Regulus Therapeutics Reinitiates Multiple Ascending Dose Study of RGLS4326 for the Treatment of ADPKD

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LA JOLLA, Calif., Feb. 13, 2020 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs, today announced that it initiated dosing of the second cohort of its Phase 1 multiple ascending dose clinical trial (the "MAD study") of RGLS4326 for the treatment of autosomal dominant polycystic kidney disease (ADPKD). The Company expects to complete this study in mid-2020 with topline results available thereafter. The Company is also planning to initiate a Phase 1b short-term dosing study in patients with ADPKD in the second half of 2020 to evaluate RGLS4326 for safety, pharmacokinetics, and biomarkers of pharmacodynamic activity. RGLS4326 is a novel oligonucleotide designed to inhibit miR-17 and to preferentially target the kidney.

"We are pleased to advance the RGLS4326 program with dosing of the second cohort in the MAD study, which will allow us to further characterize the safety and pharmacokinetic profile of RGLS4326 and establish the dose range that we plan to evaluate in the Phase 1b study in ADPKD patients," said Jay Hagan, CEO 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 these patients."

The Company previously announced that it had completed both the single ascending dose study in healthy volunteers up to the planned highest dose, as well as the first dose level of the MAD study in healthy volunteers. Clinical data generated to date showed that RGLS4326 administration was generally well-tolerated with no serious adverse events for all doses tested. Information from the clinical studies together with information from additional nonclinical studies will be used to address the requirements outlined by FDA to support studies of extended duration.

About ADPKD

ADPKD, caused by the mutations in the PKD1 or PKD2 genes, is among the most common human monogenic disorders and a leading cause of end-stage renal disease. The disease is characterized by the development of multiple fluid filled cysts primarily in the kidneys, and to a lesser extent in the liver and other organs. Excessive kidney cyst cell proliferation, a central pathological feature, ultimately leads to end-stage renal disease in approximately 50% of ADPKD patients by age 60.

About RGLS4326

RGLS4326 is a novel oligonucleotide designed to inhibit miR-17 and designed to preferentially target the kidney. Preclinical studies with RGLS4326 have demonstrated direct regulation of PKD1 and PKD2 in human ADPKD cyst cells, 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. The RGLS4326 IND is currently on a Partial Clinical Hold for long-term chronic dosing by the U.S. Food and Drug Administration.

About Regulus

Regulus Therapeutics Inc. (Nasdaq: RGLS) is a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a pipeline complemented by a

rich intellectual property estate in the microRNA field. Regulus maintains its corporate headquarters in La Jolla, CA.

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 completion of preclinical and clinical activities concerning the RGLS4326 program, the sufficiency of the data resulting from the ongoing or planned preclinical studies required to recommence clinical studies for long-term chronic dosing and the timing of preclinical and clinical activities. 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, and feedback from the FDA. These and other risks 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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