



Regulus Therapeutics Reports Third Quarter 2019 Financial Results and Recent Updates

November 12, 2019

Receipt of Final Reports from the Chronic Toxicity Studies for Submission of Complete Response to Reinitiate the Multiple Ascending Dose Clinical Study for RGLS4326 Appointment of Cris Calsada as New Chief Financial Officer

LA JOLLA, Calif., Nov. 12, 2019 /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 third quarter ended September 30, 2019 and provided a summary of recent events.



"We recently received the final reports from the chronic toxicity studies in mice and non-human primates and are preparing our submission of our complete response to Food and Drug Administration ("FDA") in our efforts to address the requirements to reinitiate the Multiple Ascending Dose ("MAD") clinical study for RGLS4326," said Jay Hagan, CEO of Regulus. "We look forward to FDA's feedback and continued productive dialogue."

Third Quarter 2019 Corporate Highlights and Recent Updates

- **Management Transition to New Chief Financial Officer:** In July 2019, the Company appointed Cris Calsada as its new Chief Financial Officer, effective August 30, 2019. Ms. Calsada's appointment follows the resignation of the Company's previous Chief Financial Officer, Dan Chevallard, in July 2019.
- **New Lease Agreement Significantly Reduces Contractual Lease Obligations:** In June 2019, the Company entered into an amendment of its lease (the "Lease Amendment") of 24,562 square feet located at 10628 Science Center Drive Suite 100, San Diego, California 92121. Under the terms of the Lease Amendment, the expiration of the lease was accelerated from June 30, 2023 to June 30, 2019, and the lease terminated on July 1, 2019. Concurrently with the Lease Amendment, the Company entered into a new lease agreement for 8,727 square feet located at 10628 Science Center Drive, Suite 225, San Diego, California, 92121, which it uses as its new principal offices and laboratory for research and development. This relocation reduced the Company's facility size by approximately 65% and reduced its future contractual lease obligations by approximately 78%.

Program Updates

- **RGLS4326 for Autosomal Dominant Polycystic Kidney Disease ("ADPKD"):** RGLS4326, a novel oligonucleotide designed to inhibit miR-17, is in Phase 1 clinical development for the treatment of ADPKD and currently on a partial clinical hold. In January 2019, the Company submitted a comprehensive data package for RGLS4326 to FDA that included the

results from the planned 13-week interim analysis of the repeat mouse chronic toxicity study, as well as results from additional investigations, analytical testing, additional data from the previously terminated mouse chronic toxicity study, data from the completed Phase I single ascending dose ("SAD") study and data from the first cohort of the Phase I MAD study, to support its plan to resume the Phase I MAD study. After review of the requested submission, FDA notified the Company in July 2019 of additional nonclinical data requirements and placed the IND on a partial clinical hold, formalizing the specific requirements to initiate the MAD study and further proceed into chronic dosing in ADPKD patients. The additional data requirements have been outlined in two parts. In order to resume the MAD study, FDA has requested the final reports from the chronic toxicity studies in both mice and non-human primates and satisfactory related analyses to ensure subjects can be safely dosed. Additional information and analyses from new nonclinical studies, planned to be generated over the next several quarters, are required for chronic dosing and will be submitted at a later date. The Company recently received the final reports from the chronic toxicity studies in mice and non-human primates and is preparing to submit a complete response to the partial clinical hold in order to be able to resume the MAD study, pending agreement with FDA. Regulus anticipates a response from FDA before the end of the year. Regulus is allowed to proceed with additional SAD clinical studies as part of the process to gather additional supporting information to guide the future development of the program.

In September 2019, the Company announced that Nature Communications published an article entitled, "Discovery and preclinical evaluation of anti-miR-17 for the treatment of polycystic kidney disease." The article highlights preclinical research that identifies RGLS4326, a first-in-class anti-miR-17 oligonucleotide, as a potential disease-modifying treatment for ADPKD. In addition, a poster highlighting this work was presented at the Oligonucleotide Therapeutics Society (OTS) Annual Meeting held October 13-16, 2019 in Munich, Germany.

Third Quarter 2019 Financial Results

Cash Position: As of September 30, 2019, Regulus had \$14.6 million in cash and cash equivalents.

Revenue: Revenue was less than \$0.1 million and \$6.8 million for the three and nine months ended September 30, 2019, respectively, compared to less than \$0.1 million and \$0.1 million for the three and nine months ended September 30, 2018. The increase for the nine months ended September 30, 2019 was attributable to revenue recognition of the upfront payments received under the 2018 Sanofi Amendment related to the transfer of RG-012.

Research and Development (R&D) Expenses: R&D expenses were \$2.4 million and \$10.3 million for the three and nine months ended September 30, 2019, compared to \$6.9 million and \$28.7 million for the same periods in 2018. The decreases were driven by decreases in external development expenses, primarily attributable to the voluntary pause of the RGLS4326 Phase 1 MAD clinical study in the third quarter of 2018 and commencement of the transfer of the RG-012 program to Sanofi 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 \$2.6 million and \$9.0 million for the three and nine months ended September 30, 2019, compared to \$3.0 million and \$10.1 million for the same periods in 2018. These amounts reflect personnel-related and ongoing general business operating costs. The decreases were driven by a reduction in costs subsequent to our corporate restructuring in the third quarter of 2018.

Net Loss: Net loss was \$5.4 million, or \$0.26 per share (basic and diluted), and \$13.7 million, or \$0.86 per share (basic and diluted), for the three and nine months ended September 30, 2019, respectively, compared to \$10.3 million, or \$1.18 per share (basic and diluted), and \$40.1 million, or \$4.62 per share (basic and diluted), for the same periods in 2018. Historical and current period net loss per share values have been retroactively adjusted to reflect our October 2018 reverse stock split.

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 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 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, California. 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, RGLS5579 or its other

preclinical programs), its estimated cash runway, the projected timeline of clinical development activities, the sufficiency of data supporting its complete response and the timing of regulatory submissions regarding RGLS4326 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 September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
Revenues:				
Revenue under strategic alliances	\$ 18	\$ 18	\$ 6,814	\$ 54
Operating expenses:				
Research and development	2,440	6,879	10,259	28,720
General and administrative	2,571	2,993	8,954	10,115
Total operating expenses	5,011	9,872	19,213	38,835
Loss from operations	(4,993)	(9,854)	(12,399)	(38,781)
Other expense, net	(430)	(419)	(1,299)	(1,365)
Loss before income taxes	(5,423)	(10,273)	(13,698)	(40,146)
Income tax expense	-	-	(1)	-
Net loss	\$ (5,423)	\$ (10,273)	\$ (13,699)	\$ (40,146)
Net loss per share, basic and diluted	\$ (0.26)	\$ (1.18)	\$ (0.86)	\$ (4.62)
Weighted average shares used to compute basic and diluted net loss per share:	20,849,083	8,703,626	16,016,515	8,688,831

	September 30, 2019	December 31, 2018
	(Unaudited)	
Cash and cash equivalents	\$ 14,646	\$ 13,935
Total assets	21,410	27,927
Term loan, less debt issuance costs	14,626	16,575
Stockholders' equity (deficit)	(143)	(5,854)

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