Regulus Therapeutics Reports Fourth Quarter and Year-End 2021 Financial Results and Recent Updates

- Successfully completed Pre-IND meeting with FDA for Autosomal Dominant Polycystic Kidney Disease (ADPKD) Program -

- On track to submit IND and Initiate Phase 1 study in second quarter 2022 -

- Enrollment completed in Phase 2 clinical trial of Lademirsen for Alport Syndrome -

- Closed $34.6 million private placement of equity extending cash runway through 2023 -

SAN DIEGO, March 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 announced its financial results for the fourth quarter and year ended December 31, 2021 and provided a corporate update.

"Last year was a transformational year for Regulus with the accomplishments in our ADPKD program including clinical validation of the target and the prioritization and advancement of our next generation compound RGLS8429," commented Jay Hagan, CEO of Regulus Therapeutics. "We are on track with an IND filing and initiation of our Phase 1 clinical study and with the recent equity financing we believe the Company is poised and capitalized to deliver on upcoming milestones."

Program Updates

RGLS8429 for ADPKD: In January 2022, the Company announced the successful completion of a pre-investigational new drug (Pre-IND) meeting with the U.S. Food and Drug Administration (FDA). The FDA provided overall agreement with the trial design and length of the Phase 1 study. Based on this feedback, the company is on track to submit an IND application in the second quarter of 2022 to obtain clearance for the initiation of the Phase 1 clinical trial. The Company anticipates reporting top-line data from the healthy volunteer portion of the study in the second half of 2022, and top-line biomarker data in the first cohort of RGLS8429-treated patients with ADPKD in the first half of 2023.

Lademirsen (RG-012) for Alport syndrome: In February 2022, the Company announced completion of enrollment 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 is 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, furthering the cash runway into 2024.

Corporate Highlights

Closed $34.6 Million Private Placement: In November 2021, the Company announced the closing of a private placement of equity. The financing was led by the Federated Hermes Kaufmann Funds and New Enterprise Associates (NEA), with participation from other new and existing investors. The Company received gross proceeds of approximately $34.6 million from the sale of 58,923,352 shares of the Company's common stock ("Common Stock") at a purchase price of $0.36 per share. In addition, the Company sold 3,725,720 shares of non-voting Class A-4 convertible preferred stock, in lieu of shares of Common Stock, at a price of $3.60 per share. Each share of non-voting Class A-4 convertible preferred stock is convertible into 10 shares of Common Stock, subject to certain beneficial ownership conversion limitations. Net proceeds from the transaction will be used for non-clinical and clinical development activities for the Company's product candidates and general corporate purposes. SVB Leerink acted as the lead placement agent for the financing and H.C. Wainwright and Co. acted as co-placement agent.

Expanded Team: In December 2021, the Company announced the appointment of Mohammad Ahmadian, Ph.D., as Vice President, Chemistry and Pharmaceutical Development. Dr. Ahmadian has worked for various life sciences companies in research and managerial capacities. Most recently, he was Vice President & Resident Director of Kinovate Life Sciences, Inc.

Financial Results

Cash Position: As of December 31, 2021, Regulus had $60.4 million in cash and cash equivalents.

Research and Development (R&D) Expenses: Research and development expenses were $4.4 million and $17.8 million for the fourth quarter and year ended December 31, 2021, respectively, compared to $4.0 million and $15.3 million for the same periods in 2020, respectively. 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 $2.6 million and $10.0 million for the fourth quarter and year ended December 31, 2021, respectively, compared to $2.1 million and $8.8 million for the same periods in 2020, respectively. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was $7.1 million, or $0.07 per share (basic and diluted), and $27.8 million, or $0.32 per share (basic and diluted), for the fourth quarter and year ended December 31, 2021, respectively, compared to $1.3 million, or $0.03 per share (basic and diluted), and $15.7 million, or $0.45 per share (basic and diluted), respectively, for the same periods in 2020.

Conference Call and Webcast Information:
The Company will host a conference call and live audio webcast today at 5:00 p.m. ET to discuss its fourth quarter and year end 2021 financial results and corporate update. To access the call, please dial (877) 257-8599 (domestic) or (970) 315-0459 (international) and refer to conference ID 3786683. To access the telephone replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode ID 3786683. The webcast and telephone replay will be archived on the Company's website at www.regulusrx.com following the call.

About ADPKD

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.

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, as well as a superior pharmacologic profile have been demonstrated. Regulus has nominated RGLS8429 as a clinical candidate 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, including the potential sufficiency of the preclinical data required to support clinical studies, the expected timing for submitting an IND and initiating a Phase 1 clinical study, the expected timing for reporting topline data, and the timing and future occurrence of clinical data concerning lademirsen. 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 December 31,		Year ended December 31,	
	2021	2020	2021	2020
Revenues:				
Revenue under strategic alliances	$ 0	$ 5,000	$ 0	$ 10,006
Operating expenses:				
Research and development	4,409	3,951	17,794	15,347
General and administrative	2,551	2,078	10,022	8,814
Total operating expenses	6,960	6,029	27,816	24,161
Loss from operations	(6,960)	(1,029)	(27,816)	(14,155)
Other (expense) income, net	(173)	(286)	9	(1,575)
Loss before income taxes	(7,133)	(1,315)	(27,807)	(15,730)
Income tax expense	0	(7)	(1)	0
Net loss	$ (7,133)	$ (1,322)	$ (27,808)	$ (15,730)
Net loss per share, basic and diluted	$ (0.07)	$ (0.03)	$ (0.32)	$ (0.45)
Weighted average shares used to compute basic and diluted net loss per share:	106,902,912	47,731,012	85,704,535	34,977,378

	December 31, 2021	December 31, 2020
Cash and cash equivalents	$ 60,383	$ 31,087
Total assets	68,454	37,604
Term loan, less debt issuance costs	4,673	4,652
Stockholders' equity	54,958	26,026

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