Regulus Therapeutics Announces Presentation at the First Annual Rare & Genetic Kidney Disease Drug Development Summit

SAN DIEGO, Dec. 1, 2021 /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 a presentation at the first annual Rare & Genetic Kidney Disease Drug Development Summit.

In the presentation, Regulus summarized its ongoing efforts to therapeutically target microRNAs (miRs), small non-coding RNAs that play an important role in the regulation of gene expression and are known to be involved in the pathogenesis of numerous genetic kidney diseases representing significant unmet medical needs. The Company believes that targeting over-expression of miRs with antagonists (anti-miRs) and normalizing the expression of miRs presents a promising therapeutic strategy. Targeting of miR-21 and miR-17 is currently being investigated for the treatment of Alport Syndrome and Autosomal Dominant Polycystic Kidney Disease (ADPKD), respectively.

ADPKD is caused by mutations of either Pkd1 (in 85% of cases) or Pkd2 genes, where reduced function of their encoded proteins polycystin-1 (PC1) or polycystin-2 (PC2) leads to formation and proliferation of multiple fluid-filled renal cysts, and results in loss of kidney function over time. The levels of urinary PC1 and PC2 are reduced in ADPKD patients and inversely correlate with ADPKD disease severity, thereby serving as potential biomarkers for disease severity and progression. PC1 and PC2 levels have been shown to be mediated by direct repression of Pkd1 and Pkd2 genes by miR-17, which is overexpressed in ADPKD. Today's presentation summarized data from the first cohort of ADPKD patients in the Phase 1b clinical trial of RGLS4326, the Company's first-generation selective inhibitor of miR-17 for the treatment of ADPKD. The presentation also summarized some of the evidence for the role of miRs in the pathogenesis of other genetic kidney diseases, including Focal Segmental Glomerulosclerosis (FSGS) and IgA nephropathy (Berger's disease).

In the first cohort, the mean increases in PC1 and PC2 at the end of study treatment compared to baseline levels were 58% (p=.0004) and 38% (p=.026), respectively. These data demonstrate clinical evidence that inhibition of miR-17 by treatment with RGLS4326 increased both PC1 and PC2. Along with other clinical data presented earlier this year, these results provide clinical proof of the mechanism for targeting miR-17 for the treatment of ADPKD. The Company recently announced the strategic prioritization of its second generation miR-17 inhibitor, RGLS8429, which is supported by strong preclinical data and a superior pharmacologic profile to that of RGLS4326. The Company is scheduled to have a pre-IND meeting with the U.S. Food and Drug Administration (FDA) for RGLS8429 in December and is on track for an IND submission and initiation of clinical development in the second quarter of 2022, subject to FDA clearance of the IND.

The details of the presentation are below:

Presentation Title: Exploring the Role of MicroRNA in Genetic Kidney Disease Pathologies

Presenter: Denis Drygin, Ph.D., Chief Scientific Officer, Regulus Therapeutics

Presentation Date and Time: Wednesday, December 1, 2021, 5:30 AM PDT

A copy of the presentation is available at www.regulusrx.com/publications/.

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 RGLS8429

RGLS8429 is a novel, second generation oligonucleotide designed to inhibit miR-17 and to preferentially target the kidney. Administration of RGLS8429 has shown robust data in preclinical models, where clear improvements in kidney function, size, and other measures of disease severity, as well as a superior pharmacologic profile have been demonstrated. Regulus has nominated RGLS8429 as a clinical candidate for the treatment of ADPKD.

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 San Diego, CA.

Forward-Looking Statements

Statements contained in this presentation 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 Company's RGLS8429 program, including the potential sufficiency of the preclinical data required to support clinical studies, the expected timing for submitting an IND and initiating a Phase 1 clinical study, the expected timing for reporting topline data, and the timing and future occurrence of other 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 the risk additional toxicology data may be negative. 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, including under the "Risk Factors" heading of Regulus most recently quarterly report on Form 10-Q. 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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