



May 14, 2013

Regulus Provides Update on 'Road to the Clinic' Strategy and Reports First Quarter 2013 Financial Results and Recent Highlights

- Nominated RG-101 in HCV as First microRNA Candidate for Clinical Development-**
- Maintained Strong Financial Position with Over \$90 million in Cash-**

LA JOLLA, Calif., May 14, 2013 /PRNewswire/ -- [Regulus Therapeutics Inc.](http://www.regulustherapeutics.com) (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today provided an update on its microRNA therapeutic pipeline under the company's 'Road to the Clinic' strategy and reported financial results for the quarter ended March 31, 2013.

Regulus Continues to Execute on 'Road to the Clinic' Strategy: *Nominated RG-101 as First microRNA Candidate for Clinical Development*

- Regulus announced today that it has nominated its first microRNA candidate for clinical development, RG-101, a GalNAc-conjugated microRNA antagonist or anti-miR, which targets microRNA-122 (miR-122) for the treatment of patients with chronic hepatitis C virus (HCV) infection. Regulus is performing additional pre-clinical studies and finalizing development plans for RG-101 in HCV and expects to submit an application with regulatory authorities in early 2014.
- Regulus plans to develop RG-101 independently of its strategic alliance with GlaxoSmithKline (GSK). The broad strategic alliance between Regulus and GSK remains intact and GSK retains its interest in the miR-122 program in HCV, as miR-122 will remain a Collaboration Target under the alliance. As such, the companies are in the process of amending the Product Development and Commercialization Agreement to clarify that RG-101 is fully owned by Regulus.

"The nomination of RG-101 as our first microRNA candidate for clinical development marks a tremendous milestone for Regulus and represents significant achievement of our initial goals under our 'Road to the Clinic' strategy," said Kleantis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "We continue to focus our efforts on preparing for a successful transition to a clinical-stage company and remain on track to nominate our second microRNA candidate for clinical development by the end of the year. Our recent progress is encouraging and we look forward to realizing the potential that our microRNA technology may have in transforming the field of drug discovery."

Neil W. Gibson, Ph.D., Chief Scientific Officer, added, "We believe that RG-101 is a novel, pan-genotypic agent that may play an important role in future HCV therapy. We are very encouraged by the pre-clinical data seen to date, which includes activity against some of the known mutations that lead to resistance to the current HCV therapies and a favorable potency and PK profile which supports a once-a-month dosing paradigm. We believe that RG-101 may be useful in difficult-to-treat HCV patients in combination with existing and emerging therapies and we look forward to exploring its clinical utility."

Garry E. Menzel, Ph.D., Chief Operating Officer and Executive Vice President of Finance, said, "As we prepare for clinical activity with RG-101, Regulus continues to maintain a strong financial position with over \$90 million in cash at quarter-end. We remain on track to achieve our stated goals of finishing 2013 with at least \$60 million in cash, while maintaining a burn rate of approximately \$30 million to \$35 million."

Recent Highlight

- **Selected New Opportunity in Oncology.** Regulus also announced today that it has selected microRNA-221 (miR-221) as an attractive target for potential clinical evaluation in patients with hepatocellular carcinoma (HCC). Regulus is currently conducting target validation activities and expects to report further progress on this opportunity in the second half of 2013.

First Quarter 2013 Financial Results & Highlights

Regulus reported a net loss of \$7.2 million for the quarter ended March 31, 2013, compared to a net loss of \$2.2 million for the quarter ended March 31, 2012. The quarter ended March 31, 2013, included non-cash charges of \$1.8 million from the change in value of the amended and restated convertible promissory note originally issued to GlaxoSmithKline in 2010, which was attributable to increases in value of the underlying common stock of the Company since December 31, 2012. Basic and diluted net loss per share was \$0.20 for the quarter ended March 31, 2013, compared to net loss per share of \$13.06 for the quarter ended March 31, 2012. The comparison of net loss per share for the periods presented is impacted by the initial public

offering and concurrent common stock issuances in October 2012.

Regulus recognized revenue of \$3.2 million for the quarter ended March 31, 2013, compared to \$3.3 million for the quarter ended March 31, 2012. 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 \$6.9 million for the quarter ended March 31, 2013, compared to \$4.6 million for the quarter ended March 31, 2012. The increase is attributable to an expansion of our research and development team, expertise and capabilities, in addition to increasing pre-clinical study activities in the first quarter of 2013, compared to the same period in 2012.

