemgenix is used for the treatment of severe and moderately severe Haemophilia B (congenital Factor IX deficiency) in adults who do not have current or past inhibitors (neutralising antibodies) against the Factor IX protein.

People with Haemophilia B are born with an altered form of a gene needed to make Factor IX, an essential protein required for blood to clot and stop any bleeding. People with Haemophilia B have insufficient levels of Factor IX and are prone to internal or external bleeding episodes.

How does Hemgenix work?

Hemgenix is a gene therapy product that contains the active substance etranacogene dezaparvovec. A gene therapy product works by delivering a gene into the body to correct a genetic defect.

The active substance in Hemgenix is based on a virus that does not cause disease in humans. This virus has been modified so that it cannot spread in the body but can deliver a copy of the Factor IX gene into the liver cells. This allows the liver to produce the Factor IX protein and raise the levels of working Factor IX in the blood. This helps the blood to clot more normally and prevents or reduces bleeding episodes.

How is Hemgenix used?

The pharmaceutical form of this medicine is a concentrate for solution for infusion.

Hemgenix will be given to the patient in a hospital setting under direction of a doctor experienced and trained in the treatment of the patient’s condition, Haemophilia B.

Hemgenix will be given to the patient only once by a single slow infusion (drip) into a vein. The infusion will take usually 1 to 2 hours to be completed.

The doctor will work out the correct dose for their patient, based on the patient's body weight.

Discontinuation of exogenous Factor IX treatment
- It may take several weeks before improved bleeding control becomes apparent after Hemgenix infusion, and you may need to continue your replacement therapy with exogenous Factor IX during the first weeks after Hemgenix infusion.
- The patient's doctor will regularly monitor the patient's blood for the Factor IX activity levels, i.e., weekly for at least first 3 months, and at regular intervals thereafter, and decide if and when the patient should receive, reduce, or stop their exogenous Factor IX therapy (see section 2 of the PIL).

For further information on how Hemgenix is used, refer to the PIL and Summary of Product Characteristics (SmPC) available on the Medicines and Healthcare products Regulatory Agency (MHRA) website.

This medicine can only be obtained with a prescription.

The patient should ask the administering healthcare practitioner if they have any questions concerning their medicine.

What benefits of Hemgenix have been shown in studies?
A study in 54 adult male patients with severe or moderately severe haemophilia B found that Hemgenix was more effective at reducing bleeding events than factor IX replacement therapy. The study compared the number of bleeding episodes patients had with factor IX replacement therapy during a 6-month period before receiving Hemgenix with the number experienced over a one-year period after achieving stable factor IX levels with Hemgenix. Data from the study showed that Hemgenix reduced the yearly bleeding rate from 4.2 to 1.5 bleeds per year. The study also found that Hemgenix was effective at increasing factor IX levels, with 96% of patients (52 out of 54) no longer needing factor IX replacement therapy for up to 2 years after the infusion.

What are the possible side effects of Hemgenix?
The most common side effects with Hemgenix (which may affect more than 1 in 10 people) are headache, increased levels of liver enzymes in the blood (alanine aminotransferase increased), increased levels of liver enzymes in the blood (aspartate aminotransferase increased), flu-like illness (Influenza-like illness), increased levels of C-reactive protein, a marker of inflammation, infusion related reaction (allergic reactions (hypersensitivity), infusion site reaction, dizziness, eye itching (pruritus), reddening of the skin (flushing), upper tummy (abdominal) pain, itchy rash (urticaria), chest discomfort, and fever.

For the full list of all side effects reported with this medicine, see Section 4 of the PIL or the SmPC available on the MHRA website.

If a patient gets any side effects, they should talk to their doctor, pharmacist or nurse. This includes any possible side effects not listed in the product information or the PIL that comes with the medicine. Patients can also report suspected side effects themselves, or a report can be made on their behalf by someone else who cares for them, directly via the Yellow Card

scheme at https://yellowcard.mhra.gov.uk or search for 'MHRA Yellow Card' online. By reporting side effects, patients can help provide more information on the safety of this medicine.

Why was Hemgenix approved?
At the time of approval, patients with severe haemophilia B required lifelong treatment with factor IX replacement therapy. Hemgenix, given as a single infusion, was effective at preventing bleeding over a period of at least 2 years, thus enabling patients to discontinue treatment with factor IX replacement therapy, which reduces the burden caused by treating the disease. There are some uncertainties about how long the benefits of Hemgenix last, given that the main ongoing study evaluated the response in a small number of patients for up to 2 years. Although the long-term safety data were limited, the safety profile was considered acceptable.

