Shire and Sangamo Collaborate to Develop Therapeutics for the Treatment of Hemophilia and Other Monogenic Diseases

Partnership in DNA-binding technology has potential to provide both companies growth through innovation of new therapies for people with serious diseases

DUBLIN and RICHMOND, Calif., Feb. 1, 2012 /PRNewswire/ -- Shire plc (LSE: SHP, NASDAQ: SHPGY), the global specialty biopharmaceutical company, and Sangamo BioSciences, Inc. (NASDAQ: SGMO), a leader in genome-editing technology, announced today that they have entered into a collaboration and license agreement to develop therapeutics for hemophilia and other monogenic diseases based on Sangamo's zinc finger DNA-binding protein (ZFP) technology.

Shire will receive exclusive world-wide rights to ZFP Therapeutics® designed to target four genes (for blood clotting Factors VII, VIII, IX and X) which will be used to investigate curative therapies for hemophilia A and B. Shire also receives the right to designate three additional gene targets. Sangamo is responsible for all activities through submission of Investigational New Drug (IND) Applications and European Clinical Trial Applications (CTA) for each product and Shire will reimburse Sangamo for its internal and external research program-related costs. Shire is responsible for clinical development and commercialization of products arising from the alliance. Shire will pay Sangamo $13 million upfront followed by research, regulatory, development and commercial milestone payments, and royalties on product sales.

"Sangamo's ground-breaking ZFP gene-editing technology will enable us to expand our therapeutic pipeline into therapies for other genetic disorders such as hemophilia," said Sylvie Gregoire, president of Shire's Human Genetic Therapies business. "While still early in the clinical development process, this DNA-binding protein technology is aligned with our focus of developing new treatments that can add value for physicians, patients and their families, and the healthcare community overall."

"We are delighted to be partnering the first of our monogenic disease programs with Shire, a company known for its development of innovative medicines for genetic diseases," said Edward Lanphier, Sangamo's president and chief executive officer. "This alliance is further validation of our ZFP platform as a transformative technology for the development of novel therapeutics, which have the potential to revolutionize the treatment of a wide range of genetic diseases."
Sangamo's ZFP Therapeutic approach utilizes its proprietary ZFP nuclease (ZFN) and ZFP transcription factor (ZFP TF) technology. ZFPs can be engineered to recognize any specific DNA sequence within a gene, and may be applicable to certain Shire therapeutic areas, including hematology and lysosomal storage disorders.

About Hemophilia

Hemophilia, a rare bleeding disorder, is an example of a monogenic disease. There are several types of hemophilia caused by mutations in genes that encode factors which help the blood clot and stop bleeding when blood vessels are injured. The most prevalent form of the disease, hemophilia A, is caused by a defect in clotting Factor VIII while defects in clotting Factor IX lead to hemophilia B. The most severe forms of hemophilia affect males. According to the National Hemophilia Foundation, hemophilia A occurs in about one in every 5,000 male births in the US, and hemophilia B in about 1 in every 25,000. The standard treatment for individuals with hemophilia is replacement of the defective clotting factor with regular infusion of concentrates or recombinant factors, which are expensive, carry the risk of transmission of blood-borne diseases and sometimes stimulate the body to produce antibodies against the factors that inhibit the benefits of treatment. In these situations, other clotting factors such as Factor VII and X may be used to treat patients.

Using a mouse model of hemophilia B, Sangamo scientists and its collaborators have already established proof of concept that ZFN-mediated genome editing can be accomplished in vivo and is curative in the animal. They have demonstrated the production of stable levels of corrected human clotting Factor IX that are clinically meaningful, restoring clotting times to normal, after a single, systemic administration of ZFNs specific for the Factor IX gene. The data were published in the scientific journal Nature in June 2011 (Nature. 2011 Jun 26; 475(7355):217-21. doi: 10.1038/nature10177).

SHIRE PLC

Shire's strategic goal is to become the leading specialty biopharmaceutical company that focuses on meeting the needs of the specialist physician. Shire focuses its business on attention deficit hyperactivity disorder, human genetic therapies, gastrointestinal diseases and regenerative medicine as well as opportunities in other therapeutic areas to the extent they arise through acquisitions. Shire's in-licensing, merger and acquisition efforts are focused on products in specialist markets with strong intellectual property protection and global rights. Shire believes that a carefully selected and balanced portfolio of products with strategically aligned and relatively small-scale sales forces will deliver strong results.

For further information on Shire, please visit the Company's website: www.shire.com.

SHIRE "SAFE HARBOR" STATEMENT UNDER THE PRIVATE SECURITIES LITIGATION REFORM ACT OF 1995

Statements included herein that are not historical facts are forward-looking statements. Such forward-looking statements involve a number of risks and uncertainties and are subject to change at any time. In the event such risks or uncertainties materialize, the Company's results could be materially adversely affected. The risks and uncertainties include, but are not limited to, risks associated with: the inherent uncertainty of research,
development, approval, reimbursement, manufacturing and commercialization of the Company's Specialty Pharmaceuticals, Human Genetic Therapies and Regenerative Medicine products, as well as the ability to secure new products for commercialization and/or development; government regulation of the Company's products; the Company's ability to manufacture its products in sufficient quantities to meet demand; the impact of competitive therapies on the Company's products; the Company's ability to register, maintain and enforce patents and other intellectual property rights relating to its products; the Company's ability to obtain and maintain government and other third-party reimbursement for its products; and other risks and uncertainties detailed from time to time in the Company's filings with the Securities and Exchange Commission.

Sangamo

Sangamo BioSciences, Inc. is focused on research and development of novel DNA-binding proteins for therapeutic gene regulation and genome editing. Sangamo has a Phase 2 clinical trial and two Phase 1/2 clinical trials to evaluate the safety and efficacy of a novel ZFP Therapeutic® for the treatment of HIV/AIDS. Other therapeutic programs are focused on monogenic diseases, including hemophilia and hemoglobinopathies, and Parkinson's disease. Sangamo's core competencies enable the engineering of a class of DNA-binding proteins known as zinc finger DNA-binding proteins (ZFPs). By engineering ZFPs that recognize a specific DNA sequence Sangamo has created sequence-specific ZFP Nucleases (ZFNs) for gene modification and ZFP transcription factors (ZFP TFs) that can control gene expression and, consequently, cell function. Sangamo has established strategic partnerships with companies in non-therapeutic applications of its technology including Dow AgroSciences and Sigma-Aldrich Corporation. For more information about Sangamo, visit the company's website at www.sangamo.com.

ZFP Therapeutic® is a registered trademark of Sangamo BioSciences, Inc

This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, references to the research and development of novel ZFNs, potential therapeutic applications of the ZFN technology for the treatment of hemophilias and other monogenic diseases and potential milestone payments. Actual results may differ materially from these forward-looking statements due to a number of factors, including technological challenges, uncertainties and risks relating to clinical trials, compliance with regulatory and other requirements, the ability of Sangamo and Shire to develop commercially viable products and technological developments by our competitors. See the SEC filings, and in particular, the risk factors described in Shire and Sangamo's Annual Reports on Form 10-K and most recent Quarterly Reports on Form 10-Q. Shire and Sangamo do not assume any obligation to update the forward-looking information contained in this press release.

SOURCE Sangamo BioSciences, Inc.