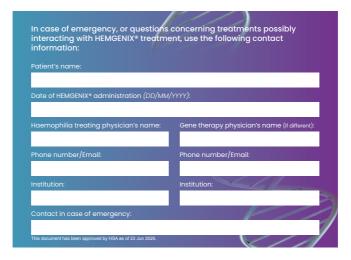


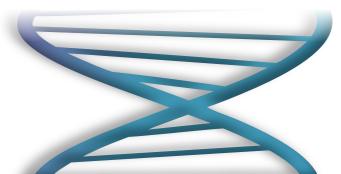


Read this patient/caregiver guide carefully before you receive **HEMGENIX®** because it contains important information for you.

In addition to this guide, your doctor will give you a Patient Card. Read it carefully and follow the instructions on it.



Keep your Patient Card with you at all times and present it to any doctor, or other healthcare professional you consult. This card contains important information for your safety related to the followup of this therapy that you and healthcare professionals need to know before taking care of you.



## WHAT CAN YOU FIND IN THIS PATIENT/ CAREGIVER GUIDE?

1. What is HEMGENIX® and what is it used for? Page 2. How does HEMGENIX® work? 1 3. What you need to know before Page you are administered HEMGENIX® 2-8 a. Liver health b. Abnormal clotting of blood (thromboembolic events) c. Risk of cancer potentially associated with **HEMGENIX®** d. Transmission of HEMGENIX® e. Development of Factor IX (FIX) inhibitors f. Pre-existing immunity against the vector g. Infusion-related reactions h. Response to treatment 4. What is the Patient Card for? Page

Page

5. What should I do if I get any side effects?

# What is HEMGENIX® and what is it used for?

**HEMGENIX**<sup>®</sup> is a gene therapy product that contains the active substance etranacogene dezaparvovec.

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.

**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 antibodies against Factor IX protein, called Factor IX inhibitors.

## How does HEMGENIX® work?

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.

# What you need to know before you are administered HEMGENIX®

It is important that you fully understand the benefits and risks of HEMGENIX® treatment, what is known and not yet known about the long-term effects of this therapy, related to both safety and efficacy. Important information about HEMGENIX® treatment is provided in the sections below. Read it carefully and ask your doctor or other healthcare professional if you have further questions.



### BEFORE TREATMENT WITH HEMGENIX®

To decide if this therapy is suitable for you, your doctor will check the status of your liver health before you start treatment with **HEMGENIX**® using:

- Blood tests to check the level of liver enzymes in your blood
- Liver ultrasound
- Elastography testing to check for scarring or thickening of your liver.

### **AFTER TREATMENT WITH HEMGENIX®**

After treatment with **HEMGENIX®**, your doctor will continue to check your health. It is **important** that you **discuss the schedule for these blood tests** with your doctor so that they can be carried out as necessary.

HEMGENIX® will trigger a response within your immune system that could lead to transaminitis, an increased level of certain liver enzymes (transaminases) in your blood, and reduce the efficacy of HEMGENIX®. Your doctor will regularly monitor your liver enzyme levels and Factor IX levels to ensure that the therapy is working as it should:



### **A** IMPORTANT

- If you experience an increase in liver enzymes, you may have more frequent blood tests to check your liver enzyme levels and your Factor IX levels, until your liver enzyme levels return to normal.
- You may also need to take another medicine (e.g., corticosteroids) to manage these side effects.
- You should inform your healthcare professional about current use
  of corticosteroids or other immunosuppressants. If you cannot take
  corticosteroids, your doctor may recommend alternative medicines
  to manage problems with the liver.
- Your doctor may also perform additional tests to exclude other causes for the increase in your liver enzymes, if needed, in consultation with a doctor experienced in liver diseases.

Both before and after treatment, it is recommended that you avoid taking medication that can cause liver damage. Tell your doctor or other healthcare professional if you are taking, have recently taken or might take any other medicines. If you are taking medications that are known to damage the liver (hepatotoxic medications), your doctor may decide that you may need to stop these medications to be able to receive **HEMGENIX**®.

# Abnormal clotting of blood (thromboembolic events)

As you have Haemophilia B, you usually have a reduced risk for developing a blood clot [e.g., blood clot in the lung (pulmonary thromboembolism) or in a blood vessel of the leg (deep venous thrombosis)] due to inborn deficiency in your blood clotting system.

Restoring Factor IX activity may bring your risk of blood clots up to a level similar to that experienced by the general non-haemophilic population.

If you have pre-existing risk factors for abnormal blood clotting, such as a history of heart disease, thick and stiff blood vessels (arteriosclerosis), hypertension, diabetes, or advanced age, the potential risk of abnormal blood clotting may be higher.

### Be aware of signs of abnormal clotting:



If you observe signs of abnormal clotting: Consult your doctor immediately



# Risk of cancer potentially associated with HEMGENIX®

**HEMGENIX®** will insert into liver cells and it could possibly insert into the liver cell DNA or the DNA of other body cells. As a consequence, **HEMGENIX®** could contribute to a risk of cancer, such as liver cancer (hepatocellular carcinoma). Although there is no evidence of this in the clinical studies so far, this remains possible because of the nature of the therapy, which has a viral component. You should therefore discuss this with your doctor.

