Regulus Therapeutics Initiates an Orphan and Rare Disease microRNA Therapeutic Effort in Brain Cancer

-Regulus, Accelerate Brain Cancer Cure (ABC2) and Samsung Medical Center to Advance microRNA Therapeutics for Glioblastoma -

LA JOLLA, Calif., Dec. 12, 2011 /PRNewswire/ -- Regulus Therapeutics Inc., a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced the initiation of a new discovery effort in microRNA therapeutics for the treatment of glioblastoma multiforme (GBM), the most common and aggressive brain tumor in humans. Regulus will apply its expertise in microRNA therapeutics to discover chemically modified oligonucleotide anti-miRs for testing at the Samsung Medical Center in preclinical models that mimic human brain cancer. Accelerate Brain Cancer Cure (ABC2), a non-profit organization dedicated to accelerating therapies for brain cancer patients, has awarded Regulus a grant to support the research.

"GBM is a devastating disease with limited treatment options," said Neil W. Gibson, Ph.D. Chief Scientific Officer of Regulus Therapeutics. "At Regulus, we have successfully targeted microRNAs in multiple disease settings and believe that targeting dysregulated microRNAs using chemically modified oligonucleotide anti-miRs provides a potential therapeutic approach for GBM."

"A major hurdle in the discovery and development of novel therapeutics for GBM has been the lack of relevant animal models," said Do Hyun Nam, Ph.D. Professor and Director of Institute for Refractory Cancer Research, Samsung Medical Center. "In our collaboration with Regulus, we will utilize a recently developed model in which tumors derived from human patients are transplanted into mice resulting in a model directly relevant to human disease."

"Regulus has an impressive track record of executing on innovation in the field of microRNA therapeutics," said Max Wallace, Chief Executive Officer, ABC2. "The combination of Regulus' expertise in microRNA therapeutics with Samsung's preclinical GBM models compelled us to support this promising collaborative approach."

Glioblastoma multiforme, also known as glioblastoma or grade IV astrocytoma, is a fast growing brain tumor that forms from glial (supportive) tissue of the brain. GBM is the most prevalent form of primary brain tumor and peak incidence is in adults between 45 and 70 years. Treatment options are limited and survival is little over one year. GBM is considered a rare, or orphan, disease with an incidence in the U.S. of approximately 12,000 per year.

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 GBM. 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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