

FIRST AND ONLY FDA APPROVED GENE THERAPY FOR HEMOPHILIA B

GENE THERAPY EDUCATIONAL PROGRAM

Patient portrayal; HEMGENIX not intended for women



LIVE EDUCATIONAL PROGRAM

This educational program offers you and your family members the opportunity to:

- Learn about HEMGENIX, the first and only gene therapy approved for hemophilia B
- Gain insightful information and deepen your understanding of HEMGENIX through a LIVE Q&A session
- Ask questions and receive new resources on gene therapy

December 15, 2023

Check-in

6:30 PM ET

Program

7:00 PM ET

Partnering Organization

Bleeding Disorders Association of South
Carolina

RSVP by December 11, 2023

RSVP to Sue Martin: sue.martin@bda-sc.org

Guy Young, MD

Children's Hospital Los Angeles
Los Angeles, CA

Lowcountry Public House

Room Name: Prohibition
1426 Central Avenue
Summerville, SC 29483

Additional Information

IMPORTANT SAFETY INFORMATION

What is HEMGENIX?

HEMGENIX[®], etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

IMPORTANT SAFETY INFORMATION (cont.)

What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

Please see full prescribing information for HEMGENIX.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

www.CSLBehring.com

www.HEMGENIX.com

HEMGENIX is manufactured by uniQure, Inc. and distributed by CSL Behring LLC.
HEMGENIX® is a registered trademark of CSL Behring LLC.

©2023 CSL Behring LLC 1020 First Avenue, PO Box 61501, King of Prussia, PA 19406-0901 USA
USA-HGX-0015-APR23

CSL Behring