 If you are a patient with pre-existing risk factors for liver cancer, your doctor will regularly (e.g., annually) monitor your long-term liver health for at least 5 years after HEMGENIX® administration and perform the following tests:

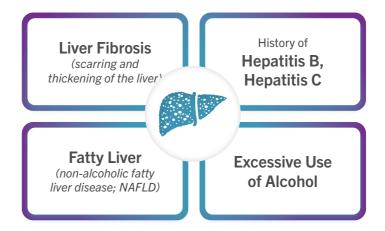


Annual liver ultrasound and



Annual blood test to check for increases in alpha-fetoprotein

### Some risk factors for liver cancer are:



 In the event of cancer, your doctor may take a sample of your cancer (biopsy) to check if HEMGENIX® has inserted into the cell DNA.

# Transmission of HEMGENIX®

The active substance in **HEMGENIX®** may temporarily be excreted through your blood, semen, breast milk or bodily waste - a process called shedding.

To ensure that people without Haemophilia B are not exposed to **HEMGENIX®** DNA through the shedding process in your body and/or semen, **you must not donate blood, semen, organs, tissues** and cells for transplantation after treatment with **HEMGENIX®**.

After a male patient has been treated with **HEMGENIX®**, the patient and any female partner **must avoid pregnancy for 12 months**. You should use **effective contraception** (e.g., barrier contraception such as condom or diaphragm). This is to minimise any risk of the Factor IX gene from a father's **HEMGENIX®** treatment being transmitted to a child with unknown consequences. For the same reason, male patients must not donate semen. **Discuss with your doctor which methods of contraception are suitable**.

**HEMGENIX®** should not be used in women who are pregnant, and is not recommended in women who may become pregnant. **HEMGENIX®** should not be used during breast feeding.



### BEFORE TREATMENT WITH HEMGENIX®

Neutralising antibodies against Factor IX (FIX) proteins, called FIX inhibitors, may stop **HEMGENIX®** from working properly. Your doctor will check for these before treatment with **HEMGENIX®**. If you test positive for these antibodies, another test will be performed in approximately 2 weeks. You will not be administered **HEMGENIX®** if both the initial test and re-test results are positive.

### AFTER TREATMENT WITH HEMGENIX®

Your doctor may check your blood for these FIX inhibitors if your bleeds are not controlled or return after you have been administered **HEMGENIX®**.



# Pre-existing immunity against the vector

Some people may have natural pre-existing "immunity" (i.e., antibodies) against the adeno-associated virus (AAV) vectors used to make  $\mathbf{HEMGENIX}^{\circledcirc}$  — this may stop the Factor IX gene from being delivered effectively.

High pre-existing immunity against the vector may reduce the efficacy of HEMGENIX®.

Therefore, your doctor will check for your level of pre-existing neutralising anti-AAV5 antibodies before treatment with HFMGFNIX®



### Infusion-related reactions

Infusion-related reactions can occur during or shortly after you are administered **HEMGENIX®**. Your doctor will monitor you during the infusion and for at least 3 hours after you are administered **HEMGENIX®**. Tell your doctor or nurse immediately if you experience these or any other symptoms during or shortly after the infusion:



Allergic Reactions



Upper abdominal pain



Infusion Site Reaction



Itchy rash (urticaria)



Dizziness



Chest discomfort



Eye Itching



Fever



Reddening of the skin (flushing)

Depending on your symptoms, your infusion may be slowed down or interrupted. If your infusion is interrupted, it can be restarted at a slower rate when the reaction has resolved. Your doctor may also consider if you need treatment (e.g., a corticosteroid or antihistamine) to manage the reaction.



### Response to treatment

There is a possibility that not all patients benefit from treatment with **HEMGENIX®**.

Patients who do not respond to **HEMGENIX®** are still exposed to the long-term risks outlined above.

**HEMGENIX**® will not be re-administered if you have not responded to treatment or if you have lost the response.

### What is the Patient Card for?

The Patient Card contains important safety information that you and any healthcare professional may need to know after treatment with **HEMGENIX**®.

As explained in this guide, certain recommendations must be followed for safe use of **HEMGENIX®** and it is therefore essential to share this information with all healthcare professionals you may need to consult. This is the role of the Patient Card.

- Your doctor should give you a HEMGENIX® Patient Card on the day of HEMGENIX® administration
- Carry it with you at all times; you can keep it in your wallet or purse
- Show the Patient Card to any doctor or healthcare professional whenever you have a medical appointment.
- If you need to go to Accident & Emergency, show the card as soon as you arrive
- Tell your caregiver or anyone close to you about your treatment and show them the Patient Card because they may notice side effects that you are not aware of

This card also reminds you of important information you need to know about significant risks of **HEMGENIX®**.

In case of emergency, or questions concerning treatments possibly interacting with HEMGENIX® treatment, use the following contact information:	
Patient's name:	
Date of HEMGENIX® administration (DD/MM,	/yyyy):
Haemophilia treating physician's name:	Gene therapy physician's name (if different):
Phone number/Email:	Phone number/Email:
Institution:	Institution:
institution.	institution.
Contact in case of emergency:	
This document has been approved by HSA as of 23 Jun 2025.	

# What should I do if I get any side effects?

If you get any side effects, talk to your doctor or any other healthcare professional. This includes any possible side effects not listed in this patient/caregiver guide. By reporting side effects, you can help provide more information on the safety of this therapy.

# NOTE PAGES

# NOTE PAGES



# CSL Behring CSL Behring Pte. Ltd. Level 14, unit 03/03A, 300 Beach Road,

The Concourse, Singapore 199555 Phone: +65 6390 9400 SGEnquiry@cslbehring.com