

## Regulus Therapeutics Reports First Quarter 2020 Financial Results and Recent Updates

*Initiated Third and Final Cohort of the Multiple Ascending Dose Study of RGLS4326 for the Treatment of Autosomal Dominant Polycystic Kidney Disease*

LA JOLLA, Calif., May 14, 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 first quarter ended March 31, 2020 and provided a corporate update.

"We have completed the second cohort and have initiated the dosing for the third and final cohort in the Phase 1 multiple ascending dose ("MAD") clinical study for RGLS4326" said Jay Hagan, CEO of Regulus. "Additionally, preparations for the Phase 1b study in patients with autosomal dominant polycystic kidney disease ("ADPKD") are well underway with plans to initiate in the second half of 2020."

### Program Highlights

**Initiated Dosing of the Third and Final Cohort in RGLS4326 Phase 1 for ADPKD:** In April 2020, the Company initiated dosing of the third and final 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 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.

### Financial Results

**Cash Position:** As of March 31, 2020, Regulus had \$28.1 million in cash and cash equivalents.

**Research and Development (R&D) Expenses:** R&D expenses were \$3.1 million for the three months ended March 31, 2020, compared to \$6.0 million for the same period in 2019. The aggregate decrease was driven by a \$1.5 million reduction in personnel and internal expenses and a \$1.0 million reduction in external development expenses, both of which were primarily attributable to a reduction in costs associated with the partial clinical hold of the RGLS4326 MAD study. In December 2019, the U.S. Food and Drug Administration ("FDA") lifted the partial clinical hold on the MAD study and it was recommenced in February 2020.

**General and Administrative (G&A) Expenses:** G&A expenses were \$2.4 million for the three months ended March 31, 2020 compared to \$3.5 million for the same period in 2019. These amounts reflect personnel-related and ongoing general business operating costs. The decrease is primarily attributable to continued cost reduction efforts subsequent to our corporate restructuring in the third quarter of 2018.

**Revenue:** Revenue was less than \$0.1 million for the three months ended March 31, 2020. Revenue was \$6.8 million for the three months ended March 31, 2019, 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 \$5.9 million, or \$0.25 per share (basic and diluted), for the three months ended March 31, 2020, compared to \$3.3 million, or \$0.31 per share (basic and diluted), for the same period in 2019.

### 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 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.

## 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. In addition, while Regulus expects the COVID-19 pandemic to adversely affect its business operations and financial results, the extent of the impact on Regulus' ability to achieve its preclinical and clinical development objectives and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. 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 March 31,	
	2020	2019
Revenues:		
Revenue under strategic alliances	\$ 6	\$ 6,778
Operating expenses:		
Research and development	3,119	5,983

General and administrative	2,422	3,533
Total operating expenses	5,541	9,516
Loss from operations	(5,535)	(2,738)
Other expense, net	(410)	(522)
Loss before income taxes	(5,945)	(3,260)
Income tax benefit	8	-
Net loss	\$ (5,937)	\$ (3,260)
Net loss per share, basic and diluted	\$ (0.25)	\$ (0.31)
Weighted average shares used to compute basic and diluted net loss per share:	24,064,373	10,379,830

	March 31, 2020	December 31, 2019
Cash and cash equivalents	\$ 28,052	\$ 34,121
Total assets	35,006	42,081
Term loan, less debt issuance costs	14,636	14,631
Stockholders' equity (deficit)	14,902	20,015

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