

Regulus Therapeutics Reports First Quarter 2019 Financial Results and Recent Updates

*Closing of First Tranche of \$41.8 Million Private Placement of Equity
Term Loan Restructured Providing Up to Two Years of Interest Only and Extended Maturity Date to May 2022
Engagement with FDA on RGLS4326 Clinical Program Ongoing*

LA JOLLA, Calif., May 9, 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 first quarter ended March 31, 2019 and provided a summary of recent events.

"I am proud of the significant progress we have made thus far in 2019 to reposition Regulus with a strengthened balance sheet, restructured term loan and reduced operating cash burn rate. Importantly, the team has maintained its focus in its engagement with FDA, and we look forward to resuming our ADPKD clinical program pending FDA alignment. We believe the total potential proceeds from the recent financing, highlighted by a strong investor syndicate, provides sufficient capital to fund planned activities into the second half of 2021," said Jay Hagan, CEO of Regulus. "We look forward to providing future updates on our ADPKD program, as well as further advancements from our promising preclinical pipeline."

First Quarter 2019 Corporate Highlights and Recent Updates

- **Private Financing:** In May 2019, the Company closed the first tranche of its \$41.8 million private placement of equity (the "Private Placement"). Regulus received net proceeds of approximately \$15.7 million from the first tranche, after deducting placement agent fees and other offering expenses. Subject to the Company's public announcement on or before December 31, 2019 of its plan to recommence the Phase 1 multiple ascending dose clinical trial for RGLS4326 based upon correspondence from FDA, the investors who purchased securities in the first tranche of the Private Placement have agreed to purchase shares of non-voting convertible preferred stock and accompanying warrants to purchase shares of common stock in a second closing (the "Milestone Closing"). If the Milestone Closing occurs, the gross proceeds to the Company from that closing will be approximately \$25.1 million. The Company expects to use the proceeds from the Private Placement primarily to advance RGLS4326 for the treatment of ADPKD, to advance select programs from its pipeline of microRNA therapies and for general corporate purposes.
- **Term Loan Amendments:** In January 2019, March 2019, and April 2019 the Company amended its Term Loan with Oxford Finance to provide for additional periods of interest only for the months of February 2019, March 2019, and April 2019, respectively. The maturity date of the Term Loan remained unchanged. In May 2019, and concurrently with the Private Placement, the Company amended its Term Loan with Oxford Finance to provide a new twelve-month period of interest-only payments, commencing May 2019, and a two-year extension of its maturity date from June 2020 to May 2022. Upon the closing of the second tranche of the Company's Private Placement, the Company will receive an additional twelve-month period of interest-only payments, commencing May 2020.
- **RG-012 Transition to Sanofi:** In November 2018, the Company and Sanofi agreed to transition further development activities of the miR-21 programs, including the Company's RG-012 program, to Sanofi who will be responsible for all costs incurred in the development of these miR-21 programs (the "2018 Sanofi Amendment"). As of March 31, 2019, the transition activities, including the transfer of the investigational new drug application ("IND"), were substantially completed. The Company received \$2.5 million and \$1.8 million in upfront and material transfer-related payments in November 2018 and March 2019, respectively. In April 2019, the Company received an additional \$2.5 million upfront payment. These payments, totaling \$6.8 million, were recognized as revenue in the first quarter ended March 31, 2019. Regulus is also eligible to receive up to \$40 million in clinical milestone payments.
- **Lease Agreement:** In February 2019, the Company entered into an amendment of its lease (the "Lease Amendment") of 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 lease was accelerated from April 30, 2024 to March 31, 2019 and the lease terminated on April 1, 2019. Concurrently with the Lease Amendment, the Company entered into a new lease agreement (the "Lease") for 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. This relocation reduced the Company's facility size by approximately 60% and reduced its future contractual lease obligations by approximately 70%.

Program Updates

- **RGLS4326 for ADPKD:** In January 2019, the Company announced data from a planned interim analysis of a new mouse chronic toxicity study after 13 weeks of dosing in which no adverse or other significant

findings across the range of doses tested were shown. 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 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. The Company's engagement with FDA is ongoing.

