Regulus to Present New RG-012 Data at The 53rd European Renal Association-European Dialysis and Transplant Association (ERA-EDTA) Congress

LA JOLLA, Calif., May 20, 2016 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that new data on RG-012, the company's anti-miR targeting microRNA-21 for the treatment of Alport syndrome, a life-threatening kidney disease driven by genetic mutations with no approved therapy, is being presented in three posters during the 53rd European Renal Association-European Dialysis and Transplant Association (ERA-EDTA) Congress May 21-24 in Vienna, Austria.

Poster Presentations: May 22, 2016, 09:30-10:45AM

- **Abstract SP120**: **Change in Glomerular Filtration Rate and Renal Biomarkers in Patients with Chronic Kidney Disease Due to Alport Syndrome: Interim Results from the ATHENA Study, a Prospectively Designed Natural History Study** (M. Rheault, United States).
- **Abstract SP283**: **Renal Impairment Effects on Plasma and Tissue Exposure of Unconjugated and Galnac-conjugated Anti-miRs in a Chronic Kidney Disease (CKD) Mouse Model** (J. Grundy, United States).
- **Abstract SP257**: **Treatment with the MicroRNA-21 Inhibitor RG-012 Given with and without Ramipril Delays Renal Impairment Progression and Prolongs Survival when Initiated up to Chronic Kidney Disease (CKD) Stage 3 in a Mouse Model of Alport Syndrome** (J. Grundy, United States).

The abstracts can be accessed through the ERA-EDTA website at www.era-edta2016.org.

About RG-012 for 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 glomerular 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 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.

Currently, there is little known information on exactly how Alport syndrome progresses, although miR-21 is believed to play a role in the disease progression. miR-21 is up-regulated in Col4A3 deficient mouse models of Alport syndrome, other renal fibrosis models and human CKD patients. The role of miR-21 has been validated through genetic knock-out models and anti-miRs targeting miR-21 have reduced the severity of fibrosis in two distinct preclinical rodent models. Regulus is developing RG-012, a single stranded, chemically modified oligonucleotide that binds to and inhibits the function of miR-21 for the treatment of Alport syndrome. In preclinical studies, RG-012 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 the Col4A3 deficient mice by up to fifty percent, and an additive benefit in combination with an emerging standard of care therapy. Further, RG-012 has received orphan drug status from the U.S. Food and Drug Administration and European Commission as a therapeutic in development for the treatment of Alport syndrome.

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 two-thirds 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 leadership in the microRNA field. 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. In addition, RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of NASH in patients with type 2 diabetes/pre-diabetes, has entered Phase I clinical development through its strategic alliance with AstraZeneca. Regulus is also advancing several programs toward clinical development in renal, hepatic and central nervous systems diseases, both independently and with our strategic alliance partners, Sanofi and AstraZeneca. 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. 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-101), 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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