Regulus Initiates Multiple Ascending Dose Study in Healthy Volunteers of RGLS4326 for the Treatment of ADPKD

May 1, 2018

Initial safety and pharmacokinetic results from single ascending dose study support advancement

LA JOLLA, Calif., May 1, 2018 /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 a Phase I multiple ascending dose (MAD) study in healthy volunteers for RGLS4326 for the treatment of autosomal dominant polycystic kidney disease, or ADPKD. RGLS4326 is a novel, first-in-class, oligonucleotide designed to inhibit miR-17 using a unique chemistry that preferentially delivers to the kidney.

Regulus Therapeutics Inc. Logo

"We are pleased to advance the RGLS4326 program with the initiation of the MAD study, which will allow us to further characterize the safety and pharmacokinetic profile of RGLS4326 and establish the dose range that we will study in patients with ADPKD," said Timothy Wright, M.D., Chief R&D Officer of Regulus. "RGLS4326 represents a novel approach to treating ADPKD, a genetic disease leading to progressive loss of kidney function and kidney failure in the majority of patients."

This study is designed to characterize the safety, tolerability, pharmacokinetics and pharmacodynamics of increasing doses of RGLS4326. The MAD study was initiated based on data from the single ascending dose (SAD) Phase I study, which has completed dose escalation and continues in the planned follow-up phase. Preclinical studies with RGLS4326 have demonstrated a reduction in kidney cyst formation, improved kidney weight/body weight ratio, and decreased cyst cell proliferation in mouse models of ADPKD; and decreased cyst formation in cultured human ADPKD cyst cells in vitro.

About Autosomal Dominant Polycystic Kidney Disease (ADPKD)

ADPKD, caused by the 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, fuels the expansion of cysts, ultimately causing end-stage renal disease in approximately 50% of ADPKD patients by age 60. Approximately 1 in 1,000 people bear a mutation in either PKD1 or PKD2 genes worldwide.

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 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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