Regulus Therapeutics Reports Third Quarter 2022 Financial Results and Recent Updates

Company announced first patient dosed in its Phase 1b Multiple-Ascending Dose (MAD) study of RGLS8429 in patients with Autosomal Dominant Polycystic Kidney Disease (ADPKD)

Company announced positive topline safety and pharmacokinetic (PK) data from its Phase 1 Single-Ascending Dose (SAD) study of RGLS8429 in healthy volunteers for the treatment of ADPKD

$4.5 million in net proceeds in At-the-Market (ATM) sale to a new institutional investor

SAN DIEGO, Nov. 10, 2022 /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 third quarter ended September 30, 2022 and provided a corporate update.

"We are pleased with the positive safety and PK data in healthy volunteers in our Phase 1 SAD study and are delighted to have the first patient dosed in our ongoing Phase 1b MAD study, which brings us a step forward in investigating RGLS8429 as a potential treatment for ADPKD," commented Jay Hagan, CEO of Regulus. "We anticipate topline data from the first cohort of patients in mid-2023."

Program Updates

RGLS8429 for ADPKD: In early November, the Company announced the dosing of the first patient in the Phase 1b MAD study of RGLS8429 for the treatment of 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, cystic 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.

In September 2022, the Company announced positive topline safety and PK data from its Phase 1 SAD clinical trial of RGLS8429. RGLS8429 was well-tolerated, with no serious adverse events reported. Among the 32 subjects treated with RGLS8429 or placebo, there were nine adverse events, all of which were mild, except one (sinus infection), which was graded moderate in severity. Preliminary results suggest plasma exposure is approximately linear across the four doses tested and is similar to the PK data from the first-generation compound, RGLS4326.

Corporate Highlights

Raised $4.5 Million in Net Proceeds Through its ATM Facility: A total of 2,205,100 shares were sold and settled for net proceeds of $4.5 million under the ATM facility during the three months ended September 30, 2022. Almost all the shares were sold to a new institutional investor. The Company believes its existing cash, cash equivalents, and short-term investments as of September 30, 2022 will now provide cash resources to fund current planned activities through 2023.

Company Presented Recent Data at the American Society of Nephrology Kidney Week 2022, which took place in Orlando, November 3-6, 2022: The poster, titled "Discovery of Next-generation Anti-miR-17 Oligonucleotide RGLS8429 for Treatment of Autosomal Dominant Polycystic Kidney Disease (ADPKD)," provides an overview of the data supporting the potential of RGLS8429 as a treatment for ADPKD.

Financial Results

Cash, Cash Equivalents and Marketable Securities: As of September 30, 2022, Regulus had $45.3 million in cash, cash equivalents and short-term investments.

Research and Development (R&D) Expenses: Research and development expenses were $5.3 million and $13.7 million for the three and nine months ended September 30, 2022, respectively, compared to $5.9 million and $13.4 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 $2.3 million and $7.6 million for the three and nine months ended September 30, 2022, respectively, compared to $2.5 million and $7.5 million for the same periods in 2021, respectively. These amounts reflect personnel-related and

ongoing general business operating costs.

Net Loss: Net loss was $7.6 million, or $0.50 per share (basic and diluted), and $21.5 million, or $1.46 per share (basic and diluted), for the three and nine months ended September 30, 2022, compared to $8.6 million, or $0.99 per share (basic and diluted), and $20.7 million, or $2.63 per share (basic and diluted), for the same periods in 2021.

Conference Call and Webcast Information: The Company will host a conference call and live audio webcast today at 5:00 p.m. Eastern Standard Time to discuss its third quarter 2022 financial results and corporate update. To access the call, please dial (866) 652-5200 (domestic) or (412) 317-6060 (international) and use the conference ID 10171026. To access the telephone replay of the call, dial (877) 344-7529 (domestic) or (412) 317-0088 (international) and refer to the entry replay code 5033234. The webcast and telephone replay will be archived on the Company's website at www.regulusrx.com following the call.

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. Preliminary results suggest plasma exposure is approximately linear across the four doses evaluated 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 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.

Regulus Therapeutics Inc.

Selected Financial Information

Condensed Statement of Operations

(In thousands, except share and per share data)

	Three months ended September 30,		Nine months ended September 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	5,310	5,915	13,697	13,385
General and administrative	2,253	2,504	7,610	7,471
Total operating expenses	7,563	8,419	21,307	20,856
Loss from operations	(7,563)	(8,419)	(21,307)	(20,856)
Other income (expense), net	12	(209)	(220)	182
Loss before income taxes	(7,551)	(8,628)	(21,527)	(20,674)
Income tax expense	-	-	(1)	(1)
Net loss	$ (7,551)	$ (8,628)	$ (21,528)	$ (20,675)
Net loss per share, basic and diluted	$ (0.50)	$ (0.99)	$ (1.46)	$ (2.63)
Weighted average shares used to compute basic and diluted net loss per share:	14,969,574	8,703,637	14,727,591	7,855,479

	September 30, 2022	December 31, 2021
	(Unaudited)	
Cash and cash equivalents	$ 45,324	$ 60,383

Total assets	52,127	68,454
Term loan, less debt issuance costs	4,478	4,673
Stockholders' equity	39,670	54,958

SOURCE Regulus Therapeutics Inc.

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