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CSL

ASX Announcement

For immediate release

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CSL Closes Commercialisation and License Agreement for Novel Late-Stage Gene Therapy Candidate from uniQure

CSL Limited (ASX:CSL; USOTC:CSLLY) announces the closing of CSL's global commercialisation and license agreement with uniQure (NASDAQ: QURE) for etranacogene dezaparvovec (AMT-061), a novel gene therapy for the treatment of haemophilia B. Etranacogene dezaparvovec is currently in Phase 3 clinical trials and has been shown to result in near-normal levels of Factor IX (FIX) – the blood clotting protein lacking in people with haemophilia B.

The global licensing agreement was announced on 24 June 2020 and the closing follows the completion of antitrust reviews of the transaction in the US, the UK and Australia. Under the terms of the agreement, uniQure will receive an upfront cash payment of US$450 million by 13 May, 2021, with the potential for regulatory and commercial sales milestone payments and royalties as the therapy is developed and commercialised. uniQure will complete the Phase 3 HOPE-B trial and scale up manufacture for initial commercial supply while CSL Behring will be responsible for regulatory submissions and commercialisation.

"We are continuing to build on our legacy of delivering lifesaving innovations in haematology with today's news. This agreement enables us to take forward a gene therapy that, if approved, has the potential to transform the lives of haemophilia B patients," said CSL CEO and Managing Director Paul Perreault. "Etranacogene dezaparvovec has the potential to be the first-ever gene therapy approved for haemophilia B and help CSL Behring deliver on our ongoing commitment to improving the lives of those living with haemophilia B."

The acquisition complements both CSL Behring's cell and gene therapy scientific platform and its haematology product portfolio, which include other treatments for

haemophilia B as well as therapies for treating haemophilia A, von Willebrand disease, thrombosis, and other life-threatening conditions.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec (also known as AMT-061) uses a specific type of AAV, called AAV5, as its delivery vehicle. The AAV5 vector carries the patent-protected Padua gene variant of Factor IX (FIX-Padua), which generates FIX proteins that work 8x harder than normal. Preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in the 95 percent of haemophilia B patients with pre-existing antibodies to AAV vectors, thereby potentially increasing patient eligibility for treatment compared to other AAV gene therapy product candidates.

Authorised by

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FURTHER INFORMATION

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