Regulus Reports Fourth Quarter and Year-End 2018 Financial Results and Recent Updates

March 18, 2019

LA JOLLA, Calif., March 18, 2019 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs, today reported financial results for the fourth quarter and year ended December 31, 2018 and provided a corporate update.

"2018 was a challenging year for Regulus, but I am encouraged by our program advancements and recent corporate and cost-saving efforts. Importantly, we have nearly completed the transition of the RG-012 program to Sanofi, have positioned the Company to advance our prioritized pipeline, and significantly reduced our operating cash burn. In January, we submitted a comprehensive data package to FDA for RGLS4326 for the treatment of Autosomal Dominant Polycystic Kidney Disease, or ADPKD, and look forward to their feedback, which we hope will define the path forward to resume clinical development," said Jay Hagan, President and Chief Executive Officer of Regulus.

Corporate Updates

- **Restructuring of Sanofi Collaboration and Transition of RG-012 to Sanofi:** In November 2018, Regulus announced that it amended and restructured its Collaboration and License Agreement with Sanofi (the "Amendment"). Under the Amendment, Regulus granted Sanofi a worldwide exclusive license to develop and commercialize its investigational drug targeting miR-21 for all indications, including Alport syndrome. Under the terms of the Amendment, Regulus is eligible to receive approximately $7 million in upfront and material transfer payments. Regulus is also eligible to receive up to $40 million in development milestone payments. In addition, Sanofi will reimburse Regulus for certain out-of-pocket transition activities and assume Regulus' upstream license royalty obligations. The transition activities for the RG-012 program to Sanofi are nearly complete and upon completion will trigger the remainder of the upfront payment due to Regulus of $2.5 million.
- **Term Loan Amendments:** In January 2019 and March 2019, the Company amended its Term Loan with Oxford Finance to provide for additional periods of interest only for the months of February 2019 and March 2019, respectively. The maturity date of the Term Loan remains unchanged.
- **Lease Agreement:** In February 2019, the Company entered into an amendment of its current lease (the "Lease Amendment") for approximately 59,248 square feet located at 10614 Science Center Drive, San Diego, California 92121. Under the terms of the Lease Amendment, the expiration of the current lease will be accelerated from April 30, 2024 to April 1, 2019. Concurrently with the Lease Amendment, the Company entered into a new lease agreement (the "Lease") for approximately 24,562 square feet at 10628 Science Center Drive, Suite 100, San Diego, California, 92121 which it expects to use as its new principal offices and laboratory for research and development. The commencement date of the Lease is expected to be April 1, 2019. This relocation will reduce the Company's facility size by approximately 60% and

reduce its future contractual lease obligations by approximately 70%.

Financial Results

Cash Position: As of December 31, 2018, Regulus had cash and cash equivalents of $13.9 million.

Research and Development (R&D) Expenses: R&D expenses were $5.3 million and $34.0 million for the quarter and year ended December 31, 2018, respectively, compared to $10.5 million and $53.2 million for the same periods in 2017. The decreases in R&D expenses were primarily attributable to a reduction in external development expenses associated with RG-012 during the negotiation and transfer of the program to Sanofi in the second half of 2018 and a reduction in personnel-related costs subsequent our corporate restructurings.

General and Administrative (G&A) Expenses: G&A expenses were $2.7 million and $12.9 million for the quarter and year ended December 31, 2018, respectively, compared to $3.3 million and $17.0 million for the same periods in 2017. The decreases in G&A expenses were primarily driven by non-recurring severance charges and non-recurring, non-cash stock-based compensation charges recorded during the year ended December 31, 2017 in connection with our May 2017 corporate restructuring.

Revenue: Revenue was less than $0.1 million for the quarters ended December 31, 2018 and 2017, and $0.1 million for the years ended December 31, 2018 and 2017.

Net Loss: Net loss was $8.6 million and $48.7 million for the quarter and year ended December 31, 2018, respectively, compared to a net loss of $14.4 million and $71.9 million for the same periods in 2017. Basic and diluted net loss per share was $0.98 and $5.59 for the quarter and year ended December 31, 2018, respectively, compared to $1.67 and $11.47 for the same periods in 2017.

About Autosomal Dominant Polycystic Kidney Disease (ADPKD)

ADPKD, caused by the mutations in the PKD1 or PKD2 genes, is among the most common human monogenic disorders and a leading genetic cause of end-stage renal disease. The clinical hallmark of this disease is the development of multiple fluid filled cysts primarily in the kidneys and to a lesser extent in the liver and other organs. Excessive kidney tubule derived cyst cell proliferation, a central pathological feature, fuels the expansion of cysts, ultimately causing end-stage renal disease in approximately 50% of ADPKD patients by age 60. Approximately 1 in 1,000 people bear a mutation in either PKD1 or PKD2 genes worldwide.

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.

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. For more information, please visit http://www.regulusrx.com.

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 expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-012, RGLS4326 and its ability to recommence human clinical trials, RGLS5579, or its Hepatitis B Virus or NASH programs), the projected timeline of clinical development activities, the anticipated engagement with the FDA regarding the Regulus' RGLS4326 program and the timing thereof, the projected timeline of clinical development activities, and expectations regarding future therapeutic and commercial potential of Regulus' business plans, technologies and intellectual property related to microRNA therapeutics and biomarkers being discovered and developed by Regulus. 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. These and other risks concerning Regulus' financial position and programs 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,	
	2018	2017	2018	2017
Revenues:				
Revenue under strategic alliances	$ 18	$ 18	$ 72	$ 72
Operating expenses:				

Research and development	5,255	10,465	33,975	53,192
General and administrative	2,745	3,259	12,860	17,011
Total operating expenses	8,000	13,724	46,835	70,203
Loss from operations	(7,982)	(13,706)	(46,763)	(70,131)
Other expense, net	(520)	(800)	(1,884)	(1,971)
Loss before income taxes	(8,502)	(14,506)	(48,647)	(72,102)
Income tax (expense) benefit	(61)	58	(62)	197
Net loss	$ (8,563)	$ (14,448)	$ (48,709)	$ (71,905)
Net loss per share, basic and diluted	$ (0.98)	$ (1.67)	$ (5.59)	$ (11.47)
Weighted average shares used to compute basic and diluted net loss per share:	8,780,779	8,663,440	8,718,563	6,269,758

	December 31, 2018	December 31, 2017
Cash, cash equivalents and short-term investments	$ 13,935	$ 60,074
Total assets	27,927	77,809
Term loan, less debt issuance costs	16,575	19,859
Stockholders' (deficit) equity	(5,854)	35,216

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