

## Regulus Reports Third Quarter 2014 Financial Results and Recent Highlights

*- Demonstrated Human Proof-of-Concept; 'Clinical Map Initiative' Updated -  
- Strengthened Financial Position with Public Offering of Common Stock; Guidance Updated to End 2014 with Greater Than \$150 Million in Cash -*

LA JOLLA, Calif., Nov. 5, 2014 /PRNewswire/ -- [Regulus Therapeutics Inc.](#) (NASDAQ:RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today reported financial results and highlights for the quarter ended September 30, 2014 and provided a summary of recent corporate highlights.

"The third quarter and recent period have been largely focused on obtaining our first human proof-of-concept results from our ongoing study of RG-101, our wholly-owned GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of HCV. The previously reported interim results are very impressive, and as a result, we have updated our 'Clinical Map Initiative' goals to rapidly advance RG-101 into a robust Phase II combination study," said Kleantis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "In addition, we were pleased to have recently strengthened our balance sheet with a successful public offering to further enable our 'Clinical Map Initiative' goals and to support the continued growth of our microRNA therapeutics pipeline."

Paul Grint, M.D., Chief Medical Officer of Regulus, added, "Our recent scientific accomplishments have positioned Regulus for an exciting period focused on the advancement of our clinical portfolio. We are preparing to file an investigational new drug application for both RG-101 and RG-012, an anti-miR targeting microRNA-21 for the treatment of renal dysfunction in Alport syndrome patients, and expect to initiate further clinical studies in both programs in the first half of 2015. Over the next several quarters, we also expect to advance the rest of our programs toward the clinic."

### Recent Highlights

- **Human Proof-of-Concept Achieved with a microRNA Therapeutic.** Regulus reported interim results from an ongoing clinical study demonstrating that treatment with a single subcutaneous dose of 2 mg/kg of RG-101 as monotherapy resulted in significant and sustained reductions in HCV RNA in a varied group of patients (10 GT1s, 5 GT3s and 1 GT4; 14 patients received RG-101, 8 naïve, 6 patients who experienced viral relapse after a prior IFN-containing regimen, 2 patients received placebo). In the 14 treated HCV patients, there was a mean viral load reduction of 4.1 log<sub>10</sub> at day 29 (range -5.8 log<sub>10</sub> to -2.3 log<sub>10</sub>). 6 out of 14 patients had HCV RNA levels below the limit of quantification at day 29 and the 3 patients from this group who have reached day 57 still have HCV RNA levels below the limit of quantification. Regulus is preparing to file an investigational new drug application for RG-101 and is completing the ongoing clinical study. Under the 'Clinical Map Initiative', Regulus expects to report additional results from the ongoing study and initiate a Phase II combination study of RG-101 in HCV patients in the second quarter of 2015.
- **Completed Public Offering of Common Stock; Updated Year-End Cash Guidance.** On November 3, 2014, Regulus completed an underwritten public offering of \$103.5 million of shares of its common stock, including \$13.5 million of shares sold pursuant to the full exercise of the underwriters' option to purchase additional shares, at a price to the public of \$17.00 per share. Of the total 6,088,235 shares sold in the offering, 4,808,824 shares were sold by Regulus and 1,279,411 shares were sold by an existing stockholder of Regulus, Isis Pharmaceuticals, Inc. The net proceeds to Regulus from the offering are expected to be approximately \$76.1 million after deducting underwriting discounts, commissions and other estimated offering expenses payable by Regulus. Regulus has updated its year-end cash guidance to end 2014 with greater than \$150.0 million in cash, cash equivalents and short-term investments.
- **Product Development and Commercialization Agreement with GlaxoSmithKline ("GSK") to Terminate.** The product development and commercialization agreement between Regulus and GSK will be terminated in light of GSK's review of their overall research priorities. Regulus continues to maintain full ownership of RG-101 and all rights in other collaboration targets and compounds have returned to Regulus. The effective date of termination will be January 15, 2015.
- **Presented New Preclinical Data at Oligonucleotide Therapeutics Society ("OTS") Meeting.** Regulus presented new preclinical data at OTS on its programs targeting microRNA-103/107 for metabolic disorders and targeting microRNA-21 for the treatment of renal dysfunction in Alport syndrome patients.