- **RGLS5579 for Glioblastoma Multiforme (GBM):** In January 2019, the Company announced RGLS5579 as a clinical candidate for the treatment of GBM. RGLS5579, which targets microRNA-10b, demonstrated statistically significant improvements in survival as both a monotherapy as well as in combination with temozolomide ("TMZ") in an orthotopic GBM animal model. In combination with TMZ, the addition of a single dose of RGLS5579, delivered intracranially, led to a more than two-fold improvement in survival compared to TMZ alone. The Company plans to seek a partner to further advance RGLS5579 development.
- **Anti-miR-132 for Nonalcoholic Steatohepatitis (NASH):** In April 2019, Regulus presented a late breaker poster at the EASL International Liver Congress™ describing the development of its lead anti-miR-132 for the treatment of NASH. Across multiple animal models of NASH, the lead candidate demonstrated improvement in key endpoints, including NAFLD Activity Score (NAS), liver transaminases, hyperglycemia, and disease-related gene expression. In the diet-induced NASH mouse model (Amylin model) after two to four weekly doses, early onset of improvement across multiple disease parameters including liver triglycerides and blood levels of transaminases was observed. After nine weeks of treatment, there was evidence of sustained benefit with significant improvement of liver fibrosis and hyperglycemia compared to control-treated animals. The Company believes that targeting dysregulated microRNA in a complex disease like NASH may offer a unique mechanism of action from other programs in development. The Company plans to seek a partner to further advance the development of this program.

First Quarter 2019 Financial Results

Revenue: Revenue was \$6.8 million for the three months ended March 31, 2019, compared to less than \$0.1 million for the three months ended March 31, 2018. The increase was associated with revenue recognition of the upfront payments received under the 2018 Sanofi Amendment related to the transfer of RG-012 during the three months ended March 31, 2019.

Cash Position: As of March 31, 2019, Regulus had \$10.3 million in cash and cash equivalents.

Research and Development (R&D) Expenses: R&D expenses were \$6.0 million for the three months ended March 31, 2019, compared to \$11.8 million for the same period in 2018. The aggregate decrease was driven by a \$3.8 million decrease in external development expenses during the three months ended March 31, 2019, primarily attributable to the pausing of the RGLS4326 program in the third quarter of 2018 and commencement of the transfer of the RG-012 program to Sanofi under the 2018 Sanofi Amendment in the fourth quarter of 2018. In addition, personnel and internal expenses decreased by approximately \$1.9 million, primarily as a result of a reduction in headcount and related costs subsequent to our corporate restructuring in the third quarter of 2018.

General and Administrative (G&A) Expenses: G&A expenses were \$3.5 million for the three months ended March 31, 2019 compared to \$3.8 million for the same period in 2018. These amounts reflect personnel-related and ongoing general business operating costs.

Net Loss: Net loss was \$3.3 million, or \$0.31 per share (basic and diluted), for the three months ended March 31, 2019, compared to \$16.0 million, or \$1.85 per share (basic and diluted), for the same period 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. It is estimated that 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, 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), Regulus' sales of securities, including timing, size and completion of the Milestone Closing, its estimated cash runway and anticipated cost savings associated with its planned reduction in workforce, 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 March 31,	
	2019	2018
Revenues:		
Revenue under strategic alliances	\$ 6,778	\$ 18
Operating expenses:		
Research and development	5,983	11,828
General and administrative	3,533	3,773
Total operating expenses	9,516	15,601

Loss from operations	(2,738)	(15,583)
Other expense, net	(522)	(441)
Loss before income taxes	(3,260)	(16,024)
Income tax benefit	-	(1)
Net loss	\$ (3,260)	\$ (16,025)

Net loss per share, basic and diluted	\$ (0.31)	\$ (1.85)
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Weighted average shares used to compute basic and diluted net loss per share:	10,379,830	8,668,695
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	March 31, 2019	December 31, 2018
	(Unaudited)	
Cash and cash equivalents	\$ 10,320	\$ 13,935
Total assets	19,877	27,927
Term loan, less debt issuance costs	15,225	16,575
Stockholders' deficit	(6,069)	(5,854)

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<https://ir.regulusrx.com/2019-05-09-Regulus-Therapeutics-Reports-First-Quarter-2019-Financial-Results-and-Recent-Updates>