Regulus Therapeutics Reports First Quarter 2023 Financial Results and Recent Updates

Enrollment completed in first cohort of patients with Autosomal Dominant Polycystic Kidney Disease (ADPKD) in Phase 1b Multiple-Ascending Dose (MAD) study of RGLS8429

Closed $15.0 million private placement of equity; expected to extend cash runway into mid-2024

SAN DIEGO, May 11, 2023 /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 reported financial results for the first quarter ended March 31, 2023 and provided a corporate update.

"Our work in ADPKD continues to progress, with enrollment now complete in cohort 1 of our Phase 1b MAD study of RGLS8429," stated Jay Hagan, CEO of Regulus. "We look forward to sharing topline data around the end of the third quarter of this year. We also appreciate the continued support of our top shareholders who participated in our recently announced private placement of $15 million in April, which we anticipate will extend our cash runway into mid-2024 and through several additional program milestones."

Program Updates

RGLS8429 for ADPKD: In April 2023, the company announced the completion of enrollment for the first cohort of patients in the Phase 1b MAD study of RGSL8429 for the treatment of ADPKD. The Phase 1b MAD study is a double-blind, placebo-controlled trial evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics (PK/PD) of RGLS8429 in adult patients with ADPKD. The study will evaluate the safety and efficacy of RGLS8429 treatment across three different dose levels, including measuring changes in polycystins, height-adjusted total kidney volume (htTKV), cyst architecture, and overall kidney function. The first cohort is being dosed at 1 mg/kg of RGLS8429 or placebo every other week for three months, with top-line data anticipated around the end of the third quarter of 2023. The Company expects to begin dosing the second cohort following a review of all available cohort 1 safety data.

Corporate Highlights

Closed $15.0 Million Private Placement: On April 13, 2023, the Company announced that it entered into a definitive securities purchase agreement in connection with a private placement to certain institutional and other accredited investors. The financing was led by the Federated Hermes Kaufmann Funds and New Enterprise Associates (NEA), with participation from additional existing shareholders.

Financial Results

Cash Position: As of March 31, 2023, Regulus had $30.3 million in cash and cash equivalents. Combined with the $15.0 million private placement in April, the Company expects its cash runway to extend into mid-2024.

Research and Development (R&D) Expenses: Research and development expenses were $4.9 million for the three months ended March 31, 2023, compared to $3.7 million for the same period in 2022. These amounts reflect internal and external costs associated with advancing our clinical and preclinical pipeline.

General and Administrative (G&A) Expenses: General and administrative expenses were $2.4 million for the three months ended March 31, 2023, compared to $2.9 million for the same period in 2022. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was $7.1 million, or $0.42 per share (basic and diluted), for the three months ended March 31, 2023, compared to $6.7 million, or $0.46 per share (basic and diluted), for the same period in 2022.

About ADPKD

Autosomal Dominant Polycystic Kidney Disease (ADPKD), caused by 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. Approximately 160,000 individuals are diagnosed with the disease in the United States alone, with an estimated global prevalence of 4 to 7 million.

About RGLS8429

RGLS8429 is a novel, next generation oligonucleotide for the treatment of ADPKD 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 have been demonstrated along with a superior pharmacologic profile in preclinical studies compared to Regulus' first-generation compound, RGLS4326. Regulus announced completion of the Phase 1 SAD study in September 2022 and completed enrollment of the first cohort of patients in the Phase 1b MAD study in April of this year. The Phase 1 SAD study demonstrated that RGLS8429 has a favorable safety and PK profile. RGLS8429 was well-tolerated with no serious adverse events reported and plasma exposure was approximately linear across the four doses tested and similar to the PK data from the first-generation compound.

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, the expected timing for initiating clinical studies, the expected timing for reporting topline data, the timing and future occurrence of other preclinical and clinical activities and the expected length of our cash runway. 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 filed annual report on Form 10-K. 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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Regulus Therapeutics Inc.
Selected Financial Information
Condensed Statement of Operations
(In thousands, except share and per share data)

	Three months ended March 31,	
	2023	2022
Operating expenses:		
Research and development	4,925	3,679
General and administrative	2,444	2,890
Total operating expenses	7,369	6,569
Loss from operations	(7,369)	(6,569)
Other income (expense), net	230	(149)
Loss before income taxes	(7,139)	(6,718)
Income tax expense	-	(1)
Net loss	$ (7,139)	$ (6,719)
Net loss per share, basic and diluted	$ (0.42)	$ (0.46)
Weighted average shares used to compute basic and diluted net loss per share:	16,844,243	14,596,789

	March 31, 2023	December 31, 2022
Cash, cash equivalents and short-term investments	$ 30,308	$ 39,160
Total assets	37,015	46,716
Term loan, less debt issuance costs	3,716	4,511
Stockholders' equity	26,595	33,291

SOURCE Regulus Therapeutics Inc.

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