### Third Quarter 2014 Highlights

- **Advanced RG-012 for the Treatment of Renal Dysfunction in Alport Syndrome Patients.** Regulus initiated its ATHENA natural history of disease study to gather information about the changes in renal function over time in patients with Alport syndrome, a life-threatening genetic kidney disease with no approved therapy. Data from the ATHENA study will provide the clinical basis for the design of a Phase II

study to monitor the therapeutic effect of RG-012 on the decline in renal function and time to end-stage renal disease in Alport syndrome patients. Regulus is currently enrolling patients in ATHENA and preparing to file an investigational new drug application for RG-012. Under the 'Clinical Map Initiative', Regulus expects to initiate a Phase I study in the first half of 2015 and a Phase II proof-of-concept study thereafter. Additionally, RG-012 has recently been granted orphan drug designation from the U.S. FDA as a therapeutic for the treatment of Alport syndrome.

- **Expanded Research with Biogen Idec on microRNA Biomarkers.** Under its Regulus microMarkers<sup>SM</sup> division, Regulus entered into a new collaboration agreement with Biogen Idec to profile a large number of whole blood samples from a cohort of multiple sclerosis ("MS") patients who have been treated with a Biogen Idec MS therapy to identify potential microRNA signatures. Regulus received \$2.0 million upfront and is eligible for future payments upon achievement of certain milestones related to the identification of potential microRNA signatures.

### **Third Quarter 2014 Financial Results & Highlights**

Regulus reported a net loss of \$9.8 million and \$34.5 million for the three and nine months ended September 30, 2014, respectively, compared to a net loss of \$2.2 million and \$16.7 million for the same periods in 2013. Basic and diluted net loss per share was \$0.23 and \$0.26 for the three months ended September 30, 2014, respectively, compared to basic and diluted net loss per share of \$0.05 and \$0.07 for the same period in 2013. Basic and diluted net loss per share was \$0.80 for the nine months ended September 30, 2014, compared to \$0.45 for the same period in 2013.

Regulus recognized revenue of \$1.1 million and \$3.5 million for the three and nine months ended September 30, 2014, respectively, compared to \$6.1 million and \$14.1 million for the same periods in 2013. Revenue during these periods consisted primarily of amortization of up-front payments received from our strategic alliances and collaborations, which is recognized over the estimated period of performance.

Research and development expenses were \$10.2 million and \$30.6 million for the three and nine months ended September 30, 2014, respectively, compared to \$7.1 million and \$21.7 million for the same periods in 2013. This increase was primarily driven by the initiation of a clinical study of RG-101, initiation of the ATHENA natural history of disease study in Alport syndrome patients, IND-enabling costs for RG-012 and the continued advancement of other preclinical development programs.

General and administrative expenses were \$2.6 million and \$8.3 million for the three and nine months ended September 30, 2014, respectively, compared to \$1.9 million and \$5.5 million for the same periods in 2013. This increase was primarily driven by an increase in salaries and related employee costs and other operating expenses associated with general business activities.

Net loss for the three and nine months ended September 30, 2014 included a non-cash gain of \$1.8 million and \$0.6 million, respectively, from the change in value of a convertible note payable, compared to a non-cash gain of \$0.7 million for the three months ended September 30, 2013 and a non-cash charge of \$3.8 million for the nine months ended September 30, 2013. Changes in value were primarily driven by Regulus' stock price changes during the respective periods.

As of September 30, 2014, Regulus had \$94.1 million in cash, cash equivalents and short-term investments, debt with a principal balance of \$5.4 million and 43,439,562 shares of common stock outstanding.

### **Conference Call & Webcast Information**

Regulus will host a conference call and webcast at 5:00 p.m. Eastern Standard Time today to discuss its third quarter 2014 financial results, recent company highlights and its expectations for the remainder of 2014. A live webcast of the call will be available online at [www.regulusrx.com](http://www.regulusrx.com). To access the call, please dial (877) 257-8599 (domestic) or (970) 315-0459 (international) and refer to conference ID 28936277. To access the telephone replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 28936277. The webcast and telephone replay will be archived on the company's website for ninety days following the call.

