

Pfizer inks deal with Sangmo for Hemophilia A gene therapy

11 May 2017 | News

There are approximately 16,000 patients in the U.S. and more than 150,000 worldwide with Hemophilia A



Singapore: US drug giant Pfizer said that it has entered into a deal with Sangamo Therapeutics for rights to its gene therapy programs to treat the rare blood-clotting disorder hemophilia A. The deal will combine Pfizer's heritage in rare disease, capabilities in gene therapy, and expertise in hemophilia with Sangamo's deep knowledge in genomic therapies.

As per the terms, Sangamo will receive a \$70 million upfront payment from Pfizer. Sangamo will be responsible for conducting the SB-525 Phase 1/2 clinical study and certain manufacturing activities. Pfizer will be operationally and financially responsible for subsequent research, development, manufacturing and commercialization activities for SB-525 and additional products, if any.

Sangamo is eligible to receive potential milestone payments of up to \$475 million, including up to \$300 million for the development and commercialization of SB-525 and up to \$175 million for additional Hemophilia A gene therapy product candidates that may be developed under the collaboration. Sangamo will also receive tiered double-digit royalties on net sales. Additionally, Sangamo will be collaborating with Pfizer on manufacturing and technical operations utilizing viral delivery vectors.

Mr Mikael Dolsten, MD, PhD, President of Worldwide Research and Development at Pfizer, said, "Sangamo brings deep scientific and technical expertise across multiple genomic platforms, and we look forward to working together to advance this potentially transformative treatment for patients living with Hemophilia A. Pfizer has made significant investments in gene therapy over the last few years and we are building an industry-leading expertise in recombinant adeno-associated virus (rAAV) vector design and manufacturing. We believe SB-525 has the potential to be a best-in-class therapy that may provide patients with stable and durable levels of Factor VIII protein with a single administration treatment."

"With a long-standing heritage in rare disease, including hemophilia, Pfizer is an ideal partner for our Hemophilia A program," said Dr. Sandy Macrae, Sangamo's Chief Executive Officer. "We believe Pfizer's end-to-end gene therapy capabilities will enable comprehensive development and commercialization of SB-525, which could potentially benefit Hemophilia A patients around the world. This collaboration also marks an important milestone for Sangamo as we continue to make progress in the translation of our ground-breaking research into new genomic therapies to treat serious, genetically tractable diseases."

Hemophilia A is a rare blood disorder caused by a genetic mutation resulting in insufficient activity of Factor VIII, a blood clotting protein the body uses to stop bleeding. There are approximately 16,000 patients in the U.S. and more than 150,000 worldwide with Hemophilia A.

Michael Goettler, global president of Pfizer Rare Disease, said the deal bolsters Pfizer's pipeline in both gene therapy and hemophilia, noting that the company already has a collaboration for a hemophilia B treatment. "We have an intention to become a leader in gene therapy and this is another step," he said in an interview.