Regulus Announces First-in-Human Dosing for Phase I Study of RGLS4326 for the Treatment of Autosomal Dominant Polycystic Kidney Disease

LA JOLLA, Calif., Dec. 19, 2017 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that it has initiated the first-in-human Phase I study of RGLS4326 and completed dosing of the first cohort of healthy volunteers. RGLS4326 is in development for the treatment of autosomal dominant polycystic kidney disease, or ADPKD.

"The initiation of this first-in-human study for RGLS4326 marks an important clinical milestone for Regulus," said Dr. Timothy Wright, Regulus' Chief R&D Officer. "Based on the strong preclinical evidence for the role of miR-17 in animal models of PKD and in vitro testing of human ADPKD cyst cell cultures, we believe that RGLS4326 has great potential as a disease modifying therapy in ADPKD. This program highlights the speed with which our scientists can progress from oligonucleotide library synthesis to human testing, which in the case of RGLS4326, was less than 24 months."

RGLS4326 is being studied in a Phase I randomized, double-blind, placebo-controlled, single ascending dose study designed to investigate the safety, tolerability, pharmacokinetics and pharmacodynamics of RGLS4326 administered subcutaneously in healthy volunteers.

About RGLS4326
RGLS4326 is a novel oligonucleotide designed to inhibit miR-17 using a unique chemistry design to preferentially target the kidney. Preclinical studies with RGLS4326 have demonstrated a reduction in kidney cyst formation, improved kidney weight/body weight ratio, decreased cyst cell proliferation, and preserved kidney function in mouse models of ADPKD.

About Autosomal Dominant Polycystic Kidney Disease (ADPKD)
ADPKD, caused by mutations in the PKD1 or PKD2 genes, is among the most common human monogenetic disorders and a leading genetic cause of end-stage renal disease. The clinical hallmark of this disease is the development of multiple fluid filled cysts primarily in the kidneys and to a lesser extent in the liver and other organs. Excessive kidney tubule derived cyst cell proliferation, a central pathological feature, drives the expansion of cysts, ultimately causing end-stage renal disease in approximately 50% of ADPKD patients by age 60. It is estimated that approximately 1 in 1,000 people possess a mutation in the PKD1 or PKD2 genes worldwide.

About Regulus
Regulus Therapeutics Inc. (Nasdaq: RGLS) is a clinical stage biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus is leveraging its oligonucleotide drug discovery and development expertise to develop a microRNA therapeutics pipeline complemented by a rich intellectual property estate to retain its leadership in the microRNA field. Regulus is advancing several programs in renal, hepatic and central nervous systems diseases. 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 or RGLS4326), 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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