



Regulus Reports Fourth Quarter and Year-end 2017 Financial Results and Pipeline Update

March 7, 2018

**Full enrollment of RG-012 studies anticipated in 2H 2018
RGLS4326 Phase I study on track
Conference call today at 5:00 p.m. ET**

LA JOLLA, Calif., March 7, 2018 /PRNewswire/ -- [Regulus Therapeutics Inc.](http://www.regulusrx.com) (Nasdaq: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today reported financial results for the fourth quarter and year ended December 31, 2017 and provided a pipeline update.

Regulus Therapeutics Inc. Logo

"2017 was a transitional year for Regulus. We made important changes to our portfolio and applied key learnings from earlier clinical and preclinical programs, which we believe position us for future success," said Jay Hagan, President and Chief Executive Officer of Regulus.

Mr. Hagan continued, "The RG-012 start-up activities and enrollment of patients with Alport syndrome have proven more challenging than anticipated. We have added internal and external resources to accelerate patient recruitment. The Phase I single ascending dose (SAD) study for RGLS4326 is progressing well, and planning for the multiple ascending dose (MAD) portion is underway."

Pipeline Update

- **RG-012 for Alport syndrome:** Patient recruitment activities for the Phase II HERA and the renal biopsy studies are on-going. Based on revised enrollment assumptions, the Company believes that both studies will be fully enrolled in the second half of 2018.
- **RGLS4326 for autosomal dominant polycystic kidney disease (ADPKD):** A Phase I SAD study was initiated in December 2017. Data from this study in healthy volunteers will provide pharmacokinetics and safety data, and the study is currently on track for completion in the third quarter 2018.

Financial Results

Cash Position: As of December 31, 2017, Regulus had cash, cash equivalents, and short-term investments of \$60.1 million.

Research and Development (R&D) Expenses: R&D expenses were \$10.5 million and \$53.2 million for the quarter and year ended December 31, 2017, respectively, compared to \$15.0 million and \$64.3 million for the same periods in 2016. The fourth quarter decrease was primarily the result of a reduction in personnel-related costs, subsequent to our May 2017 corporate restructuring. The year-over-year decrease was further driven by the wind-down of clinical activities related to the RG-101 program.

General and Administrative (G&A) Expenses: G&A expenses were \$3.3 million and \$17.0 million for the quarter and year ended December 31, 2017, respectively, compared to \$4.8 million and \$18.4 million for the same periods in 2016. The decreases in G&A expenses were primarily attributable to a reduction in non-cash stock-based compensation.

Revenue: Revenue was less than \$0.1 million and \$0.1 million for the quarter and year ended December 31, 2017, respectively, compared to less than \$0.1 million and \$1.2 million for the same periods in 2016.

Net Loss: Net loss was \$14.4 million and \$71.9 million for the quarter and year ended December 31, 2017, respectively, compared to a net loss of \$20.0 million and \$81.8 million for the same periods in 2016. Basic and diluted net loss per share was \$0.14 and \$0.96 for the quarter and year ended December 31, 2017, respectively, compared to \$0.38 and \$1.55 for the same periods in 2016.

Conference Call Details

Regulus will host a conference call and webcast today at 5:00 p.m. Eastern Time to discuss fourth quarter financial results and provide a general business update. A live webcast of the call will be available online at www.regulusrx.com. To access the call, please dial (877) 257-8599 (domestic) or (970) 315-0459 (international) and refer to conference ID 4794278. To access the replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), conference ID 4794278. The webcast and telephone replay will be archived on the company's website following the call.

About Regulus

Regulus Therapeutics Inc. (Nasdaq: RGLS) is a clinical stage biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a well-balanced microRNA therapeutics

pipeline complemented by a rich intellectual property estate to retain its leadership in the microRNA field. Regulus is advancing several programs in renal, hepatic and central nervous systems diseases. 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 or RGLS4326), 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,	
	2017	2016	2017	2016
Revenues:				
Revenue under strategic alliances	\$ 18	\$ 18	\$ 72	\$ 1,194
Operating expenses:				
Research and development	10,465	14,979	53,192	64,305
General and administrative	3,259	4,783	17,011	18,391
Total operating expenses	<u>13,724</u>	<u>19,762</u>	<u>70,203</u>	<u>82,696</u>
Loss from operations	(13,706)	(19,744)	(70,131)	(81,502)
Other expense, net	(800)	(271)	(1,971)	(338)
Loss before income taxes	(14,506)	(20,015)	(72,102)	(81,840)
Income tax benefit (expense)	58	(5)	197	4
Net loss	<u>\$ (14,448)</u>	<u>\$ (20,020)</u>	<u>\$ (71,905)</u>	<u>\$ (81,836)</u>
Net loss per share, basic and diluted	<u>\$ (0.14)</u>	<u>\$ (0.38)</u>	<u>\$ (0.96)</u>	<u>\$ (1.55)</u>
Weighted average shares used to compute basic and diluted net loss per share:	<u>103,955,147</u>	<u>52,923,713</u>	<u>75,230,762</u>	<u>52,813,474</u>

	December 31, 2017	December 31, 2016
Cash, cash equivalents and short-term investments	\$ 60,074	\$ 76,111
Total assets	77,809	100,661
Term loan, less debt issuance costs	19,859	19,802
Stockholders' equity	35,216	56,075

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