Regulus Therapeutics Reports Fourth Quarter and Year-End 2022 Financial Results and Recent Updates

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

Top-line data in the first cohort of RGLS8429-treated ADPKD patients anticipated in second half of 2023

Expansion of team with key leadership appointments in clinical development, regulatory and clinical operations

SAN DIEGO, March 23, 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 fourth quarter and year ended December 31, 2022, and provided a corporate update.

"We ended 2022 in a position of strength, with enrollment in our Phase 1b MAD study under way. This momentum was reinforced by the appointments of Drs. Rekha Garg and Claire Padgett, who bring significant depth of industry, clinical and regulatory experience to help us advance development of RGLS8429," commented Jay Hagan, CEO of Regulus. "With three months of dosing and one month of follow-up for each patient in the study, we would anticipate topline data approximately five months after we complete enrollment of the first cohort."

Program Updates

RGLS8429 for ADPKD: The Phase 1b MAD study is a double-blind, placebo-controlled trial to assess safety, tolerability, and pharmacokinetics 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), 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 in the second half of 2023. The company also recently completed the in-life portion of the 27-week chronic mouse toxicity study for RGLS8429. No CNS toxicity was observed at all dose levels up to the top dose of 300 mg/kg administered every other week.

Corporate Highlights

Expanded Team: In November 2022, the Company announced the appointments of Rekha Garg, M.D., M.S., to Senior Vice President, Clinical Development and Regulatory, and Claire Padgett, Ph.D., to Senior Vice President, Clinical Operations. In addition, the company announced the promotions of Morgan Carlson, Ph.D., to Vice President, Biology and Edmund Lee, Ph.D., to Vice President, Translational Medicine.

Presented Data at the 5th Chronic Kidney Disease Drug Development Summit : On March 9, 2023, the Company presented preclinical data on RGLS8429 at the 2023 CKD Summit, entitled "Targeting miR-17 to Address the Genetic Cause of ADPKD." Drs. Edmund Lee and Rekha Garg represented the Company.

Financial Results

Cash, Cash Equivalents and Marketable Securities: As of December 31, 2022, Regulus had $39.2 million in cash, cash equivalents and short-term investments.

Research and Development (R&D) Expenses: Research and development expenses were $4.7 million and $18.4 million for the fourth quarter and year ended December 31, 2022, respectively, compared to $4.4 million and $17.8 million for the same periods in 2021, respectively. These amounts reflect internal and external costs associated with advancing our pipeline.

General and Administrative (G&A) Expenses: General and administrative expenses were $2.2 million and $9.8 million for the fourth quarter and year ended December 31, 2022, respectively, compared to $2.6 million and $10.0 million for the same periods in 2021, respectively. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was $6.8 million, or $0.40 per share (basic and diluted), and $28.3 million, or $1.86 per share (basic and diluted), for the fourth quarter and year ended December 31, 2022, compared to $7.1 million, or $0.67 per share (basic and diluted), and $27.8 million, or $3.24 per share (basic and diluted), for the same periods in 2021.

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, RGLS326. Regulus announced completion of the Phase 1 SAD study in September 2022 and dosed the first patient in the Phase 1b MAD study in early November. 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 is 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, including the expected timing for initiating clinical studies, 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 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.

Regulus Therapeutics Inc.

Selected Financial Information

Condensed Statement of Operations

(In thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	4,713	4,409	18,410	17,794
General and administrative	2,219	2,551	9,829	10,022
Total operating expenses	6,932	6,960	28,239	27,816
Loss from operations	(6,932)	(6,960)	(28,239)	(27,816)
Other income (expense), net	137	(173)	(83)	9
Loss before income taxes	(6,795)	(7,133)	(28,322)	(27,807)
Income tax expense	-	-	(1)	(1)
Net loss	$ (6,795)	$ (7,133)	$ (28,323)	$ (27,808)
Net loss per share, basic and diluted	$ (0.40)	$ (0.67)	$ (1.86)	$ (3.24)
Weighted average shares used to compute basic and diluted net loss per share:	16,839,700	10,689,683	15,259,958	8,569,854

	December 31, 2022	December 31, 2021

Cash, cash equivalents and short-term investments	$ 39,160	$ 60,383
Total assets	46,716	68,454
Term loan, less debt issuance costs	4,511	4,673
Stockholders' equity	33,291	54,958

SOURCE Regulus Therapeutics Inc.

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