

Regulus Reports Fourth Quarter 2016 Financial Results and Pipeline Update

*RG-012 development program to include a separate biopsy study, which may provide earlier POC
Announces final top-line 48-week results for RG-101-02 DAA combo study
Conference call today at 5:00 p.m. ET*

LA JOLLA, Calif., March 2, 2017 /[PRNewswire](#)/ -- [Regulus Therapeutics Inc.](#) (Nasdaq: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today reported financial results for the fourth quarter and full year ended December 31, 2016 and provided a summary of corporate highlights and pipeline update.

"2016 was a year of accomplishments and important key learnings that position us for meaningful advancements in our clinical and pre-clinical programs in 2017 and beyond. Based on these learnings, I am even more convinced that targeting microRNAs offer significant advancements in therapeutic potential for many diseases for which there are currently no or limited treatment options," said Paul Grint, M.D., President and Chief Executive Officer of Regulus.

Fourth Quarter 2016 Highlights

- **Initiated a Phase I MAD study for RG-012:** Data from this study in healthy volunteers will provide additional PK and safety data and will be available in the second quarter 2017. The Phase II HERA study is expected to resume in the second half of 2017 following completion of the MAD study.
- **Announced two new clinical candidates at the Company's first R&D Day:** RGLS5040 (anti-miR-17) for the treatment of cholestatic diseases; and RGLS4326 (anti-miR-27) for the treatment of autosomal dominant polycystic kidney disease (ADPKD). IND-enabling activities are currently underway. Regulus expects to file the INDs in the second half of 2017.
- **Attracted Key Talent:** Dr. Timothy Wright joined Regulus in October as its Chief R&D Officer.

Pipeline Update

RG-012 for treatment in patients with Alport syndrome

- Today, Regulus announced that it has modified the Phase II RG-012 clinical development program in patients with Alport syndrome to accelerate patient enrollment and potentially achieve early proof of mechanism/proof of concept. The Phase II program will include the HERA study, a randomized, double-blind, placebo-controlled 48-week study in 30 patients with rapid renal function decline. The objective of the HERA study will be to evaluate the safety and efficacy of RG-012 for the treatment of renal function decline in patients with Alport syndrome. In parallel, a separate Phase I/II renal biopsy study in Alport patients will be initiated to evaluate RG-012 tissue PK, target engagement of miR-21, and effects on downstream genomic markers of disease. Both studies are anticipated to begin enrollment following the completion of the Phase I MAD study in Q2 2017. Based on projected enrollment rates, data from the renal biopsy study are expected in Q4 2017 and interim data from HERA are expected in mid-2018.

RG-101 for the treatment of HCV

- Today, Regulus reported top-line results from the primary endpoint analysis of its completed Phase II study of RG-101 in combination with 4 weeks of once/daily approved anti-viral agents Harvoni[®], Olysio[®], or Daklinza[™]. The study enrolled 79 treatment naïve genotype 1 and 4 HCV patients (Harvoni[®] combination arm, n=27, Olysio[®] combination arm, n=27, Daklinza[™] combination arm, n=25). The results from this final analysis demonstrated significant virologic response through 48 weeks of follow-up: RG-101 plus Harvoni demonstrated 100% SVR48; RG-101 plus Olysio demonstrated 77% SVR48; and RG-101 plus Daklinza[™] demonstrated 84% SVR48. There were 10 relapses: six in the Olysio[®] combination arm (weeks 8, 20, 24 and 32); and four in the Daklinza[™] combination arm (weeks 8, 12 and 24). RG-101 in combination with four weeks of oral DAA therapy was generally well tolerated with the majority of adverse events (AEs) considered mild or moderate with no AE-related discontinuations. Commonly reported AEs included fatigue, headache, and injection site reactions. There were four patients across all arms that experienced adverse events of asymptomatic transient hyperbilirubinemia (greater than or equal to two times the upper limit of normal). No cases met criteria for Hy's law. Over the course of the one-year study, five subjects reported serious adverse events (SAE). As previously reported, there was a single SAE of jaundice in a patient at seven weeks in the Daklinza[™] combination arm of the study. Since the interim analysis in June 2016, there were two additional SAEs: one was a trauma-related knee injury; and the other was an upper respiratory infection.
- In January, the Company reported that it received written communication from the FDA that the clinical development program from RG-101 remains on clinical hold. The FDA has requested the final safety and efficacy data from all on-going RG-101 pre-clinical and clinical studies before reconsidering the clinical hold. These data are anticipated to be available in Q4 2017.

