

Regulus Reports Second Quarter 2013 Financial Results and Recent Highlights

- 'Road to the Clinic' Strategy On Track; Preparing for Clinical Development of RG-101 for HCV; Second microRNA Clinical Candidate to be Nominated by Year End -

- Entered into Option Agreement with Sanofi -

- Strengthened Financial Position with Successful Public Offering of Common Stock; Maintained Strong Balance Sheet with Pro forma Cash Position of Approximately \$129 Million; YE Cash Guidance Updated to End 2013 with Approximately \$110 Million in Cash -

LA JOLLA, Calif., Aug. 13, 2013 /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 quarter ended June 30, 2013 and provided a summary of recent corporate highlights.

"The second quarter was a period of significant progress for Regulus. We continued to execute on our 'Road to the Clinic' strategy and are preparing for clinical development of RG-101, a GalNAc-conjugated anti-miR which targets miR-122 for HCV. We also made great progress across the pipeline, advancing additional proprietary and partnered programs and identifying attractive new opportunities to broaden our microRNA platform," said Kleantis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "On the business front, we had a very productive quarter and recent period. We are pleased to have entered into an exclusive option agreement with Sanofi to negotiate the terms of a potential new relationship. Additionally, we strengthened our balance sheet with a successful public offering which will enable us to accelerate our 'Road to the Clinic' goals, expand our microRNA therapeutic pipeline and increase our cash runway to fund our operations into 2017."

"These recent accomplishments have positioned us for an exciting period focused on scientific execution," said Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus. "We look forward to entering the clinic with RG-101, nominating a second candidate for clinical development by the end of the year, and continuing to build a meaningful clinical pipeline based on microRNAs."

Recent Corporate Highlights

- **Secured Rights to Proprietary Process for Manufacturing GalNAc Conjugates from Alnylam .**

Regulus entered into an amendment to its Amended and Restated Collaboration Agreement with its founding companies, Isis Pharmaceuticals, Inc. and Alnylam Pharmaceuticals, Inc., to provide Regulus access to Alnylam's proprietary process for manufacturing GalNAc conjugates, a carbohydrate-based chemistry approach for asialoglycoprotein receptor-mediated delivery of oligonucleotides to hepatocyte cells of the liver.

The GalNAc conjugate chemistry has been utilized in the development of Regulus' lead program, RG-101, which targets miR-122 for the treatment of chronic hepatitis C virus, or HCV, infection. miR-122 is the most abundant microRNA in hepatocytes and the HCV virus co-opts miR-122 for its own replication and function. Utilizing the GalNAc conjugate chemistry has significantly improved the potency of the active oligonucleotide of RG-101 by achieving targeted delivery of the oligonucleotide to the infected hepatocytes. Regulus believes that GalNAc conjugate delivery is a critical component to its clinical development strategy for RG-101 and the Company expects to explore its potential applicability to additional microRNA programs.

- **Completed Successful Public Offering of Common Stock; Updated Year-End Cash Guidance Based on Strengthened Balance Sheet.** Regulus completed an underwritten public offering of 5,175,000 shares of common stock resulting in \$45.9 million of aggregate net proceeds, after deducting underwriting discounts and commissions and estimated offering expenses payable by Regulus. Primarily as a result of the successful offering, Regulus revised its prior year-end cash guidance of ending 2013 with at least \$60 million in cash, cash equivalents and short-term investments to ending 2013 with approximately \$110 million in cash, cash equivalents and short-term investments.

Second Quarter 2013 Highlights

- **Prepared for Clinical Development of RG-101 for the Treatment of HCV.** Regulus nominated its first microRNA candidate for clinical development, RG-101 for the treatment of patients with HCV. Regulus is developing RG-101 independently of its strategic alliance with GlaxoSmithKline (GSK), and as such, the companies amended the Product Development and Commercialization Agreement to clarify that RG-101 is fully owned by Regulus.

