

Regulus Reports Third Quarter 2016 Financial Results

Conference Call Today at 5:00 p.m. ET

LA JOLLA, Calif., Nov. 1, 2016 /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 three and nine months ended September 30, 2016 and provided a summary of corporate highlights.

"Our focus in the third quarter included the advancement of the RG-012 development program and addressing the clinical hold for RG-101 while expanding the pre-clinical pipeline," said Paul Grint, M.D., President and Chief Executive Officer of Regulus. "We continue to be excited with the progress of our research programs, and look forward to sharing an update at our first R&D day on December 6th."

Financial Results

Revenue: Revenue was \$0.2 million and \$1.2 million for the three and nine months ended September 30, 2016, respectively, compared to \$1.9 million and \$9.9 million for the same periods in 2015. Revenue for the three and nine months ended September 30, 2016 and 2015 consisted of amortization of up-front payments from Regulus' strategic alliances and collaborations. Revenue for the three and nine months ended September 30, 2015 included \$0.9 million and \$4.1 million, respectively, for research services under Regulus' strategic alliances and collaborations. Preclinical milestones earned under Regulus' strategic alliances and collaborations were \$0.3 million and \$3.2 million for the three and nine months ended September 30, 2015, respectively.

Research and Development (R&D) Expenses: R&D expenses were \$14.6 million and \$49.3 million for the three and nine months ended September 30, 2016, respectively, compared to \$11.0 million and \$43.6 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 \$13.6 million for the three and nine months ended September 30, 2016, respectively, compared to \$4.2 million and \$13.7 million for the same periods in 2015.

Net Loss: Net loss was \$19.5 million, or \$0.37 per share, and \$61.8 million, or \$1.17 per share, for the three and nine months ended September 30, 2016, respectively, compared to a net loss of \$13.0 million, or \$0.25 per share, and \$48.5 million, or \$0.95 per share, for the same periods in 2015.

Cash Position: Cash, cash equivalents, and short-term investments were \$91.7 million at September 30, 2016, compared with \$108.0 million at June 30, 2016 and \$115.3 million at December 31, 2015.

Recent Events

- In October, Dr. Timothy Wright joined Regulus as its Chief R&D Officer.
- In September, Regulus initiated the HERA study, an international randomized, double-blind, placebo-controlled, multi-center Phase 2 clinical trial designed to evaluate the safety, pharmacodynamics, pharmacokinetics, dose selection, and preliminary efficacy of weekly RG-012 injections in approximately 30 patients with Alport syndrome. In order to address study design comments from European regulators, a multiple-ascending dose (MAD) study in healthy volunteers will be implemented (4-week repeat dosing) prior to expanding to Alport patients. Regulus anticipates the MAD study will be completed in the first half of 2017. Based on predicted enrollment rates, Regulus anticipates interim results from HERA in the first half of 2018.
- In July, as anticipated, Regulus received written communication from the U.S. Food and Drug Administration (FDA) outlining information required to resolve the clinical hold for its Investigational New Drug (IND) for RG-101, which was announced on June 27, 2016. Based on the completion of additional mechanistic pre-clinical studies, Regulus expects a response to its submission from the FDA in the first quarter of 2017.

Upcoming Events

- On November 13, 2016, Regulus will present three posters at American Association for the Study of Liver Disease (AASLD) in Boston.
- On November 15, 2016 at 4:30 pm Eastern Time, Regulus will present a corporate overview at the Stifel 2016 Healthcare Conference in New York.
- On November 18 and 19, 2016, Regulus will present two posters at American Society of Nephrology (ASN) Kidney Week in Chicago.
- On December 6, 2016, Regulus will host its first R&D Day.
- On December 13, 2016, Regulus will participate in the 4th Annual Boston Healthcare Conference.
- On December 14, 2016, Regulus will participate in the BMO Capital Markets Prescriptions for Success Healthcare Conference.

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 99499252. To access the replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 99499252. 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 maturing microMarkersSM biomarkers platform and a rich intellectual property estate to retain its leadership in the microRNA field. Regulus is developing RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection, and RG-012, an anti-miR targeting microRNA-21 for the treatment of Alport syndrome, a life-threatening kidney disease driven by genetic mutations with no approved therapy. In addition, RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of NASH in patients with type 2 diabetes/pre-diabetes, has entered Phase I/IIa clinical development through its strategic alliance with AstraZeneca. Regulus is also advancing several programs toward clinical development in renal, hepatic and central nervous systems diseases. Regulus' commitment to innovation has resulted in multiple peer-reviewed publications in notable scientific journals and has resulted in the formation of strategic alliances with AstraZeneca and Sanofi. 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), 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
September 30,

Nine months ended
September 30,

2016

2015

2016

2015

(Unaudited)

Revenues:								
Revenue under strategic alliances	\$	204	\$	1,865	\$	1,176	\$	9,899
Operating expenses:								
Research and development		14,554		10,965		49,326		43,593
General and administrative		4,842		4,245		13,609		13,703
Total operating expenses		19,396		15,210		62,395		57,296
Loss from operations		(19,192)		(13,345)		(61,759)		(47,397)
Other (expense) income, net		(323)		329		(66)		(1,147)
Loss before income taxes		(19,515)		(13,016)		(61,825)		(48,544)
Income tax (expense) benefit		(4)		16		9		22
Net loss	\$	(19,519)	\$	(13,000)	\$	(61,816)	\$	(48,522)
Net loss per share, basic and diluted								
	\$	(0.37)	\$	(0.25)	\$	(1.17)	\$	(0.95)
Weighted average shares used to compute basic and diluted net loss per share:		52,835,414		51,990,460		52,776,459		51,052,068

**Regulus Therapeutics Inc.
Condensed Balance Sheets
(In thousands)**

	September 30, 2016	December 31, 2015
	(Unaudited)	
Assets		
Cash, cash equivalents, short-term investments and restricted cash	\$ 91,663	\$ 115,319
Other current assets	11,167	18,939
Non-current assets	13,436	6,825
Total assets	\$ 116,266	\$ 141,083
Liabilities and stockholders' equity		
Current liabilities	\$ 13,080	\$ 11,438
Total deferred revenue	2,083	3,259

Term Loan, less debt issuance costs	19,787	—
Other long-term liabilities	8,631	2,308
Stockholders' equity	72,685	124,078
Total liabilities and stockholders' equity	\$ 116,266	\$ 141,083

Logo - <http://photos.prnewswire.com/prnh/20160519/370141LOGO>

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/regulus-reports-third-quarter-2016-financial-results-300355307.html>

SOURCE Regulus Therapeutics Inc.

For further information: Investor Relations Contact: Allison Wey, 858-202-6321, away@regulusrx.com

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

<https://ir.regulusrx.com/2016-11-01-Regulus-Reports-Third-Quarter-2016-Financial-Results>