Sangamo Therapeutics Logo

Sangamo Therapeutics And Pfizer Announce Collaboration For Hemophilia A Gene Therapy

May 10, 2017

Collaboration combines Pfizer's heritage in Rare Disease, capabilities in gene therapy, and expertise in hemophilia with Sangamo's deep knowledge in genomic therapies

RICHMOND, Calif., May 10, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO) and Pfizer Inc. (NYSE: PFE) today announced an exclusive, global collaboration and license agreement for the development and commercialization of gene therapy programs for Hemophilia A, including SB-525, one of Sangamo's four lead product candidates, which Sangamo expects will enter the clinic this quarter.

"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," said Mikael Dolsten, MD, PhD, President of Worldwide Research and Development at Pfizer. "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."

Under the terms of the collaboration agreement, 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.

Gene therapy is a potentially transformational technology for patients, focused on highly specialized, one-time, treatments that address the root cause of diseases caused by genetic mutation. The technology involves introducing genetic material into the body to deliver a correct copy of a gene to a patient's cells to compensate for a defective one. The genetic material can be delivered to the cells by a variety of means, most frequently using a viral vector such as rAAV. There have been no gene therapy products approved in the U.S. to date.

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. SB-525 is comprised of a rAAV vector carrying a Factor VIII gene construct driven by a proprietary, synthetic, liver-specific promoter. The U.S. Food and Drug Administration has cleared initiation of human clinical trials for SB-525, which also has been granted orphan drug designation. Sangamo is on track this quarter to start a Phase 1/2 clinical trial to evaluate safety and to measure blood levels of Factor VIII protein and other efficacy endpoints.

Conference Call
Sangamo will host a conference call today, May 10, 2017 at 5:00 p.m. ET, which will be open to the public, to discuss the details of the collaboration and the Company's first quarter business and financial results. The call will also be webcast live and can be accessed via a link the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations. A replay of the webcast will also be available for one week after the call.

The conference call dial-in numbers are (877) 377-7553 for domestic callers and (678) 894-3968 for international callers. The conference ID number for the call is 15225000. For those unable to listen in at the designated time, a conference call replay will be available for one week following the conference call, from approximately 8:00 p.m. ET on May 10, 2017 to 11:59 p.m. ET on May 17, 2017. The conference call replay numbers for domestic and international callers are (855) 859-2056 and (404) 537-3406, respectively. The conference ID number for the replay is 15225000.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company is advancing Phase 1/2 clinical programs in Hemophilia A and Hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo has a strategic collaboration with Pfizer for Hemophilia A, with Bioverativ Inc. for hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at www.sangamo.com.

Forward Looking Statements
This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation references relating to the collaboration agreement with Pfizer, potential milestone payments and royalties under the collaboration agreement, ability of the collaboration to advance and commercialize SB-525 as a treatment for Hemophilia A, research and development of therapeutic applications of Sangamo's genomic therapy platforms, the expected timing of clinical trials of lead programs, including SB-525 and the release of data from these trials, the impact of Sangamo's clinical trials on the field of genetic medicine and the benefit of orphan drug status. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to substantial dependence on the clinical success of lead therapeutic programs, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs, technological challenges, Sangamo's ability to develop commercially viable products and technological developments by our competitors. For a more detailed discussion of these and other risks, please see Sangamo's SEC filings, including the risk factors described in its Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo Therapeutics, Inc. assumes no obligation to update the forward-looking information contained in this press release.

Pfizer and Rare Disease
Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,[i] representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including hematology, neuroscience, and inherited metabolic disorders.[ii]

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness and meet the needs of patient families.

Pfizer Inc: Working together for a healthier world®
At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at www.pfizer.com. In addition, to learn more, follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

Pfizer Disclosure Notice:
The information contained in this release is as of May 10, 2017. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about an investigational Hemophilia A agent, SB-525, including its potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical study commencement and completion dates as well as the possibility of unfavorable study results, including unfavorable new clinical data and additional analyses of existing clinical data; risks associated with initial data, including the risk that the final results of the Phase I/2 study for SB-525 and/or additional clinical trials may be different from (including less favorable than) the initial data results and may not support further clinical development; whether and when any applications may be filed with regulatory authorities for SB-525; whether and when regulatory authorities may approve any such applications, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of SB-525; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2016 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission

and available at www.sec.gov and www.pfizer.com.

[i] Rare Disease: Facts and Statistics. http://globalgenes.org/rare-diseases-facts-statistics. Accessed September 7, 2016.
[ii] Pfizer Inc. Rare Disease. http://www.pfizer.com/health-and-wellness/health-topics/rare-diseases/areas-of-focus. Accessed December 20, 2016.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/sangamo-therapeutics-and-pfizer-announce-collaboration-for-hemophilia-a-gene-therapy-300455555.html

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