



# ASX Announcement

For immediate release

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## CSL Behring Acquires Biotech Company Calimmune and its Proprietary Stem Cell Gene Therapy Platform

- *Builds on CSL Behring's promise to patients with rare and serious diseases*
- *Gene and stem cell-based therapies complements the Company's extensive expertise in protein-based therapies*
- *Sickle cell product candidate (CAL-H) - a strategic fit for CSL Behring's current hematology product line*
- *Novel platform technologies aim to address major commercialization challenges faced by gene therapies to date*

**CSL Limited (ASX:CSL; USOTC:CSLLY)** today announced that CSL Behring has agreed to acquire Calimmune, Inc., a US biotechnology company focused on the development of ex vivo hematopoietic stem cell (**HSC**) gene therapy with R&D facilities in Pasadena, California, USA and Sydney, New South Wales, Australia for an upfront payment of \$91 million.

The acquisition will provide CSL Behring with Calimmune's pre-clinical asset, CAL-H, an HSC gene therapy for the treatment of sickle cell disease and  $\beta$ -thalassemia, which complements CSL Behring's current product portfolio and deep expertise in hematology.

Additionally, CSL Behring will acquire two unique proprietary platform technologies, Select+™ and Cytegrity™ (see below for further details). These technologies are designed to address some of the major challenges currently associated with the commercialization of stem cell therapy, including the ability to manufacture consistent, high-quality products, and to improve engraftment, efficacy and tolerability. Both technologies have broad applications in ex vivo stem cell gene therapy.

"Calimmune shares in our promise and focus to improve the lives of patients with rare and serious medical conditions," said CSL Limited Chief Executive Officer and Managing Director, Paul Perreault. "The acquisition represents another important step in the execution of our strategy for sustainable growth."

"Calimmune's scientific accomplishments are impressive," Perreault added. "The team has built a robust technology platform, and designed a promising HSC gene therapy candidate - CAL-H, which strongly aligns with our longer-term strategic goals, and complements our core competencies and areas of therapeutic focus. While Calimmune is still in the early stages, we believe that our combined strengths have tremendous



# ASX Announcement

potential to change treatment paradigms, and most importantly, significantly improve the lives of our patients.”

Calimmune Chief Executive Officer Louis Breton said, “We are excited to become part of CSL Behring. They are an established global industry leader in protein-replacement therapies and have a proven track record of driving innovations through the development pipeline and delivering differentiated products to the global marketplace. Together, we are well positioned to take our achievements to the next level.”

CAL-H, Calimmune’s HSC gene therapy for sickle cell and  $\beta$ -thalassemia, employs both the Select+™ system, and the Cytegrity™ virus production platform. CAL-H has yielded early positive preclinical results and demonstrates the potential to offer a significant advantage to patients suffering from these currently incurable genetic diseases.

Both proprietary technologies have the potential to be used in treatments for a wide range of other rare diseases that would complement CSL Behring’s business, including those within the company’s current product portfolio.

## **Transaction & Closing**

Under the terms of the agreement between the parties, CSL Behring will have operational control of Calimmune following closing of the transaction. In addition to the upfront payment, the agreement between the parties includes the potential for performance based milestone payments of up to \$325 million over a period currently anticipated to be around eight years or more following the closing of the transaction. The transaction is expected to close within the next two weeks, subject to the satisfaction of various closing conditions.

The additional research and development expenditure is expected to be contained within CSL’s previously announced guidance that R&D expense will make up 10 to 11% of sales per annum over the next few years.

CSL Group’s profit outlook also remains unchanged from previous guidance and announced at the Company’s FY17 results briefing.

**About Sickle Cell Disease and  $\beta$  thalassemia**

Sickle cell disease and  $\beta$ -thalassemia are inherited disorders that affect hemoglobin, the protein in red blood cells that carries oxygen to different parts of the body. They are chronic diseases that dramatically impair the function of many organs and are associated with substantial morbidity, poor quality of life and a shortened life expectancy. The severe forms of both these diseases remain areas of high unmet need with sickle cell disease affecting approximately 150,000 Americans and Europeans and  $\beta$ -thalassemia approximately 16,000. Although there are effective treatments available to relieve the symptoms of these diseases, there are no disease modifying treatments and in many cases regular blood transfusions are also required.

**About Select+™**

Select+™ is a proprietary technology aimed at driving selection of the genetically modified stem cells once they are given back to patients, to decrease toxicity and improve efficacy.

**About Cytegrity™**

Cytegrity™ is a scalable manufacturing technology for the production of lentiviral vectors, which are used as a delivery mechanism for gene therapy.

**About Calimmune:** Calimmune is a privately owned company committed to accelerating the promise of gene therapy to liberate patients from chronic and currently incurable diseases. To achieve this ambitious goal, Calimmune has built a suite of technologies to advance the delivery, manufacturing, and overall efficiency of these life-changing medicines. Calimmune's lead development programs are novel ex vivo gene therapies for hematologic diseases.

**About CSL:** CSL is a leading global biotechnology company with a dynamic portfolio of life-saving medicines, including those that treat haemophilia and immune deficiencies, as well as vaccines to prevent influenza. Since our start in 1916, we have been driven by our promise to save lives using the latest technologies. Today, CSL - including our businesses, CSL Behring and Seqirus - provides life-saving products to more than 60 countries and employs nearly 20,000 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.



# ASX Announcement

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Page 4

28 August 2017

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