

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

- 'Clinical Map Initiative' On Track; Human Proof of Concept Results for RG-101 and Nomination of Third Clinical Candidate Expected by YE 2014 -

- Maintained Strong Financial Position Ending Q2 2014 with \$103.5 Million in Cash -

LA JOLLA, Calif., Aug. 6, 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 June 30, 2014 and provided a summary of recent corporate highlights.

"The first half of 2014 has been a period of steady progress for us as represented by the growth of our clinical and preclinical portfolios under our 'Clinical Map Initiative', the expansion of our strategic relationships and our consistently strong financial position, all which support our leadership position in the microRNA therapeutics field," said Kleanthis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "Recently, we were pleased to report additional progress on the business front with the expansion of our relationship with Biogen Idec to identify microRNA biomarkers for multiple sclerosis. The Regulus microMarkers™ division continues to mature and we hope to leverage our innovative technology platform to further advance our microRNA therapeutic pipeline, our collaborations and partners' programs in a meaningful way."

Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus, added, "We are very pleased with our recent scientific progress and hope to extend our good track record into the second half of 2014. We have several significant catalysts on the horizon - by the end of the year, we have the opportunity to report our first human proof of concept results with a microRNA therapeutic, strengthen our focus on orphan diseases by advancing RG-012 toward the clinic, and expand our overall microRNA therapeutics pipeline with the nomination of a third candidate for clinical development."

### Recent Highlights

- **Expanded microRNA Biomarker Research with Biogen Idec; Regulus to Receive \$2M Upfront Payment.** Regulus entered into a new collaboration agreement with Biogen Idec to expand its research focused on identifying microRNAs as biomarkers for multiple sclerosis ("MS") under its Regulus microMarkers™ division. The new research will focus on profiling a large number of whole blood samples from a cohort of MS patients who have been treated with a Biogen Idec MS therapy to identify potential microRNA signatures. Regulus will receive \$2.0 million upfront and is eligible for future payments upon achievement of certain milestones related to the identification of potential microRNA signatures. Regulus and Biogen Idec began their collaboration in August 2012 to identify potential microRNA biomarkers in MS. The results of this previous work helped define the scope of the research under the new collaboration agreement, which resulted in a termination of the August 2012 agreement.
- **Orphan Designation Granted to RG-012 from the U.S. Food and Drug Administration.** Recently, the U.S. Food & Drug Administration granted orphan drug designation to RG-012, an anti-miR targeting microRNA-21 ("miR-21"), as a therapeutic for the treatment of Alport syndrome, a life-threatening genetic kidney disease with no approved therapy. In the near term, Regulus expects to initiate a natural history of disease study to gather greater information about the progression of Alport syndrome and to inform future clinical development plans of RG-012. Under the 'Clinical Map Initiative', Regulus expects to initiate a Phase I clinical study of RG-012 in the first half of 2015.

### Second Quarter 2014 Highlights

- **Phase I Clinical Study of RG-101 for the Treatment of HCV Ongoing.** Regulus is evaluating RG-101, a GalNAc-conjugated anti-miR that targets microRNA-122 ("miR-122"), in a Phase I clinical study for the treatment of chronic hepatitis C virus infection ("HCV"). The study is being conducted in the Netherlands and has four parts: (i) a single ascending-dose study in healthy volunteer subjects; (ii) a multiple-ascending dose study in healthy volunteer subjects; (iii) a single-dose drug-drug interaction study of RG-101 in combination with an approved oral direct-acting antiviral ("DAA") in healthy volunteer subjects; and (iv) a single-dose study in HCV patients to assess the safety and viral load reduction. The primary objective of the study is to evaluate safety and tolerability and the secondary objectives are to evaluate pharmacokinetics, viral load reduction and any impact an oral DAA may have on the pharmacokinetics of RG-101. Up to 100 healthy volunteer subjects and HCV patients are planned to be enrolled in the Phase I study. Under the 'Clinical Map Initiative', Regulus expects to report human proof-of-concept results from Part IV of the Phase I study by the end of 2014.
- **Strengthened Leading IP Estate; Expanded Coverage on anti-miR-122 for HCV.** The U.S. Patent and Trademark Office granted a patent in Regulus' exclusively licensed 'Sarnow' patent estate

for claims related to targeting miR-122 for the treatment of HCV in combination with other anti-viral therapies.

- **Advanced Preclinical microRNA Portfolio, Independently and with Strategic Partners.** Regulus continued to advance several additional programs, both independently and with its strategic alliance partners, targeting miR-21 and microRNA-221 for the treatment of hepatocellular carcinoma, targeting microRNA-19 for oncology indications and targeting microRNA-103/107 for the treatment of metabolic diseases. Additionally, Regulus continued to pursue several undisclosed microRNA targets, namely for orphan disease and oncology indications, to expand its therapeutics pipeline. Under the 'Clinical Map Initiative', Regulus expects to nominate a third microRNA candidate for clinical development (either independently or with a strategic partner) by the end of 2014.
- **Attracted Key Talent.** Regulus appointed Paul C. Grint, M.D. as Chief Medical Officer. Dr. Grint is responsible for leading and expanding Regulus' microRNA clinical portfolio and is a key member of its executive management team.

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

Regulus reported a net loss of \$12.0 million and \$24.7 million for the three and six months ended June 30, 2014, respectively, compared to a net loss of \$7.3 million and \$14.6 million for the same periods in 2013. Basic and diluted net loss per share was \$0.28 and \$0.29 for the three months ended June 30, 2014, respectively, compared to basic and diluted net loss per share of \$0.20 for the same period in 2013. Basic and diluted net loss per share was \$0.57 for the six months ended June 30, 2014, compared to \$0.41 for the same period in 2013.