Currently, Regulus is conducting GLP toxicology studies with RG-101 designed to support clinical studies in man and expects to submit an application with regulatory authorities in the first half of 2014. For more information on RG-101, please visit <http://www.regulusrx.com/therapeutic-areas/rg-101/>.

- **Expanded and Advanced Therapeutic Pipeline.** Regulus expanded its microRNA therapeutic pipeline with the selection of microRNA-221 (miR-221) as an attractive target for potential clinical evaluation in

patients with hepatocellular carcinoma (HCC). Regulus continued to pursue several additional programs, both independently and with its strategic alliance partners, targeting miR-21 for the treatment of HCC and kidney fibrosis, targeting miR-10b for the treatment of glioblastoma, and targeting miR-33 for atherosclerosis. Additionally, Regulus advanced its exploratory efforts with the identification of several attractive microRNA targets, namely for oncology indications. In the second half of 2013, Regulus expects to nominate a second microRNA candidate for clinical development.

- **Entered into Option Agreement with Sanofi; Received Payment of \$2.5 Million.** In June 2013, the original term of the research and development phase of the Amended and Restated Collaboration and License Agreement with Sanofi expired, upon which the companies entered into an Option Agreement to enable exclusive negotiation of a potential new co-development and commercialization agreement, with such option period expiring on December 31, 2013, subject to an extension under specified circumstances. In July, Regulus received a \$2.5 million payment for such exclusivity, of which \$1.25 million is creditable against future amounts payable by Sanofi to Regulus under any future agreement we enter into pursuant to the Option Agreement. Specifically, Sanofi has the exclusive right to negotiate with the Company to enter into a development and license agreement for the co-development and commercialization of certain unencumbered microRNA targets and microRNA biomarkers. Regulus has the exclusive right to negotiate with Sanofi to enter into a co-development and commercialization agreement for certain miR-21 antimicroRNAs in oncology and in Alport Syndrome, a genetic disorder characterized by end-stage renal disease and hearing loss.
- **Revised Terms of Biomarker Agreement with Biogen Idec.** Regulus and Biogen Idec MA Inc. (Biogen Idec) entered into an amendment to the Collaboration and License Agreement to revise the terms with respect to phase 1 of the research plan and to reflect revised payment provisions relating to the phase 1 milestones. Regulus continues to explore microRNA biomarkers for multiple sclerosis and is eligible to receive milestone payments of up to \$1.3 million.
- **Named New Chairman of the Board.** Regulus named Stelios Papadopoulos, Ph.D. as Chairman of its Board of Directors. He assumed the role from John Maraganore, Ph.D., who remains a member of the Board. Dr. Papadopoulos, who has been a member of the Board since 2008, has more than 30 years of experience in the biotechnology and pharmaceutical industries as an investment banker, board member, company founder and investor. Dr. Papadopoulos is a board member of several companies including Biogen Idec, Inc. and Exelixis, Inc., where he is Chairman.

Second Quarter 2013 Financial Results & Highlights

Regulus reported a net loss of \$7.3 million and \$14.6 million for the three and six months ended June 30, 2013, respectively, compared to a net loss of \$2.6 million and \$4.8 million for the same periods in 2012. Net loss in the three and six months ended June 30, 2013 included \$2.7 million and \$4.5 million in non-cash charges, respectively, from the change in value of the amended and restated convertible promissory note originally issued to GSK in 2010, with such change in value attributable to increases in value of the common stock of Regulus since December 31, 2012. Basic and diluted net loss per share was \$0.20 and \$0.41 for the three and six months ended June 30, 2013, respectively, compared to net loss per share of \$10.78 and \$23.46 for the same periods in 2012. The comparability of net loss per share for the periods presented is impacted by our initial public offering and concurrent common stock issuances in October 2012.

