Regulus Therapeutics Announces FDA Orphan Drug Designation of RGLS4326 for the Treatment of Autosomal Dominant Polycystic Kidney Disease (ADPKD)

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LA JOLLA, Calif., July 29, 2020 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs (the "Company" or "Regulus"), today announced that the U.S. Food and Drug Administration ("FDA") has granted Orphan Drug Designation to RGLS4326 for the treatment of patients with ADPKD.

"We are pleased RGLS4326 has been granted Orphan Drug Designation by FDA," said Jay Hagan, CEO of Regulus. "This is an important milestone for our APDKD program and our efforts to address the significant unmet medical needs with this disease."

FDA's Office of Orphan Drug Products grants orphan status to drugs intended to treat rare disorders that affect fewer than 200,000 people in the U.S. The designation provides certain potential benefits to the drug developer, including seven years of market exclusivity upon FDA approval, prescription drug user fee waivers and tax credits for qualified clinical trials. For more information about orphan designation, please visit the FDA website at www.fda.gov.

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*, reduction of cyst growth in human in vitro ADPKD models, and attenuation of cyst proliferation and improvement of kidney function in mouse models of ADPKD. The Company recently completed the multiple ascending dose study of RGLS4326 in healthy volunteers. Administration of RGLS4326 was well-tolerated with no serious adverse events reported. The Company is initating a Phase 1b study in patients with ADPKD to evaluate short-term treatment of RGLS4326 for safety, tolerability, pharmacokinetics, and changes in biomarkers of the disease. The RGLS4326 IND is currently on a Partial Clinical Hold for treatment of extended duration beyond the current Phase 1b study until the second set of requirements outlined by FDA have been satisfactorily addressed. Information from the Phase 1 clinical studies, together with information from the recently completed additional nonclinical studies, will be used to address the second set of requirements to support studies of extended duration.

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 extended duration 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. In addition, while Regulus expects the COVID-19 pandemic to adversely affect its business operations and financial results, the extent of the impact on Regulus' ability to achieve its preclinical and clinical development objectives and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. 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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