Hemgenix has been authorised with a conditional marketing authorisation (CMA). CMAs are intended for medicinal products that address an unmet medical need, such as a lack of alternative therapy for a serious and life-threatening disease. CMAs may be granted where comprehensive clinical data is not yet complete, but it is judged that such data will become available soon.

Hemgenix has been authorised with the condition to perform further studies and/or to provide additional measures to minimise the risk. See section below "What measures are being taken to ensure the safe and effective use of Hemgenix?"

Hemgenix has been authorised as a GB Orphan medicine. Orphan medicines are intended for use against rare conditions that are life-threatening or chronically debilitating. To qualify as an orphan medicine, certain criteria, for example concerning the rarity of the disease and the lack of currently available treatments, must be fulfilled.

The MHRA decided that the benefits are greater than the risks and recommended that this medicine can be approved for use.

What measures are being taken to ensure the safe and effective use of Hemgenix?
As for all newly authorised medicines, a Risk Management Plan (RMP) has been developed for Hemgenix. The RMP details the important risks of Hemgenix, how these risks can be minimised, any uncertainties about Hemgenix (missing information), and how more information will be obtained about the important risks and uncertainties.

The following safety concerns have been recognised for Hemgenix:

Summary of safety concerns	
Important identified risks	• Hepatotoxicity • Infusion reactions (including hypersensitivity)
Important potential risks	• Risk of malignancy in relation to vector integration in the DNA of body cells • Bleeding as a result of lack of efficacy due to immune-mediated neutralization of the AAV-5 vector capsid • Thromboembolic events • Germline transmission • Transmission to third parties (horizontal transmission) • Development of FIX inhibitors
Missing information	• Use in patients with severe hepatic impairment • Long-term effect • Use in female patients

The information included in the SmPC and the PIL is compiled based on the available quality, non-clinical and clinical data, and includes appropriate precautions to be followed by healthcare professionals and patients. Side effects of Hemgenix are continuously monitored and reviewed including all reports of suspected side-effects from patients, their carers, and healthcare professionals.

In addition to the safety information provided in the Hemgenix product information, the Marketing Authorisation Holder (MAH) has committed to (i) additional pharmacovigilance activities through post-authorisation studies to further evaluate the long-term effectiveness and safety of Hemgenix, (ii) additional risk minimisation activities through the provision of a Healthcare professional guide, patient guide and patient card.

An RMP and a summary of the pharmacovigilance system have been provided with this application and are satisfactory.

Other information about Hemgenix
A marketing authorisation was granted in Great Britain on 22 March 2023.

The full PAR for Hemgenix follows this summary.

This summary was last updated in May 2023.

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I. INTRODUCTION

Based on the review of the data on quality, safety and efficacy, the Medicines and Healthcare products Regulatory Agency (MHRA) considered that the application for Hemgenix 1 x 10^{13} genome copies/mL concentrate for solution for infusion (PLGB 15036/0160) could be approved. The product will be referred to as Hemgenix concentrate for solution for infusion in this report.

The product is approved for the following indication:

- the treatment of severe and moderately severe Haemophilia B (congenital Factor IX deficiency) in adult patients without a history of Factor IX inhibitors.

This product has been authorised by MHRA for Great Britain (consisting of England, Scotland and Wales). In coming to its decision, MHRA has relied on a European Commission (EC) decision on 20 February 2023 (EMEA/H/C/004827), in accordance with the advice from the Committee for Medicinal Products for Human Use (CHMP).

For the scientific discussion of the quality, non-clinical and clinical assessment conducted by the European Medicines Agency (EMA), please refer to the European Public Assessment Report, available on the EMA website.

This application was approved under Regulation 50 of the Human Medicines Regulation 2012, as amended (previously Article 8.3 of Directive 2001/83/EC, as amended).

