Regulus Therapeutics to Present microRNA Platform and Pipeline Updates at Two Leading Scientific Conferences

-New Pre-clinical Data to be Presented on Proprietary Therapeutic Programs in Metabolic Disease and Cancer at Keystone Symposium and AACR Special Conference

LA JOLLA, Calif., Jan. 5, 2012 /PRNewswire/ -- Regulus Therapeutics Inc., a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that data from its proprietary lead program targeting microRNA-33a/b (miR-33a/b), currently in preclinical development for the treatment of atherosclerosis, will be presented at the Keystone Symposium "Nucleic Acid Therapeutics: From Base Pairs to Bedsides" held Jan. 10–15, 2012, in Santa Fe, New Mexico. In addition, information from its preclinical oncology programs will be presented at the American Association for Cancer Research's (AACR) Noncoding RNAs and Cancer workshop held Jan. 8-11, 2012, in Miami, Florida.

"We are pleased to present data about our proprietary microRNA programs in metabolic disease and oncology at the Keystone Symposium and AACR Special Conference respectively," said Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus Therapeutics. "Our scientists have made significant progress discovering potent and safe therapeutic oligonucleotide anti-miRs. We look forward to selecting clinical candidates this year for the advancement of multiple microRNA therapeutic programs into the clinic."

- Keystone Symposium: A Regulus scientist will present that targeting miR-33a/b is a promising therapeutic strategy for atherosclerosis and other aspects of the metabolic syndrome in an oral presentation titled "*Targeting miR-33 for Atherosclerosis*" at 8 a.m. MST on January 14. miR-33a and miR-33b are found in the introns of SREBF2 and SREBF1 transcription factors, respectively, and work in concert with their host genes to regulate cholesterol and fatty acid synthesis. miR-33a/b target the cellular cholesterol efflux transporter ABCA1 as well as key regulators of fatty acid oxidation and insulin signaling, suggesting that inhibition of miR-33a/b could have therapeutic benefit in atherosclerosis and other aspects of the metabolic syndrome, including hepatosteatosis and insulin resistance. Studies in mice showed that antagonizing miR-33a enhanced reverse cholesterol transport, promoted regression of atherosclerosis by increasing ABCA1 in the liver and peripheral macrophages, and raised plasma HDL cholesterol. Importantly, these findings have now been translated to non-human primates, demonstrating that systemic delivery of an anti-miR-33a/b oligonucleotide increased hepatic expression of ABCA1, induced a sustained increase in circulating HDL cholesterol and decreased plasma triglycerides.
- AACR Special Conference: A Regulus scientist will present data demonstrating that microRNAs are dysregulated in cancer and are drivers of disease pathogenesis in an oral presentation titled "Anti-miR Therapeutics for Cancer" at 10:15 a.m. EST on January 11. Regulus' oncology programs include targeting microRNA-21 (miR-21), which has been shown to be overexpressed in many cancer types promoting tumor progression and metastasis. Regulus data has demonstrated that miR-21 is up-regulated in patients with hepatocellular carcinoma (HCC) and, in preclinical models of HCC, treatment with anti-miR-21 reduces tumor formation resulting in significant survival benefit. Other efforts in oncology include the discovery of microRNA therapeutics for the treatment of glioblastoma multiforme (GBM), an orphan and rare disease with limited treatment options, performed in collaboration with Samsung Medical Center and ABC^2, a non-profit organization dedicated to accelerating therapies for brain cancer patients.

Regulus is advancing multiple microRNA therapeutic programs to the clinic in the areas of fibrosis, HCV infection, immuno-inflammatory disease, metabolic disease, and oncology.

About microRNAs
The discovery of microRNA in humans during the last decade is 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.

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. The company 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 toward clinical development in several areas, including fibrosis, hepatitis C, 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 with an initial focus on fibrosis.

For more information, please visit http://www.regulusrx.com. Regulus is also on YouTube at http://www.youtube.com/user/RegulusRx#p/f and on Twitter at www.twitter.com/regulusrx .

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, including statements regarding the therapeutic potential of targeting microRNAs for metabolic disease and oncology. 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.

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