

Regulus Therapeutics and GlaxoSmithKline Name a Third Alliance microRNA Target Triggering a Preclinical Milestone Payment

LA JOLLA, Calif., July 20, 2011 /PRNewswire/ -- [Regulus Therapeutics Inc.](#), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that it has identified a third microRNA target in its alliance with GlaxoSmithKline (GSK), triggering a preclinical milestone payment from GSK. This selection represents another significant milestone for Regulus in its microRNA alliance with GSK, which is focused on the discovery and development of compounds targeting four different microRNAs in inflammation and HCV infection.

"GSK has been a committed and valuable partner, providing Regulus with their vast expertise in preclinical models and an understanding of clinical pathways and commercial opportunities," said Neil W. Gibson, Ph.D., chief scientific officer of Regulus. "The alliance has benefitted from Regulus' proprietary technology platform and leading knowledge base of microRNA biology."

Under the 2008 alliance with GSK, Regulus has the potential to earn more than \$600 million in payments across all programs including license and milestone payments, and tiered royalties up to double digits on worldwide sales of products resulting from the alliance. Regulus is responsible for identifying compounds that target four microRNAs through to completion of proof of concept trials. GSK has an exclusive option to license the worldwide development and commercialization rights under each target microRNA.

About microRNAs

The discovery of [microRNA](#) in humans during the last decade is potentially one of the most exciting scientific breakthroughs in recent history. microRNAs are small RNA molecules, typically 20 to 25 nucleotides in length, that do not encode proteins but instead regulate gene expression. More than 700 microRNAs have been identified in the human genome, and over one-third of all human genes are believed to be regulated by microRNAs. A single microRNA can regulate entire networks of genes. As such, these molecules are considered master regulators of the human genome. microRNAs have been shown to play an integral role in numerous biological processes, including the immune response, cell-cycle control, metabolism, viral replication, stem cell differentiation and human development. Most microRNAs are conserved across multiple species, indicating the evolutionary importance of these molecules as modulators of critical biological pathways. Indeed, microRNA expression, or function, has been shown to be significantly altered in many disease states, including cancer, heart failure and viral infections. Targeting microRNAs with anti-miRs, antisense oligonucleotide inhibitors of microRNAs, or miR-mimics, double-stranded oligonucleotides to replace microRNA function opens potential for a novel class of therapeutics and offers a unique approach to treating disease by modulating entire biological pathways. To learn more about microRNAs, please visit <http://www.regulusrx.com/microna/microna-explained.php>.

About Regulus Therapeutics Inc.

Regulus Therapeutics is a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus is using a mature therapeutic platform based on technology that has been developed over 20 years and tested in more than 5,000 humans. In addition, Regulus works with a broad network of academic collaborators and leverages the oligonucleotide drug discovery and development expertise of its founding companies, Alnylam Pharmaceuticals (*NASDAQ:ALNY*) and Isis Pharmaceuticals (*NASDAQ:ISIS*). Regulus is advancing microRNA therapeutics towards the clinic in several key areas including fibrosis, HCV, immuno-inflammatory diseases, metabolic diseases, and oncology. Regulus' intellectual property estate contains both the fundamental and core patents in the field and includes over 600 patents and more than 300 pending patent applications pertaining primarily to chemical modifications of oligonucleotides targeting microRNAs for therapeutic applications. In April 2008, Regulus formed a major alliance with GlaxoSmithKline to discover and develop microRNA therapeutics for immuno-inflammatory diseases. In February 2010, Regulus and GlaxoSmithKline entered into a new collaboration to develop and commercialize microRNA therapeutics targeting microRNA-122 for the treatment of hepatitis C infection. In June 2010, Regulus and sanofi-aventis entered into the largest-to-date strategic alliance for the development of microRNA therapeutics. This alliance is focused initially on fibrosis. For more information, please visit <http://www.regulusrx.com>.

Forward-Looking Statements

This press release includes forward-looking statements regarding the future therapeutic and commercial potential of Regulus' business plans, technologies and intellectual property related to microRNA therapeutics

being discovered and developed by Regulus. Any statement describing Regulus' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such products. Such forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause the results to differ materially from those expressed or implied by such forward-looking statements. Although these forward-looking statements reflect the good faith judgment of Regulus' management, these statements are based only on facts and factors currently known by Regulus. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Regulus', Alnylam's, and Isis' programs are described in additional detail in Alnylam's and Isis' annual reports on Form 10-K for the year ended December 31, 2010, and its most recent quarterly report on Form 10-Q. Copies of these and other documents are available from Alnylam or Isis.

SOURCE Regulus Therapeutics Inc.

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