CSL Behring
Biotherapies for Life™

CSL Behring Agrees to Acquire Novel Late-Stage Gene Therapy Candidate for Hemophilia B Patients from uniQure

KING of PRUSSIA, Pa.

- Unique gene therapy has the potential to be one of the first to market treatments to provide potentially long-term benefits with only one dose
- CSL Behring builds on legacy of delivering innovative treatment options for people with Hemophilia B

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KING OF PRUSSIA, Pa., – 24 June 2020 – Global biotherapeutics leader CSL Behring announced today that it has agreed to acquire exclusive global license rights to commercialize an adeno-associated virus (AAV) gene therapy program, AMT-061 (etranacogene dezaparvovec), for the treatment of hemophilia B from uniQure (NASDAQ: QURE), a leading gene therapy company. The AMT-061 program, currently in Phase 3 clinical trials, could be one of the first gene therapies to provide potentially long-term benefits to patients with hemophilia B.

One dose of AMT-061 has shown to increase Factor IX (FIX) plasma levels – the blood clotting protein lacking in people with hemophilia B – to a degree that reduces or eliminates the tendency for bleeding for many years. Should AMT-061 be successful, appropriate candidate hemophilia B patients would be able to have a one-time treatment to restore FIX activity to functional levels capable of eliminating the need for frequent and ongoing replacement therapies.

“Our vision with hemophilia B patients is to offer transformational treatment paradigms that help free them from the lifelong burden of this disease,” said CSL’s CEO and Managing Director Paul Perreault. “With more than three decades of providing lifesaving innovations for the global bleeding disorders community, we are well positioned to maximize the potential benefit of this therapy.”

Under the agreement with uniQure, upon closing the transaction CSL Behring will have the exclusive global right to commercialize AMT-061. uniQure will receive an upfront cash payment of US$450 million followed by regulatory and commercial sales milestone payments and royalties. Under the terms of the agreement, uniQure will complete the Phase 3 trial and scale up manufacture for early commercial supply under an agreed plan with CSL Behring. The transaction is subject to customary regulatory clearances before closing.

"We are thrilled to enter into this commercialization and license agreement with CSL Behring, an ideal commercial partner with global reach and decades of expertise in hemophilia," stated Matt Kapusta, chief executive officer of uniQure. "We believe that through this arrangement, we are ideally positioned to deliver our innovative gene therapy to the largest number of hemophilia B patients as quickly as possible. The transaction represents a major milestone in the development of etranacogene dezaparvovec and, when closed, we expect that it will provide uniQure with significant financial resources to advance and expand our pipeline of gene therapy candidates, anchored by AMT-130 in Huntington's disease, as well as to invest further in our leading manufacturing and technology platform."

In December 2019, uniQure announced that data from its Phase 2b dose-confirmation study of AMT-061 showed that all patients stabilized and sustained FIX activity at functionally high levels one year after a single dose – with increases in FIX activity of up to 50% of normal and a mean of 41%. This exceeds the levels considered sufficient to eliminate or significantly reduce the risk of bleeding events.

According to CSL Behring's Executive Vice President, Head of Research and Development and Chief Medical Officer Bill Mezzanotte, "We are exceedingly encouraged by the data we've seen on AMT-061. Not only has the treatment option demonstrated robust clinically meaningful responses in FIX activity, but it has also exhibited excellent safety over multiple years of observation. Expanding our gene therapy portfolio to treat hemophilia B, a disease state well known to CSL Behring, exemplifies how we are strategically aligning our rare and serious disease focus and our targeted therapeutic area focus with our core scientific platforms to transform the lives of patients."

This acquisition will also enhance CSL Behring's capabilities in its growing gene therapy portfolio. The company is currently developing a stem cell gene therapy

(CSL200) for the treatment of sickle cell disease and has recently established an alliance with Seattle Children's Research Institute to develop a stem cell gene therapy for primary immunodeficiency diseases -- another rare disease area where CSL Behring has leading capabilities.

Perreault added, "Upon approval, this next-generation therapy will be highly complementary to our existing best-in-class hemophilia B product portfolio with an alternate best-in-class treatment option. With the license to AMT-061, we are building on our legacy of delivering lifesaving innovations in hematology where, today, we offer a market leading product for hemophilia B and we are a leader in therapies for treating hemophilia A, von Willebrand disease, thrombosis, and other life-threatening conditions."

CSL Behring has put patients first by addressing the world's most serious, complicated and rare diseases for over 100 years. The company is now bringing that same commitment to gene therapy; its mission is to address unmet patient needs and enable patients to get the very most out of life.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec, also known as AMT-061, consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). AAV5-based gene therapies have been demonstrated to be safe and well tolerated in many clinical trials, including four uniQure trials conducted in 25 patients in hemophilia B and other indications. No patient treated in clinical trials with uniQure's AAV5-based gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

About CSL Behring

CSL Behring is a global biotherapeutics leader driven by its promise to save lives. Focused on serving patients' needs by using the latest technologies, we develop and deliver innovative therapies that are used to treat coagulation disorders, primary immune deficiencies, hereditary angioedema, respiratory disease, and neurological disorders. The company's products are also used in cardiac surgery, burn treatment and to prevent hemolytic disease of the newborn.

CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. The parent company, CSL Limited (ASX:CSL;USOTC:CSLLY), headquartered in Melbourne, Australia, employs more than 26,000 people, and delivers its life-saving therapies to people in more than 100 countries. For inspiring stories about the promise of biotechnology, visit Vita CSLBehring.com/vita and follow us on Twitter.com/CSLBehring.

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