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CSL

ASX Announcement

For immediate release

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CSL Agrees to Acquire Late-Stage Gene Therapy Candidate for Haemophilia B from uniQure

CSL Limited (ASX:CSL; USOTC:CSLLY) today announces that CSL has agreed to acquire from uniQure exclusive global license rights to commercialise an adeno-associated virus (AAV) gene therapy program, AMT-061 (etranacogene dezaparvovec), for the treatment of haemophilia B. 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 haemophilia B.

One dose of AMT-061 has shown to increase Factor IX (FIX) plasma levels – the blood clotting protein lacking in people with haemophilia B – to a degree that reduces or eliminates the tendency for bleeding for many years. Should AMT-061 be successful, appropriate candidate haemophilia 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 for haemophilia 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 maximise the potential benefit of this therapy.”

Under the agreement with uniQure, upon closing the transaction CSL will have the exclusive global right to commercialise AMT-061. uniQure (NASDAQ: QURE), a leading gene therapy company, 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.

The transaction is subject to customary regulatory clearances before closing.

Mr. Perreault added, “Upon approval, we believe this next-generation therapy would be highly complementary to our existing haemophilia B product portfolio. We hope that it provides patients with an alternate best-in-class treatment option, building on our legacy of delivering lifesaving innovations in hematology.”

This acquisition will also enhance CSL’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.

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.

Authorised by
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FURTHER INFORMATION

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