### **Update to the 'Clinical Map Initiative'**

Regulus' 'Clinical Map Initiative' outlines certain corporate goals to advance its microRNA therapeutics pipeline over the next several years. Under this initiative, Regulus is developing RG-101, a wholly-owned GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of HCV and RG-012, an anti-miR targeting microRNA-21 for the treatment of Alport syndrome, a life-threatening genetic kidney disease with no approved therapy, and expects to nominate a third candidate for clinical development in the first half of 2015. Regulus plans to rapidly advance RG-101 into a Phase II combination study in HCV patients, which is planned to commence in the second quarter of 2015. Regulus also plans to initiate a Phase I clinical study of RG-012 in the first half of 2015 and a Phase II proof-of-concept study in Alport syndrome patients thereafter.

## About Regulus

Regulus Therapeutics Inc. (*NASDAQ:RGLS*) is a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus is uniquely positioned to leverage a mature therapeutic platform that harnesses the oligonucleotide drug discovery and development expertise of Alnylam Pharmaceuticals, Inc. and Isis Pharmaceuticals, Inc., which founded the company. Regulus has a well-balanced microRNA therapeutics pipeline entering clinical development, an emerging microRNA biomarkers platform to support its therapeutic programs, 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. Regulus is also advancing several programs toward clinical development in oncology, fibrosis and metabolic diseases. Regulus' commitment to innovation and its leadership in the microRNA field have enabled the formation of strategic alliances with AstraZeneca and Sanofi and a research collaboration with Biogen Idec focused on microRNA biomarkers. In addition, the company has established Regulus microMarkers<sup>SM</sup>, a division focused on identifying microRNAs as biomarkers of human disease, which is designed to support its therapeutic pipeline, collaborators and strategic partners.

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 financial estimates (including Regulus' projected cash at the end of 2014), the projected sufficiency of Regulus' capital position for future periods, 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 and RG-012 and with respect to the nomination of a third microRNA candidate for clinical development), the projected timeline of clinical development activities, and expectations regarding future therapeutic and commercial potential of Regulus' business plans (including Regulus' expected future activities in 2014), 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,	
2014	2013	2014	2013

(Unaudited)

Revenues:					
Revenue under strategic alliances	\$	1,083	\$	6,118	\$ 3,450 \$ 14,115
Total revenues		1,083		6,118	3,450 14,115
Operating expenses:					
Research and development		10,173		7,106	30,572 21,710
General and administrative		2,569		1,917	8,255 5,545
Total operating expenses		12,742		9,023	38,827 27,255
Loss from operations		(11,659)		(2,905)	(35,377) (13,140)
Other income (expense), net		1,861		736	866 (3,605)
Loss before income taxes		(9,798)		(2,169)	(34,511) (16,745)
Income tax (benefit) expense		—		(5)	1 (4)
Net loss	\$	(9,798)	\$	(2,164)	\$ (34,512) \$ (16,741)
Net loss per share:					
Basic	\$	(0.23)	\$	(0.05)	\$ (0.80) \$ (0.45)
Diluted	\$	(0.26)	\$	(0.07)	\$ (0.80) \$ (0.45)
Weighted average shares used to compute net loss per share:					
Basic		43,406,251		40,154,812	43,155,601 37,367,368
Diluted		44,855,463		41,555,660	43,155,601 37,367,368

## Regulus Therapeutics Inc.

### Condensed Balance Sheets

(In thousands)

September 30, 2014 December 31, 2013

(Unaudited)

#### Assets

Cash, cash equivalents and short-term investments	\$	94,065	\$	114,005
Other current assets		3,958		3,177
Non-current assets		5,992		5,883

Total assets	\$	104,015	\$	123,065
<b>Liabilities and stockholders' equity</b>				
Current liabilities	\$	7,719	\$	5,482
Total deferred revenue		10,409		11,388
Convertible notes payable, at fair value		10,665		11,279
Other long-term liabilities		1,142		1,459
Stockholders' equity		74,080		93,457
Total liabilities and stockholders' equity	\$	104,015	\$	123,065

SOURCE Regulus Therapeutics Inc.

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<https://ir.regulusrx.com/2014-11-05-Regulus-Reports-Third-Quarter-2014-Financial-Results-and-Recent-Highlights>