Regulus recognized revenue of \$0.7 million and \$2.4 million for the three and six months ended June 30, 2014, respectively, compared to \$4.8 million and \$8.0 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 \$10.8 million and \$20.4 million for the three and six months ended June 30, 2014, respectively, compared to \$7.7 million and \$14.6 million for the same periods in 2013. This increase was primarily driven by the initiation of a Phase I clinical study for RG-101, IND-enabling costs for RG-012 and the continued advancement of other preclinical development programs.

General and administrative expenses were \$3.0 million and \$5.7 million for the three and six months ended June 30, 2014, respectively, compared to \$1.7 million and \$3.6 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 in the three months ended June 30, 2014 included a non-cash gain of \$1.0 million from the change in value of a convertible note payable, compared to a non-cash charge of \$2.7 million for the same period in 2013. Non-cash charges recorded from changes in the value of a convertible note payable were \$1.2 million and \$4.5 million for the six months ended June 30, 2014 and 2013, respectively. Changes in value were primarily driven by our stock price changes during the respective periods.

As of June 30, 2014, Regulus had \$103.5 million in cash, cash equivalents and short-term investments, debt with a principal balance of \$5.4 million and 43,383,863 shares of common stock outstanding. Regulus expects to maintain its strong financial position and finish 2014 with at least \$75.0 million in cash, cash equivalents and short-term investments.

## **Conference Call & Webcast Information**

Regulus will host a conference call and webcast at 5:00 p.m. Eastern Daylight Time today to discuss its second 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 79690344. To access the telephone replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 79690344. The webcast and telephone replay will be archived on the company's website for ninety days following the call.

## **About the 'Clinical Map Initiative'**

Launched in February 2014, Regulus' 'Clinical Map Initiative' outlines certain corporate goals to advance its microRNA therapeutics pipeline over the next several years. Regulus expects to report human proof-of-concept results in the Phase I clinical study of RG-101 for the treatment of HCV by the end of 2014, initiate a Phase I clinical study of RG-012 for the treatment of Alport syndrome in the first half of 2015, nominate a third microRNA candidate for clinical development by the end of 2014, and maintain a strong financial position and

end 2014 with at least \$75.0 million in cash, cash equivalents and short-term investments.

## 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 intends to focus its proprietary efforts on developing microRNA therapeutics for oncology indications and orphan diseases and is currently advancing several programs toward clinical development in oncology, fibrosis and metabolic diseases. Specifically, Regulus is developing 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, and RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection. Regulus' commitment to innovation and its leadership in the microRNA field have enabled the formation of strategic alliances with AstraZeneca, GlaxoSmithKline and Sanofi and a research collaboration with Biogen Idec focused on microRNA biomarkers. In addition, the Company has established Regulus microMarkers™, 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-012 and RG-101 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 June 30,		Six months ended June 30,	
2014	2013	2014	2013

(Unaudited)

Revenues:

Revenue under strategic alliances and collaboration

\$ 736      \$ 4,759      \$ 2,367      \$ 7,997

Operating expenses:

Research and development

10,795      7,722      20,399      14,604

General and administrative

2,954      1,723      5,686      3,628

Total operating expenses

13,749      9,445      26,085      18,232

Loss from operations

(13,013)      (4,686)      (23,718)      (10,235)

Other income (expense), net

1,040      (2,652)      (995)      (4,341)

Loss before income taxes

(11,973)      (7,338)      (24,713)      (14,576)

Income tax expense

-      10      1      1

Net loss

\$ (11,973)      \$ (7,348)      \$ (24,714)      \$ (14,577)

Net loss per share:

Basic

\$ (0.28)      \$ (0.20)      \$ (0.57)      \$ (0.41)

Diluted

\$ (0.29)      \$ (0.20)      \$ (0.57)      \$ (0.41)

Weighted average shares used to compute net loss per share:

Basic

43,362,483      35,994,642      43,028,198      35,933,961

Diluted

44,799,536      35,994,642      43,028,198      35,933,961

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

**June 30,  
2014****December 31,  
2013**

(Unaudited)

**Assets**

Cash, cash equivalents and short-term investments	\$ 103,501	\$ 114,005
Other current assets	4,383	3,177
Non-current assets	6,037	5,883
Total assets	\$ 113,921	\$ 123,065
<b>Liabilities and stockholders' equity</b>		
Current liabilities	\$ 8,637	\$ 5,482
Total deferred revenue	9,491	11,388
Convertible notes payable, at fair value	12,450	11,279
Other long-term liabilities	1,260	1,459
Stockholders' equity	82,083	93,457
Total liabilities and stockholders' equity	\$ 113,921	\$ 123,065

SOURCE Regulus Therapeutics Inc.

For further information: Investor Relations Contact, Amy Conrad, Senior Director, Investor Relations and Corporate Communications, Regulus Therapeutics Inc., 858-202-6321, [aconrad@regulusrx.com](mailto:aconrad@regulusrx.com), Media Contact, Liz Bryan, Spectrum Science, [lbryan@spectrumscience.com](mailto:lbryan@spectrumscience.com), 202-955-6222 x2526

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<https://ir.regulusrx.com/2014-08-06-Regulus-Reports-Second-Quarter-2014-Financial-Results-and-Recent-Highlights>