This product has been authorised as a conditional marketing authorisation (CMA). CMAs are granted in the interest of public health and are intended for medicinal products that fulfil an unmet medical need and the benefit of immediate availability outweighs the risk posed from less comprehensive data than normally required. Unmet medical needs include, for example, treatment or diagnosis of serious and life-threatening diseases where no satisfactory treatment methods are available. CMAs may be granted where comprehensive clinical data is not yet complete, but it is judged that such data will become available soon. Adequate evidence of safety and efficacy to enable the MHRA to conclude that the benefits are greater than the risks is required and has been provided for Hemgenix concentrate for solution for infusion. The CMA for Hemgenix concentrate for solution for infusion, including the provision of any new information, will be reviewed every year and this report will be updated as necessary.

This application was evaluated for fulfilment of orphan designation criteria. It was concluded that fulfilment of the criteria for approval as an orphan medicinal product was satisfactorily demonstrated. Please see Annex 1 for a summary of the orphan approval.

In line with the legal requirements for children's medicines, the application included a licensing authority decision on the agreement of a paediatric investigation plan (PIP) (MHRA-100695-PIP01-22-M02). At the time of the submission of the application the PIP was not yet completed as some measures were deferred.

The MHRA has been assured that acceptable standards of Good Manufacturing Practice (GMP) are in place for this product at all sites responsible for the manufacture, assembly and batch release of this product.

A Risk Management Plan (RMP) and a summary of the pharmacovigilance system have been provided with this application and are satisfactory.

A marketing authorisation was granted on 22 March 2023.

II. PRODUCT INFORMATION

SUMMARY OF PRODUCT CHARACTERISTICS (SmPC)

The SmPC is in line with current guidelines and is satisfactory.

PATIENT INFORMATION LEAFLET (PIL)

The PIL is in line with current guidelines and is satisfactory.

LABEL

The labelling is in line with current guidelines and is satisfactory.

III. QUALITY ASPECTS

The MHRA considered that the quality data submitted for this application is satisfactory.

The grant of a marketing authorisation was recommended.

IV. NON-CLINICAL ASPECTS

The MHRA considered that the non-clinical data submitted for this application is satisfactory.

The grant of a marketing authorisation was recommended.

V. CLINICAL ASPECTS

The MHRA considered that the clinical data submitted for this application is satisfactory.

The grant of a marketing authorisation was recommended.

VI. RISK MANAGEMENT PLAN (RMP)

The applicant has submitted an RMP, in accordance with the requirements of Regulation 182 of The Human Medicines Regulation 2012, as amended. In addition to routine pharmacovigilance and risk minimisation measures, the following additional pharmacovigilance and risk minimisation measures have been proposed:

Table 1: Summary table of pharmacovigilance activities and risk minimization activities by safety concern

Safety concern	Risk minimization measures	Pharmacovigilance activities
Hepatotoxicity	Routine risk minimization measures: SmPC sections 4.2, 4.4, 4.8 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: Questionnaire on Liver toxicity Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_5001 • Study CSL222_3003 • Study CSL222_2001 • Study CSL222_3001
Infusion reactions (including hypersensitivity)	Routine risk minimization measures: SmPC sections 4.2, 4.4, 4.8 Legal status: Prescription only product. Additional risk minimization measures: None	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_2001 • Study CSL222_3001

Safety concern	Risk minimization measures	Pharmacovigilance activities
Risk of malignancy in relation to vector integration in the DNA of body cells	Routine risk minimization measures: SmPC section 4.2, 4.4 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: Questionnaire on Hemgenix Liver malignancy Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_3003 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001
Bleeding as a result of lack of efficacy due to immune -mediated neutralization of the AAV-5 vector capsid	Routine risk minimization measures: SmPC sections 4.2, 4.4, 5.1 Legal status: Prescription only product. Additional risk minimization measures: None	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_2001 • Study CSL222_3001
Thromboembolic events	Routine risk minimization measures: SmPC section 4.2., 4.4 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: Questionnaire on Thromboembolic Events (TEE) Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_3003 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001

Safety concern	Risk minimization measures	Pharmacovigilance activities
Germline transmission	Routine risk minimization measures: SmPC sections 4.2, 4.4, 4.6 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001
Transmission to third parties (horizontal transmission)	Routine risk minimization measures: SmPC sections 4.4, 5.2 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001
Development of FIX inhibitors	Routine risk minimization measures: SmPC sections 4.1, 4.2, 4.4, 4.8 Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide, patient guide and patient card	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_3003 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001
Use in patients with severe hepatic impairment	Routine risk minimization measures: SmPC sections 4.2, 4.3, 4.4, 4.5, 5.2 Legal status: Prescription only product. Additional risk minimization measures:	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: Questionnaire on Liver toxicity Additional pharmacovigilance activities: Study CSL222_4001

