CSL and uniQure Win 2023 Prix Galien USA Award

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HEMGENIX®, the first and only FDA-approved gene therapy for adults with hemophilia B, wins for Best Product for Rare/Orphan Diseases

KING OF PRUSSIA, Pa. and LEXINGTON, Mass., Oct. 27, 2023 /PRNewswire/ -- Global biotechnology leader CSL (ASX: CSL) and uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that the companies have received the 2023 Prix Galien USA Award in the category of Best Product for Rare/Orphan Diseases for HEMGENIX® (etranacogene dezaparvovec-drlb).

CSL received approval from the U.S. Food and Drug Administration (FDA) for HEMGENIX in November 2022. It is the first and only 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.

"CSL is humbled and honored to receive a Prix Galien award for HEMGENIX," said Dr. Bill Mezzanotte, Head of Research and Development, CSL. "We thank The Galien Foundation for acknowledging the enormous promise and hope that HEMGENIX offers to eligible people living with hemophilia B."

The multi-year clinical development of HEMGENIX was led by uniQure and sponsorship of the clinical trials transitioned to CSL after it licensed global rights to commercialize the treatment.

"HEMGENIX represents a major milestone in the field of genomic medicine and ushers in a new treatment paradigm for eligible people living with hemophilia B," said Matt Kapusta, Chief Executive Officer, uniQure. "It gives us great pride that after many years of development, this important gene therapy is now being made available by CSL to patients in geographies around the world."

HEMGENIX also has been granted conditional marketing authorization by the European Commission (EC) for the European Union and European Economic Area, and the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA), as well as authorization by Health Canada.

The Prix Galien USA is America's preeminent prize acknowledging the leading-edge of scientific advances in life sciences.

About Hemophilia B
Hemophilia B is a life-threatening rare disease caused by a mutation on the F9 gene, resulting in low levels of functional clotting factor IX. People with the condition are particularly vulnerable to bleeds in their joints, muscles, and internal organs, leading to pain, swelling, and joint damage. Current treatments for moderate to severe hemophilia B include life-long prophylactic infusions of factor IX to temporarily replace or supplement low levels of the blood-clotting factor.

About HEMGENIX®
HEMGENIX® is a gene therapy that reduces the rate of abnormal bleeding in eligible people with hemophilia B by enabling the body to continuously produce factor IX, the deficient protein in hemophilia B. It uses AAV5, a non-infectious viral vector, called an adeno-associated virus (AAV). The AAV5 vector carries the Padua gene variant of Factor IX (FIX-Padua) to the target cells in the liver, generating factor IX proteins that are 5x-8x more active than normal. These genetic instructions remain in the target cells, but generally do not become a part of a person's own DNA. Once delivered, the new genetic instructions allow the cellular machinery to produce stable levels of factor IX.

Important Safety Information (ISI)

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.

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.

About CSL
CSL (ASX:CSL; USOTC:CSLLY) is a leading global biotechnology company with a dynamic portfolio of lifesaving medicines, including those that treat hemophilia and immune deficiencies, vaccines to prevent influenza, and therapies in iron deficiency, dialysis and nephrology. Since our start in 1916, we have been driven by our promise to save lives using the latest technologies. Today, CSL – including our three businesses, CSL Behring, CSL Seqirus and CSL Vifor – provides lifesaving products to patients in more than 100 countries and employs 30,000 people. Our unique combination of commercial strength, R&D focus and operational excellence enables us to identify, develop and deliver innovations so our patients can live life to the fullest. For inspiring stories about the promise of biotechnology, visit CSLBehring.com/Vita and follow us on Twitter.com/CSL.

For more information about CSL, visit www.CSL.com.

About uniQure
uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy for hemophilia B – a historic achievement based on more than a decade of research and clinical development – represents a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (ALS), Fabry disease, and other severe diseases. www.uniQure.com

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