Regulus Therapeutics Reports First Quarter 2022 Financial Results and Recent Updates

FDA Acceptance of Investigational New Drug (IND) Application for RGLS8429 for Autosomal Dominant Polycystic Kidney Disease (ADPKD)

On track to initiate Phase 1 study in second quarter 2022

SAN DIEGO, May 12, 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 first quarter ended March 31, 2022 and provided a corporate update.

"The year is off to a great start as we continue to advance our pipeline, including the recent acceptance of our IND application for RGLS8429. We are thrilled to begin clinical development where we will investigate the safety, tolerability, pharmacokinetics, and preliminary efficacy of RGLS8429 for the treatment of ADPKD," stated Jay Hagan, CEO of Regulus. "We also entered into an exciting research collaboration with Brigham and Women's Hospital, which will further our understanding into broader applications of miR-155 inhibitors and potentially bring us closer to improving outcomes for Amyotrophic Lateral Sclerosis (ALS) patients. We look forward to providing updates in the coming quarters regarding progress on these fronts."

Program Updates

RGLS8429 for ADPKD: As announced yesterday, the U.S. Food and Drug Administration (FDA) recently accepted the Company's IND for RGLS8429 for the treatment of ADPKD**.** The Company plans to initiate a Phase 1 single-ascending dose (SAD) study in healthy volunteers to assess safety, tolerability and pharmacokinetics of RGLS8429. Following the SAD study, the Company plans to initiate a Phase 1b multiple ascending dose (MAD) study in adult patients with ADPKD to assess safety, tolerability and pharmacokinetics of RGLS8429, and to evaluate the dose response of RGLS8429 treatment on ADPKD biomarkers including polycystins, cystic kidney volume (htTKV), and overall kidney function. Top-line data from the healthy volunteer study are expected in the second half of 2022, and top-line biomarker data for the first cohort of RGLS8429-treated patients with ADPKD are expected in the first half of 2023.

Lademirsen (RG-012) for Alport syndrome: In February 2022, the Company announced completion of enrollment by Sanofi in the Phase 2 HERA clinical study evaluating lademirsen for the treatment of adult patients with Alport Syndrome under the Company's Collaboration and License Agreement with Sanofi. Final data are expected in the first half of 2023 and, if successful, could provide further validation of the Company's platform technology designed to address genetic kidney diseases and earn the Company a $25 million milestone.

Corporate Highlights

Collaboration Agreement with Brigham and Women's Hospital: In March 2022, the Company announced a collaboration agreement with the laboratories of Oleg Butovsky, Ph.D., and Howard L. Weiner, M.D., at Brigham and Women's Hospital to investigate the biologic effects of miR-155 inhibitors in both in vitro and in vivo models of ALS.

Financial Results

Cash Position: As of March 31, 2022, Regulus had $53.9 million in cash and cash equivalents.

Research and Development (R&D) Expenses: Research and development expenses were $3.7 million for the three months ended March 31, 2022, compared to $3.3 million for the same period in 2021. 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 $2.9 million for the three months ended March 31, 2022, compared to $2.5 million for the same period in 2021. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was $6.7 million, or $0.05 per share (basic and diluted), for the three months ended March 31, 2022, compared to $6.0 million, or $0.08 per share (basic and diluted), for the same period 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 Daylight Time to discuss its first quarter 2022 financial results and corporate update. To access the call, please dial (866) 652-5200 (domestic) or (412) 317-6060 (international). To access the telephone replay of the call, dial (877) 344-7529 (domestic) or (412) 317-0088 (international), passcode ID 6812601. 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 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. Approximately 140,000 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 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 and has demonstrated a superior pharmacologic profile compared to Regulus' first generation compound in preclinical studies. The U.S. Food and Drug Administration (FDA) accepted the Company's IND for RGLS8429 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, the expected timing for initiating a Phase 1 clinical study, the expected timing for reporting topline data, and the timing and future occurrence of data concerning the Company's preclinical programs. 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 March 31,	
	2022	**2021**
Operating expenses:		
Research and development	3,679	3,320
General and administrative	2,890	2,478
Total operating expenses	6,569	5,798
Loss from operations	(6,569)	(5,798)
Other expense, net	(149)	(215)
Loss before income taxes	(6,718)	(6,013)
Income tax expense	(1)	-
Net loss	$ (6,719)	$ (6,013)
Net loss per share, basic and diluted	$ (0.05)	$ (0.08)
Weighted average shares used to compute basic and diluted net loss per share	145,973,989	71,290,918

	March 31, 2022	December 31, 2021
Cash and cash equivalents	$ 53,902	$ 60,383
Total assets	60,985	68,454
Term loan, less debt issuance costs	4,674	4,673
Stockholders' equity	49,241	54,958

SOURCE Regulus Therapeutics Inc.

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