Safety concern	Risk minimization measures	Pharmacovigilance activities
	None	
Long-term effect	Routine risk minimization measures: SmPC section 4.2, 4.4 (risk of carcinogenicity) Legal status: Prescription only product. Additional risk minimization measures: Health care professional guide and patient guide	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: • Study CSL222_4001 • Study CSL222_3003 • Study CSL222_5001 • Study CSL222_2001 • Study CSL222_3001
Use in female patients	Routine risk minimization measures: SmPC section 4.2, 4.6 (Fertility, pregnancy and lactation) Legal status: Prescription only product. Additional risk minimization measures: None	Routine pharmacovigilance activities beyond adverse reactions reporting and signal detection: None Additional pharmacovigilance activities: Study CSL222_4001

This is acceptable.

VII. USER CONSULTATION

A full colour mock-up of the PIL has been provided with the application, in accordance with legal requirements.

The PIL has been evaluated via a user consultation study in accordance with legal requirements. The results show that the PIL meets the criteria for readability as set out in the Guideline on the readability of the label and package leaflet of medicinal products for human use.

VIII. OVERALL CONCLUSION, BENEFIT/RISK AND RECOMMENDATION

The quality of the product is acceptable. The non-clinical and clinical data submitted have shown the positive benefit/risk of this product in the treatment of severe and moderately severe Haemophilia B (congenital Factor IX deficiency) in adult patients without a history of Factor IX inhibitors.

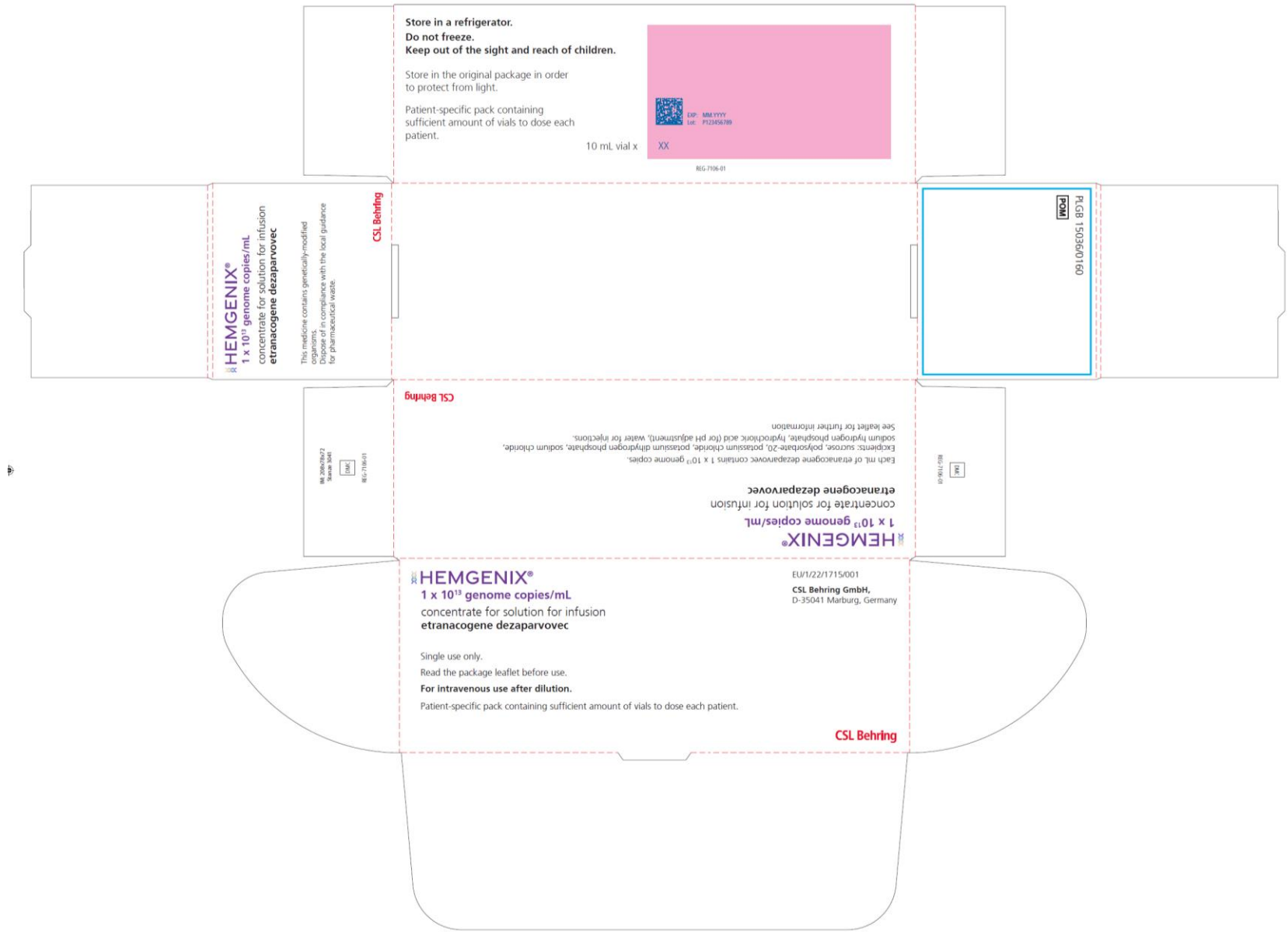
Hemgenix concentrate for solution for infusion has been authorised with a conditional marketing authorisation (CMA). The Marketing Authorisation Holder shall complete, within the stated timeframe, the following measures:

