Regulus Therapeutics Reports Second Quarter 2022 Financial Results and Recent Updates

IND accepted and first subject dosed in the Phase 1 Single-Ascending Dose (SAD) study of RGLS8429 for the treatment of Autosomal Dominant Polycystic Kidney Disease (ADPKD)

RGLS8429 granted Orphan Drug Designation (ODD) from the U.S. Food and Drug Administration (FDA)

Scientific leadership strengthened with the appointment of Amin Kamel, Ph.D., as Vice President, Drug Metabolism and Pharmacokinetics (DMPK)

SAN DIEGO, Aug. 11, 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 second quarter ended June 30, 2022 and provided a corporate update.

"This has been an exciting quarter, which culminated in the achievement of multiple milestones. We continue to make steady progress with RGLS8429, evidenced by the dosing of the first subject in our Phase 1 SAD study," commented Jay Hagan, CEO of Regulus. "Moreover, we are pleased to have Dr. Amin Kamel join the Regulus team at this pivotal moment. We are proud of the work our team is doing to advance our novel therapeutic candidate for ADPKD patients, and we look forward to providing additional updates on progress in the coming quarters."

Program Updates

RGLS8429 for ADPKD: In early June 2022, the Company announced the dosing of the first subject in its Phase 1 SAD study of RGLS8429 for the treatment of ADPKD. The study will assess the safety, tolerability, and pharmacokinetics of RGLS8429 in healthy volunteers. The Company subsequently plans to initiate a Phase 1b multiple ascending dose (MAD) study to assess safety, tolerability, and pharmacokinetics of RGLS8429 in adult patients with ADPKD, and evaluate the efficacy of RGLS8429 treatment across three different dose levels measuring changes in polycystins, cystic kidney volume (htTKV), and overall kidney function.

In late June, the Company announced that FDA had granted ODD to RGLS8429 for the treatment of ADPKD. The FDA's Office of Orphan Products Development grants orphan designation status to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases, or conditions that affect fewer than 200,000 people in the U.S. Orphan designation status is intended to facilitate drug development for rare diseases and may provide several benefits to drug developers, including financial incentives, to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.

Corporate Highlights

Expanded Team: In June 2022, the Company announced the appointment of Amin Kamel, Ph.D., as Vice President, DMPK. Most recently, Dr. Kamel was the Scientific Director, DMPK at Takeda, where he also served as a scientific advisor. Prior to Takeda, Dr. Kamel held positions at Biogen, Novartis, and Pfizer.

Financial Results

Cash, Cash Equivalents and Marketable Securities: As of June 30, 2022, Regulus had $47.5 million in cash, cash equivalents and marketable securities.

Research and Development (R&D) Expenses: Research and development expenses were $4.7 million and $8.4 million for the three and six months ended June 30, 2022, respectively, compared to $4.2 million and $7.5 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 $2.5 million and $5.4 million for the three and six months ended June 30, 2022, respectively, compared to $2.5 million and $5.0 million for the same periods in 2021, respectively. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was $7.3 million, or $0.50 per share (basic and diluted), and $14.0 million, or $0.96 per share (basic and diluted), for the three and six months ended June 30, 2022, compared to $6.0 million,

or $0.78 per share (basic and diluted), and $12.0 million, or $1.62 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 Daylight Time to discuss its second 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) and refer to the entry replay code 5578933. 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. Regulus is currently conducting a Phase 1 single-ascending dose study in healthy volunteers to assess safety, tolerability, and pharmacokinetics of RGLS8429. In June 2022, FDA granted ODD for RGLS849 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 June 30,		Six months ended June 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	4,708	4,150	8,387	7,470
General and administrative	2,467	2,488	5,357	4,967
Total operating expenses	7,175	6,638	13,744	12,437
Loss from operations	(7,175)	(6,638)	(13,744)	(12,437)
Other (expense) income, net	(83)	605	(232)	391
Loss before income taxes	(7,258)	(6,033)	(13,976)	(12,046)
Income tax expense	-	(1)	(1)	(1)
Net loss	$ (7,258)	$ (6,034)	$ (13,977)	$ (12,047)
Other comprehensive loss:				
Unrealized loss on short-term investments, net	(36)	-	(36)	-
Comprehensive loss	(7,294)	(6,034)	(14,013)	(12,047)
Net loss per share, basic and diluted	$ (0.50)	$ (0.78)	$ (0.96)	$ (1.62)
Weighted average shares used to compute basic and diluted net loss per share:	14,612,312	7,716,982	14,604,594	7,424,371

	June 30, 2022	**December 31, 2021**
Cash, cash equivalents and short-term investments	$ 47,534	$ 60,383

Total assets	54,298	68,454
Term loan, less debt issuance costs	4,446	4,673
Stockholders' equity	42,367	54,958

SOURCE Regulus Therapeutics Inc.

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