General and administrative expenses were \$1.9 million for the quarter ended March 31, 2013, compared to \$0.9 million for the quarter ended March 31, 2012. The increase is attributable to costs associated with increases related to additional personnel required to support the growth of the business, in addition to an increase in overall operating costs associated with being an SEC registrant in the first quarter of 2013, compared to the same period in 2012.

As of March 31, 2013, Regulus had \$90.7 million in cash, cash equivalents and short-term investments, debt with a principal balance of \$5.4 million and approximately 36 million shares of common stock outstanding. The Company expects its current cash position to be sufficient to fund operations into 2016 and to finish 2013 with at least \$60.0 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 for an update on the 'Road to the Clinic' Strategy and to discuss its first quarter 2013 financial results and recent highlights. A live webcast of the call will be available online at www.regulusrx.com. A replay will also be available approximately one hour after completion of the call. To access the replay, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 59485223. The webcast and replay will be archived on the company's website following the call.

About the 'Road to the Clinic' Strategy in 2013

Launched in February 2013, the 'Road to the Clinic' Strategy outlines certain corporate goals that seek to advance our microRNA therapeutic pipeline toward the clinic this year. Specifically, Regulus set the goal of nominating two microRNA candidates for clinical development in 2013. We announced the first candidate today as RG-101 for the treatment of HCV. The company also expects to file its first applications with regulatory authorities in 2014, with the first being for RG-101 in early 2014. In order to achieve these stated goals and advance our programs toward clinical development, Regulus expects to maintain its strong financial position and end 2013 with at least \$60 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 leveraging a mature therapeutic platform based on technology that has been developed over 20 years. Regulus works with a broad network of academic collaborators and leverages the oligonucleotide drug discovery and development expertise of its founding companies, Alnylam Pharmaceuticals and Isis Pharmaceuticals. Regulus has nominated RG-101 for the treatment of HCV as a candidate for clinical development and is advancing other microRNA therapeutics toward clinical development in several areas, including oncology, fibrosis and metabolic diseases. Regulus has formed strategic alliances with AstraZeneca, GlaxoSmithKline and Sanofi and a research collaboration with Biogen Idec.

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, the projected sufficiency of Regulus' capital position for future periods, the expected ability of Regulus to undertake certain activities and accomplish certain goals, 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 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' SEC filings. 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,	
	2013	2012
	(Unaudited)	
Revenues:		
Revenues under strategic alliances and collaborations	\$ 3,238	\$ 3,344
Operating expenses:		
Research and development	6,883	4,603
General and administrative	1,905	921
Total operating expenses	<u>8,788</u>	<u>5,524</u>
Loss from operations	(5,550)	(2,180)
Other income (expense), net	(1,689)	(66)
Loss before income taxes	(7,239)	(2,246)
Income tax (benefit) expense	(10)	1
Net loss	<u>\$ (7,229)</u>	<u>\$ (2,247)</u>
Basic and diluted net loss per share	<u>\$ (0.20)</u>	<u>\$ (13.06)</u>
Shares used to compute basic and diluted net loss per share	<u>35,872,606</u>	<u>171,998</u>

Regulus Therapeutics Inc.
Condensed Balance Sheets
(In thousands)

	March 31,	December 31,
	2013	2012
	(Unaudited)	
Assets		
Cash, cash equivalents and short-term investments	\$ 90,715	\$ 98,100
Other current assets	1,054	829
Noncurrent assets	5,258	4,589
Total assets	<u>\$ 97,027</u>	<u>\$ 103,518</u>
Liabilities and stockholders' equity		
Current liabilities	\$ 3,492	\$ 2,317
Current portion of deferred revenue	9,201	10,451
Convertible notes payable, at fair value	11,895	10,134
Other long-term liabilities	804	767
Deferred revenue, less current portion	15,768	17,756
Stockholders' equity	55,867	62,093
Total liabilities and stockholders' equity	<u>\$ 97,027</u>	<u>\$ 103,518</u>

SOURCE Regulus Therapeutics Inc.

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