Regulus recognized revenue of \$4.8 million and \$8.0 million for the three and six months ended June 30, 2013, respectively, compared to \$3.3 million and \$6.7 million for the same periods in 2012. Revenue during these periods consisted primarily of amortization of upfront payments received from our strategic alliances and collaborations, which is recognized over the estimated period of performance. The increase in revenue was primarily a result of the amendment to the Product Development and Commercialization Agreement with GSK in June 2013, upon which we and GSK agreed that we will develop RG-101 for HCV independently of the strategic alliance. As a result of this amendment, the remaining unamortized \$1.1 million associated with the upfront payment for the expansion into HCV was recognized due to an acceleration of our estimated period of performance.

Research and development expenses were \$7.7 million and \$14.6 million for the three and six months ended June 30, 2013, respectively, compared to \$4.9 million and \$9.5 million for the same periods in 2012. The increase is attributable to expansion of our research and development personnel and increase in IND-enabling activities for RG-101 in the first half of 2013, compared to the same period in 2012.

General and administrative expenses were \$1.7 million and \$3.6 million for the three and six months ended June 30, 2013, respectively, compared to \$1.0 million and \$1.9 million for the same periods in 2012. The increase is primarily attributable to an increase in headcount and additional incremental operating costs associated with being a public company.

As of June 30, 2013, Regulus had \$82.7 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.

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 2013 financial results and recent corporate highlights. 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 24265676. To access the telephone replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 24265676. The webcast and telephone 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. In May, Regulus nominated its first microRNA candidate for clinical development, RG-101, a GalNAc-conjugated microRNA antagonist or anti-miR, which targets miR-122 for the treatment of HCV. Regulus also expects to file its first applications with regulatory authorities in 2014, with the first being for RG-101 in the first half of 2014. Regulus believes its strong financial position supports these stated goals and expects to end 2013 with approximately \$110 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 is developing RG-101 for the treatment of HCV 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 (including Regulus' projected cash at the end of 2013), the projected sufficiency of Regulus' capital position for future periods, the future negotiation and execution of a co-development agreement with Sanofi, 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 Alnylam's proprietary process for manufacturing GalNAc conjugates and with respect to the nomination of a second microRNA candidate for clinical development), 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.

		Three months ended June 30,		Six months ended June 30,	
		2013	2012	2013	2012
(Unaudited)					
Revenues:					
Revenue under strategic alliances and collaborations	\$	4,759	\$ 3,309	\$ 7,997	\$ 6,653
Total revenues		4,759	3,309	7,997	6,653
Operating expenses:					
Research and development		7,722	4,883	14,604	9,487
General and administrative		1,723	984	3,628	1,905
Total operating expenses		9,445	5,867	18,232	11,392
Loss from operations		(4,686)	(2,558)	(10,235)	(4,739)
Other income (expense), net		(2,652)	(63)	(4,341)	(130)
Loss before income taxes		(7,338)	(2,621)	(14,576)	(4,869)
Income tax (benefit) expense		10	(22)	1	(22)
Net loss	\$	(7,348)	\$ (2,599)	\$ (14,577)	\$ (4,847)
Basic and diluted net loss per share	\$	(0.20)	\$ (10.78)	\$ (0.41)	\$ (23.46)
Shares used to compute basic and diluted net loss per share		35,994,642	241,223	35,933,961	206,610

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

**June 30,
2013** **December 31,
2012**

(Unaudited)

Assets

Cash, cash equivalents and short-term investments	\$	82,715	\$	98,100
Other current assets		4,158		829

Non-current assets	5,644	4,589
Total assets	\$ 92,517	\$ 103,518
Liabilities and stockholders' equity		
Current liabilities	\$ 4,129	\$ 2,317
Current portion of deferred revenue	14,876	10,451
Convertible notes payable, at fair value	14,592	10,134
Other long-term liabilities	1,765	767
Deferred revenue, less current portion	7,834	17,756
Stockholders' equity	49,321	62,093
Total liabilities and stockholders' equity	\$ 92,517	\$ 103,518

SOURCE Regulus Therapeutics Inc.

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<https://ir.regulusrx.com/2013-08-13-Regulus-Reports-Second-Quarter-2013-Financial-Results-and-Recent-Highlights>