Regulus Reports Fourth Quarter and Year-End 2019 Financial Results and Recent Updates

March 12, 2020

FDA Removal of Partial Clinical Hold for Phase I Multiple Ascending Dose Study of RGLS4326
Reinitiated Multiple Ascending Dose Study of RGLS4326 for the Treatment of ADPKD
Closed $26 Million Second Tranche of Private Financing

LA JOLLA, Calif., March 12, 2020 /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, 2019 and provided a corporate update.

"We have made significant progress over the past few months, including receiving notification from FDA of their decision to lift the partial clinical hold on our Phase 1 multiple ascending dose clinical study for RGLS4326, enabling the initiation of dosing of the second cohort of that study for the treatment of autosomal dominant polycystic kidney disease this past February," said Jay Hagan, CEO of Regulus. "This important milestone, coupled with the closing of the second tranche of financing, enables us to advance the program toward key data read-outs."

Corporate Highlights

Closed $26 Million Second Tranche of Private Financing: In December 2019, following the announcement of the Company's plan to recommence the Phase 1 Multiple Ascending Dose ("MAD") clinical study of RGLS4326 for the treatment of autosomal dominant polycystic kidney disease ("ADPKD") in the first quarter of 2020, the Company completed a second and final closing under the May 2019 securities purchase agreement, pursuant to which the Company sold and issued 3,288,390 shares of non-voting Class A-2 convertible preferred stock, in lieu of shares of common stock, at a price of $6.66 per share, and accompanying warrants to purchase an aggregate of 32,883,900 shares of common stock at a price of $0.125 for each share of common stock underlying such warrants. Each share of the non-voting Class A-2 convertible preferred stock is convertible into 10 shares of common stock, subject to certain beneficial ownership conversion limitations. The warrants are exercisable for a period of five years following the date of issuance and have an exercise price of $0.666 per share, pursuant to proportional adjustments in the event of stock splits or combinations or similar events. Together with the first tranche, which closed in May 2019, the Company raised a total of $42.7 million from the private financing, which the Company expects will provide cash resources to fund planned activities into mid-2021.

Program Highlights

Initiated Dosing of the Second Cohort in RGLS4326 Phase 1 for ADPKD: In February 2020, the Company initiated dosing of the second cohort of the MAD clinical study of RGLS4326, a novel oligonucleotide designed to inhibit miR-17 for the treatment of ADPKD. The Company expects to complete this study in mid-2020 with topline results available thereafter. The Company is also planning to initiate a Phase 1b short-term dosing study in patients with ADPKD in the second half of 2020 to evaluate RGLS4326 for safety, pharmacokinetics, and biomarkers of pharmacodynamic activity. The re-initiation of the MAD study was allowed following a satisfactory complete response provided to FDA in November 2019 to address the partial clinical hold placed on the clinical program in July 2019. A partial clinical hold on studies of RGLS4326 dosed for extended durations remains in effect until the second set of requirements outlined by FDA have been satisfactorily addressed. Information from the Phase 1 clinical studies, together with information from additional nonclinical studies, will be used to address the second set of requirements to support studies of extended duration.

Financial Results

Cash Position: As of December 31, 2019, Regulus had $34.1 million in cash and cash equivalents.

Research and Development (R&D) Expenses: R&D expenses were $2.1 million and $12.3 million for the quarter and year ended December 31, 2019, respectively, compared to $5.3 million and $34.0 million for the same periods in 2018. The decreases were largely driven by decreases in external development expenses, primarily attributable to the pause of the RGLS4326 MAD study in the third quarter of 2018 and transfer of the RG-012 program to Sanofi beginning in the fourth quarter of 2018. Additionally, the decreases were driven by reductions in personnel and internal expenses, primarily attributable to a reduction in costs subsequent to our corporate restructuring in the third quarter of 2018.

General and Administrative (G&A) Expenses: G&A expenses were $2.4 million and $11.3 million for the quarter and year ended December 31, 2019, respectively, compared to $2.7 million and $12.9 million for the same periods in 2018. The decreases were mostly driven by a reduction in costs subsequent to our corporate restructuring in the third quarter of 2018.

Revenue: Revenue was less than $0.1 million and $6.8 million for the quarter and year ended December 31, 2019, respectively, compared to less than $0.1 million and $0.1 million for the same periods in 2018. The increase for the year ended December 31, 2019 was attributable to revenue recognition of the upfront payments received under the 2018 Sanofi Amendment related to the transfer of the RG-012 program to Sanofi.

Net Loss: Net loss was $4.9 million and $18.6 million for the quarter and year ended December 31, 2019, respectively, compared to a net loss of $8.6 million and $48.7 million for the same periods in 2018. Basic and diluted net loss per share was $0.23 and $1.08 for the quarter and year ended December 31, 2019, respectively, compared to $0.98 and $5.59 for the same periods in 2018.

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 RGLS4326

RGLS4326 is a novel oligonucleotide designed to inhibit miR-17 and designed to preferentially target the kidney. Preclinical studies with RGLS4326 have demonstrated direct regulation of PKD1 and PKD2 in human ADPKD cyst cells, a reduction in kidney cyst formation, improved kidney weight/body weight ratio, decreased cyst cell proliferation, and preserved kidney function in mouse models of ADPKD. The RGLS4326 IND is currently on a Partial Clinical Hold for treatment of extended duration by the U.S. Food and Drug Administration.

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 La Jolla, CA.

Forward-Looking Statements

Statements contained in this press release 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 completion of preclinical and clinical activities concerning the RGLS4326 program, the sufficiency of the data resulting from the ongoing or planned preclinical studies required to recommence clinical studies for extended duration dosing and the timing of 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 feedback from the FDA. These and other risks are described in additional detail in Regulus' filings with the Securities and Exchange Commission. 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,	
	2019	2018	2019	2018
Revenues:				
Revenue under strategic alliances	$ 18	$ 18	$ 6,832	$ 72
Operating expenses:				
Research and development	2,090	5,255	12,349	33,975
General and administrative	2,363	2,745	11,317	12,860
Total operating expenses	4,453	8,000	23,666	46,835
Loss from operations	(4,435)	(7,982)	(16,834)	(46,763)
Other expense, net	(458)	(520)	(1,757)	(1,884)

Loss before income taxes	(4,893)	(8,502)	(18,591)	(48,647)
Income tax expense	-	(61)	(1)	(62)
Net loss	$ (4,893)	$ (8,563)	$ (18,592)	$ (48,709)
Net loss per share, basic and diluted	$ (0.23)	$ (0.98)	$ (1.08)	$ (5.59)
Weighted average shares used to compute basic and diluted net loss per share:	20,950,602	8,780,779	17,260,176	8,718,563

	December 31, 2019	December 31, 2018
Cash and cash equivalents	$ 34,121	$ 13,935
Total assets	42,081	27,927
Term loan, less debt issuance costs	14,631	16,575
Stockholders' equity (deficit)	20,015	(5,854)

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