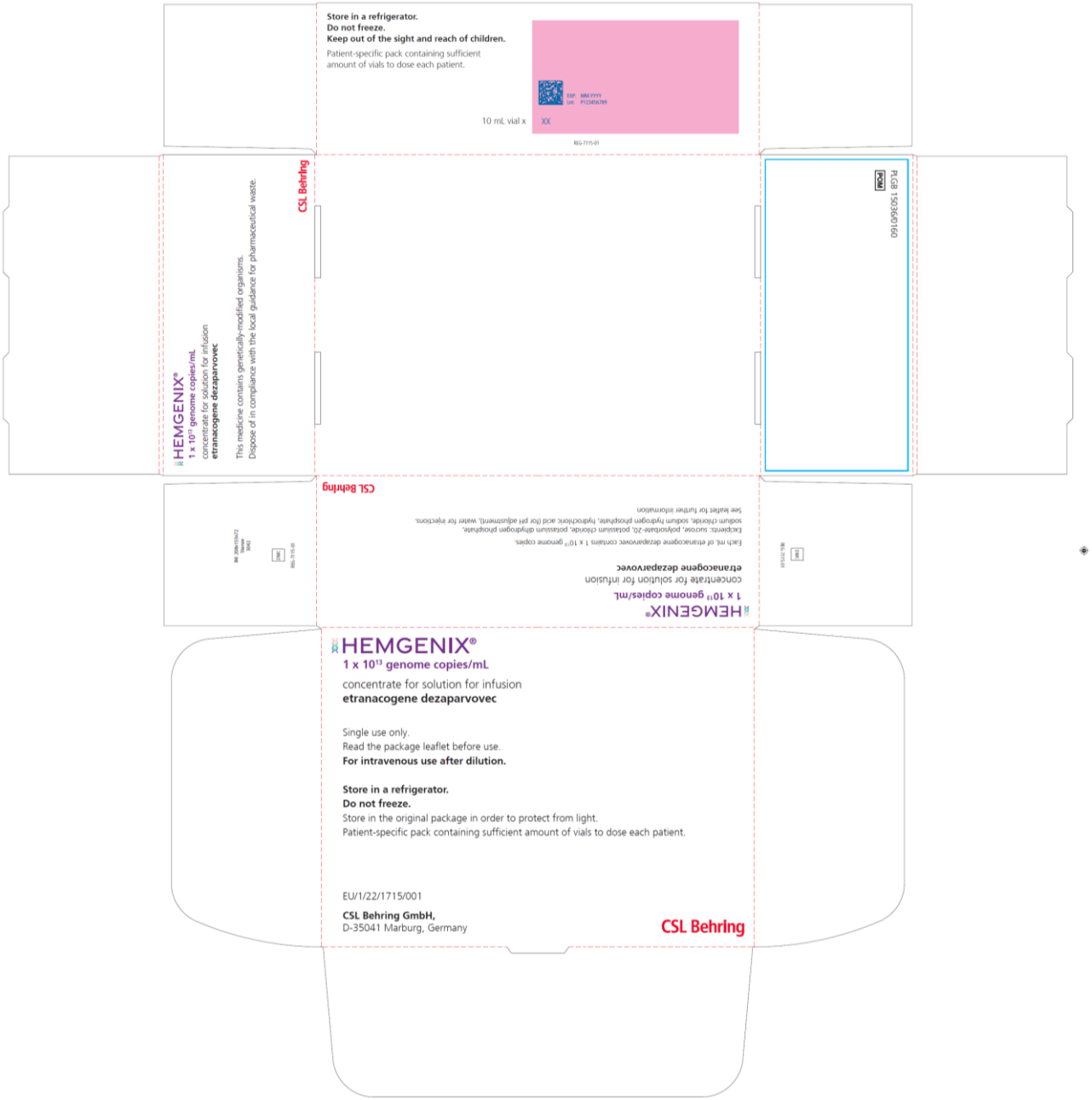
Description	Due date
1. In order to confirm the efficacy and safety of etranacogene dezaparvovec in adult patients with severe and moderately severe Haemophilia B (congenital Factor IX deficiency) without a history of Factor IX inhibitors, the MAH should submit the final results including 5 years follow-up of the pivotal Study CT-AMT-061-01.	30/06/2024
2. In order to confirm the efficacy and safety of etranacogene dezaparvovec in adult patients with severe and moderately severe Haemophilia B (congenital Factor IX deficiency) without a history of Factor IX inhibitors, the MAH should submit the final results (5 years of data) of pivotal Study CT-AMT-061-02 with 54 subjects.	31/10/2025
3. In order to confirm the efficacy and safety of etranacogene dezaparvovec in adult patients with severe and moderately severe Haemophilia B (congenital Factor IX deficiency) without a history of Factor IX inhibitors, irrespective of baseline anti-AAV5 neutralising antibody titre, the MAH should submit the 1-year follow-up interim analysis report after the first 50 subjects are enrolled in Study CSL222_4001.	31/12/2026

