Regulus Receives Orphan Medicinal Product Designation from the European Commission for RG-012, a microRNA Therapeutic in Development for the Treatment of Alport Syndrome

LA JOLLA, Calif., March 25, 2015 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ:RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today that the European Commission has granted orphan medicinal product designation for RG-012, a single stranded, chemically modified oligonucleotide that binds to and inhibits the function of microRNA-21 ("miR-21") for the treatment of Alport syndrome, a life-threatening genetic kidney disease with no approved therapy. In July 2014, the U.S. Food & Drug Administration granted orphan drug designation to RG-012 for the treatment of Alport syndrome.

"We are pleased to have received orphan medicinal product designation in the European Union for RG-012, a key microRNA therapeutic program under our 'Clinical Map Initiative'," 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."

Regulus is currently enrolling patients in a natural history of disease study called ATHENA to gather information about the changes in renal function over time in patients with Alport syndrome. Data from the ATHENA study will provide the clinical basis for the design of a Phase II study to monitor the therapeutic effect of RG-012 on the decline in renal function and time to end-stage renal disease in Alport syndrome patients. Under Regulus' 'Clinical Map Initiative', the company expects to initiate a Phase I study evaluating RG-012 in healthy volunteers in the first half of 2015 and a Phase II proof-of-concept study thereafter.

Orphan drug designation by the European Commission provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and where no satisfactory treatment is available. In addition to a 10-year period of marketing exclusivity in the EU after product approval, orphan drug designation provides incentives for companies seeking protocol assistance from the European Medicines Agency during the product development phase, and direct access to the centralized authorization procedure.

About Alport Syndrome

Alport syndrome is an inherited form of kidney disease caused by mutations in the type IV collagen genes (Col4A3, Col4A4 and Col4A5). Type IV collagen is important for maintaining the integrity of the glomerular basement membrane (GBM), a vital component in the kidney structure and filtration process. The genetic mutation in the collagen gene results in thickening in the GBM and impairment of glomerulus filtration. Alport syndrome patients experience a progressive loss of kidney function, which ultimately leads to end stage renal disease requiring dialysis or kidney transplantation, or may even lead to death. Alport syndrome can also cause hearing loss and eye abnormalities during late childhood or early adolescence. ACE (angiotensin-converting enzyme) inhibitors are emerging as standard of care in patients with Alport syndrome used to treat proteinuria, or abnormal amounts of protein in the urine, an indicator of chronic kidney disease. Alport syndrome represents a high unmet medical need with no approved therapy.

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 Regulus

Regulus Therapeutics Inc. (*NASDAQ:RGLS*) is a biopharmaceutical company leading the discovery and

development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a well-balanced microRNA therapeutics pipeline complemented by a maturing microMarkers^{SM} biomarkers platform and a rich intellectual property estate to retain its domain dominant leadership in the microRNA field. Under its 'Clinical Map Initiative', Regulus is developing RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection, and 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. Regulus is also advancing several programs toward clinical development in orphan disease indications, oncology and fibrosis. Regulus' commitment to innovation has resulted in multiple peer-reviewed publications in notable scientific journals and has resulted in the formation of strategic alliances with AstraZeneca and Sanofi and a research collaboration with Biogen Idec focused on microRNA biomarkers. Regulus maintains its corporate headquarters in La Jolla, CA. 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 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), 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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