Regulus Receives Orphan Drug Designation for RG-012, a microRNA Therapeutic for the Treatment of Alport Syndrome

LA JOLLA, Calif., July 21, 2014 /PRNewswire/ -- Regulus Therapeutics Inc. (*NASDAQ:RGLS*), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today that the U.S. Food & Drug Administration (FDA) has granted orphan drug designation to RG-012, a single stranded, chemically modified oligonucleotide that binds to and inhibits the function of microRNA-21 ("miR-21"), as a therapeutic for the treatment of Alport syndrome, a life-threatening genetic kidney disease with no approved therapy.

"We are pleased to have received orphan drug designation for RG-012 and are encouraged by the FDA's recognition for the need of innovative new treatments like microRNA therapeutics for rare and orphan diseases such as Alport syndrome," said Paul Grint, M.D., Chief Medical Officer of Regulus. "Alport syndrome is a life threatening disease and patients have very limited treatment options because there is currently no approved therapy. We believe that RG-012 represents an opportunity to make a significant impact in the lives of patients with Alport syndrome and we look forward to advancing this program into the clinic."

In the near term, Regulus expects to initiate a natural history of disease study to gather further information about the progression of Alport syndrome and to inform future clinical development plans of RG-012, a key program under Regulus' 'Clinical Map Initiative'.

About Orphan Drug Designation

According to the FDA, the Orphan Drug Designation program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected the costs of developing and marketing a treatment drug. The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies.

About Alport Syndrome

Alport syndrome is a life-threatening genetic kidney disease that impacts the body's ability to create a specific type of collagen highly expressed in the kidney and essential to normal kidney structure. In the absence of this type of collagen, the kidneys are unable to effectively filter toxins and waste products, resulting in end-stage renal disease and also hearing loss or effects on vision. Currently, there is no approved therapy for Alport syndrome.

About RG-012 for the Treatment of Alport Syndrome

Regulus has discovered that miR-21 is highly overexpressed in mouse models of Alport syndrome. Regulus is developing RG-012, a single stranded, chemically modified oligonucleotide that binds to and inhibits the function of miR-21, which has demonstrated potent inhibition of miR-21 *in vitro* and *in vivo,* a decrease in the rate of progression of renal fibrosis, an increase in the lifespan of mice by up to fifty percent, and a favorable pharmacokinetic profile that supports the potential for a once per week dosing regimen. In the near term, Regulus expects to initiate a natural history of disease study to gather greater information about the progression of Alport syndrome and expects to initiate a Phase I clinical study of RG-012 for the treatment of Alport syndrome in the first half of 2015.

About microRNAs

The discovery of microRNAs 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 800 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. microRNA expression, or function, has been shown to be significantly altered or dysregulated in many disease states, including oncology, fibrosis, metabolic diseases, immune-inflammatory diseases and HCV. Targeting microRNAs with anti-miRs, chemically modified, single-stranded oligonucleotides, offers a unique approach to treating disease by modulating entire biological pathways and may become a new and major class of drugs with broad therapeutic application.

About the 'Clinical Map Initiative'

Launched in February 2014, Regulus' 'Clinical Map Initiative' outlines certain corporate goals to advance its microRNA therapeutics pipeline over the next several years. Regulus expects to demonstrate human proof-of-concept results in the Phase I clinical study of RG-101 for the treatment of HCV by the end of 2014, initiate a Phase I clinical study of RG-012 for the treatment of Alport syndrome in the first half of 2015, nominate a third microRNA candidate for clinical development by the end of 2014, and maintain a strong financial position and end 2014 with at least $75.0 million in cash, cash equivalents and short-term investments.

About Regulus

Regulus Therapeutics Inc. (*NASDAQ:RGLS*) is a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus is uniquely positioned to leverage a mature therapeutic platform that harnesses the oligonucleotide drug discovery and development expertise of Alnylam Pharmaceuticals, Inc. and Isis Pharmaceuticals, Inc., which founded the company. Regulus has a well-balanced microRNA therapeutics pipeline entering clinical development, an emerging microRNA biomarkers platform to support its therapeutic programs, and a rich intellectual property estate to retain its leadership in the microRNA field. Regulus intends to focus its proprietary efforts on developing microRNA therapeutics for oncology indications and orphan diseases and is currently advancing several programs toward clinical development in oncology, fibrosis and metabolic diseases. Specifically, Regulus is developing RG-012, an anti-miR targeting microRNA-21 for the treatment of Alport syndrome, a life-threatening kidney disease driven by genetic mutations with no approved therapy, and RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection. Regulus' commitment to innovation and its leadership in the microRNA field have enabled the formation of strategic alliances with AstraZeneca, GlaxoSmithKline and Sanofi and a research collaboration with Biogen Idec focused on microRNA biomarkers. In addition, the Company has established Regulus microMarkers™, a research and development division focused on identifying microRNAs as biomarkers of human disease, which is designed to support its therapeutic pipeline, collaborators and strategic partners.

For more information, please visit http://www.regulusrx.com.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements associated with financial estimates (including Regulus' projected cash at the end of 2014), the projected sufficiency of Regulus' capital position for future periods, the expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-012 and RG-101 and with respect to the nomination of a third microRNA candidate for clinical development), the projected timeline of clinical development activities, and expectations regarding future therapeutic and commercial potential of Regulus' business plans, technologies and intellectual property related to microRNA therapeutics and biomarkers being discovered and developed by Regulus. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "intends," "will," "goal," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Regulus' current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks associated with 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 drugs. These and other risks concerning Regulus' financial position and programs are described in additional detail in Regulus filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made. Regulus undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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