The SmPC, PIL and labelling are satisfactory.

In accordance with legal requirements, the current approved UK versions of the SmPC and PIL for this product are available on the MHRA website.

Representative copies of the labels at the time of UK licensing are provided below.


Store in a refrigerator.
Do not freeze.
Keep out of the sight and reach of children.
Store in the original package in order to protect from light.
Patient-specific pack containing sufficient amount of vials to dose each patient.
10 mL vial x XX
EXP: MM.YYYY
Lot: P123456789
REG-7106-01
HEMGENIX®
1 x 10¹³ genome copies/mL
concentrate for solution for infusion
etranacogene dezaparvovec
This medicine contains genetically-modified organisms.
Dispose of in compliance with the local guidance for pharmaceutical waste.
CSL Behring
PLGB 15036/0160
POM
Each mL of etranacogene dezaparvovec contains 1 x 10¹³ genome copies.
Excipients: sucrose, polysorbate-20, potassium chloride, potassium dihydrogen phosphate, sodium chloride, sodium hydrogen phosphate, hydrochloric acid (for pH adjustment), water for injections.
See leaflet for further information
HEMGENIX®
1 x 10¹³ genome copies/mL
concentrate for solution for infusion
etranacogene dezaparvovec
EU/1/22/1715/001
CSL Behring GmbH,
D-35041 Marburg, Germany
Single use only.
Read the package leaflet before use.
For intravenous use after dilution.
Patient-specific pack containing sufficient amount of vials to dose each patient.
CSL Behring


Store in a refrigerator.
Do not freeze.
Keep out of the sight and reach of children.
Patient-specific pack containing sufficient amount of vials to dose each patient.
10 mL vial x XX
EXP MM.YYYY
Lot P12345678
HEMGENIX®
1 x 10¹³ genome copies/mL
concentrate for solution for infusion
etranacogene dezaparvovec
This medicine contains genetically-modified organisms.
Dispose of in compliance with the local guidance for pharmaceutical waste.
CSL Behring
PLGB 15036/0160
POM
HEMGENIX®
1 x 10¹³ genome copies/mL
concentrate for solution for infusion
etranacogene dezaparvovec
Single use only.
Read the package leaflet before use.
For intravenous use after dilution.
Store in a refrigerator.
Do not freeze.
Store in the original package in order to protect from light.
Patient-specific pack containing sufficient amount of vials to dose each patient.
EU/1/22/1715/001
CSL Behring GmbH,
D-35041 Marburg, Germany
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Steps taken after the initial procedure with an influence on the Public Assessment Report (non-safety variations of clinical significance).