RGLS5040 for the treatment of cholestatic diseases

- IND-enabling activities are currently underway, and an IND filing is currently on track for the second half of 2017.

RGLS4326 for the treatment of ADPKD

- IND-enabling activities are currently underway, and an IND filing is currently on track for the second half of 2017.

Financial Results

Cash Position: Cash, cash equivalents and short-term investments were \$76.1 million at December 31, 2016, compared with \$115.3 million at December 31, 2015.

Research and Development (R&D) Expenses: R&D expenses were \$15.0 million and \$64.3 million for the quarter and year ended December 31, 2016, respectively, compared to \$12.8 million and \$56.4 million for the same periods in 2015. The increases in R&D expenses were primarily driven by the advancement of our clinical programs and increased investment in our preclinical pipeline.

General and Administrative (G&A) Expenses: G&A expenses were \$4.8 million and \$18.4 million for the quarter and year ended December 31, 2016, respectively, compared to \$5.4 million and \$19.1 million for the same periods in 2015. The decreases in G&A expenses were principally driven by a decrease in personnel costs, including non-cash stock-based compensation.

Revenue: Revenue was less than \$0.1 million and \$1.2 million for the quarter and year ended December 31, 2016, respectively, compared to \$10.9 million and \$20.8 million for the same periods in 2015.

Net Loss: Net loss was \$20.0 million, or \$0.38 per share, and \$81.8 million, or \$1.55 per share, for the quarter and year ended December 31, 2016, respectively, compared to a net loss of \$7.2 million, or \$0.14 per share, and \$55.7 million, or \$1.08 per share, for the same periods in 2015.

Conference Call Details

Regulus will host a conference call and webcast at 5:00 p.m. Eastern Time today to discuss third 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 75961316. To access the replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 75961316. 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 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, both independently and with our strategic alliance partners, Sanofi and AstraZeneca. 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-101, RG-012, RGLS426 and RGLS456070), 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

Statement of Operations

(In thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2016	2015	2016	2015
Revenues:				
Revenue under strategic alliances	\$ 18	\$ 10,860	\$ 1,194	\$ 20,759
Operating expenses:				
Research and development	14,979	12,794	64,305	56,387
General and administrative	4,783	5,427	18,391	19,130
Total operating expenses	19,762	18,221	82,696	75,517
Loss from operations	(19,744)	(7,361)	(81,502)	(54,758)
Other (expense) income, net	(271)	139	(338)	(1,008)
Loss before income taxes	(20,015)	(7,222)	(81,840)	(55,766)
Income tax (expense) benefit	(5)	(4)	4	18
Net loss	\$ (20,020)	\$ (7,226)	\$ (81,836)	\$ (55,748)
Net loss per share, basic and diluted	\$ (0.38)	\$ (0.14)	\$ (1.55)	\$ (1.08)
Weighted average shares used to compute basic and diluted net loss per share:	52,923,713	52,477,489	52,813,474	51,411,353

Regulus Therapeutics Inc.

Balance Sheets

(In thousands)

	December 31, 2016	December 31, 2015
Assets		
Cash, cash equivalents, short-term investments and restricted cash	\$ 76,111	\$ 115,319
Other current assets	11,363	18,939
Non-current assets	13,187	6,825
Total assets	\$ 100,661	\$ 141,083
Liabilities and stockholders' equity		
Current liabilities	\$ 13,735	\$ 11,438
Total deferred revenue	2,065	3,259
Term Loan, less debt issuance costs	19,802	—
Other long-term liabilities	8,984	2,308
Stockholders' equity	56,075	124,078
Total liabilities and stockholders' equity	\$ 100,661	\$ 141,083

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/regulus-reports-fourth-quarter-2016-financial-results-and-pipeline-update-300417255.html>

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Additional assets available online: [Photos \(1\)](#)

<https://ir.regulusrx.com/2017-03-02-Regulus-Reports-Fourth-Quarter-2016-Financial-Results-and-Pipeline-Update>