Please note that only non-safety variations of clinical significance are recorded below and in the annexes to this PAR. The assessment of safety variations, where significant changes are made, is recorded on the MHRA website or European Medicines Agency (EMA) website. Minor changes to the marketing authorisation are recorded in the current SmPC and/or PIL available on the MHRA website.

Application type	**Scope**	**Product information affected**	**Date of grant**	**Outcome**	**Assessment report attached Y/N**

ANNEX 1

Summary of fulfilment of the criteria for orphan drug designation

Product: Hemgenix 1 x 10^{13} genome copies/mL concentrate for solution for infusion
Active substance: Etranacogene dezaparvovec
Orphan Designation Number: PLGB 15036/0160/OD1
Background:

This application was evaluated for fulfilment of orphan designation criteria and the designation criteria were considered fulfilled.

Evaluation:

Orphan condition
The orphan condition is Haemophilia B.

Orphan indication
The orphan indication is the treatment of severe and moderately severe Haemophilia B (congenital Factor IX (FIX) deficiency) in adult patients without a history of Factor IX inhibitors.

Life threatening/debilitating condition
Chronic debilitating joint disease in patients with haemophilia results from recurrent bleeding into the joint, synovial membrane inflammation, hypertrophy and, eventually, destructive arthritis. Chronic joint deformities may occur that need to be managed by an orthopaedic specialist, and joint replacement may be needed. Current therapy with FIX replacement products reduces the risk for spontaneous bleeds but do not eliminate them completely. Breakthrough bleeding particularly in the joints and muscles still occur in many patients on prophylaxis when the factor activity levels are low.

When untreated, persons with haemophilia have a life expectancy of about 25 years. With current therapies, persons with haemophilia have nearly normal life expectancy yet deaths still occur at higher rates due to bleeding episodes. In severe haemophilia, all-cause mortality exceeded mortality in the general population by a factor of 2.69 and median life expectancy in severe haemophilia was 63 years (15 years less than that of the general male population).

The life-threatening and seriously debilitating nature of the condition may be agreed.

Prevalence of the Condition in Great Britain (GB)
Suitable evidence has been provided that demonstrates that, at the time of orphan designation, the condition affects less than 5 in 10,000 people in GB. This does not exceed the upper limit of prevalence for orphan designation, which is 5 in 10,000 people in GB.

Existing methods of prevention/treatment
The following methods have been identified:

Haemophilia B is currently managed by intravenous injections of the deficient clotting factor, Factor IX, either at the time of a bleed (on-demand) or by regular infusions several times a week (prophylactically).

The approved recombinant factor IX products in GB are BeneFIX, Idelvion, Alprolix, Refixia, Rixubis, Replenine and NovoSeven.

Justification of significant benefit
Methods for the treatment of the orphan condition already exist in GB.

Suitable justification has been provided that Hemgenix 1 x 10^{13} genome copies/mL concentrate for solution for infusion provides a significant benefit to those affected by the condition as specified in the orphan indication, on the basis of long-term efficacy and (known) safety.

A single administration of the current product has the potential to much reduce/stop bleeding episodes in those with haemophilia B (and obviate the need for administration of parenteral factor IX) and so reduce long-term consequences that include chronic joint disease. A 'one-off' administration is considered to be a major contribution to patient care based on a comparison to existing treatments that require repeat administration of factor IX product.

Clinical studies submitted by the company demonstrate superiority of etranacogene dezaparvovec compared to standard of care routine FIX prophylaxis in reducing annualised bleed rates.

Conclusion:

Conclusion on acceptability of orphan designation
The applicant has demonstrated fulfilment of the criteria for approval as an orphan medicinal product.

All medicines that gain an orphan marketing authorisation from the UK Licensing Authority are listed on its publicly available Orphan Register until the end of the market exclusivity period. The authorised orphan indication defines the scope of orphan market exclusivity.

Decision: Grant

Date: 22 March 2023