

Regulus Reports Fourth Quarter and Year-End 2012 Financial Results and Highlights

- Progressed Partnered and Proprietary microRNA Programs-*
- Formed or Expanded Multiple Strategic Relationships to Support our microRNA-based Therapeutics and Biomarker Platforms-*
- Successfully Completed our Initial Public Offering-*
- Maintained Strong Financial Position with over \$98 Million in Year End Cash, Cash Equivalents and Short-Term Investments-*

LA JOLLA, Calif., Feb. 20, 2013 /PRNewswire/ -- [Regulus Therapeutics Inc.](#) (NASDAQ:RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today reported its financial results for the fourth quarter and year-ended December 31, 2012 and recent company highlights.

"In 2012, we successfully achieved significant strategic and financial objectives that have enabled Regulus to become a publicly-traded biopharmaceutical company. In particular, we successfully completed our initial public offering, formed alliances with new partners and expanded existing relationships with our pre-existing partners. Our strategic relationships provide therapeutic expertise that will support our growing microRNA therapeutic platform," said Kleanthis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "With our solid strategic foundation in place, we are now focused on building a meaningful clinical portfolio. We continue to advance our partnered and proprietary programs toward the clinic and look forward to nominating our first microRNA candidate for clinical development in the first half of this year."

"Regulus continues to maintain a strong balance sheet, ending 2012 with greater than \$98 million in cash," said Garry E. Menzel, Ph.D., Chief Operating Officer and Executive Vice President of Finance. "Our solid financial position should enable us to operate into 2016 and execute on our current research and development objectives."

Fourth Quarter 2012 and Recent Corporate Highlights

- **Presented Positive Preclinical Data on miR-21 at Kidney Week 2012.** Regulus presented data demonstrating that therapeutic oligonucleotides targeting miR-21 (anti-miR-21) reduces the severity of fibrosis and improves renal function in a rodent model of Alport syndrome. Regulus believes these data validate that anti-miR-21 could be a potential new therapy for human kidney disease. Regulus' miR-21 therapeutic development program, which is partnered with Sanofi, is currently in preclinical testing for both kidney fibrosis and hepatocellular carcinoma.
- **Enhanced Executive Management Team and Augmented Board of Directors.** Victor Knopov, Ph.D., a recognized expert in oligonucleotide drug development, joined Regulus as Vice President, Pharmaceutical Development and added over thirty years of experience to its accomplished leadership team. Additionally, Douglas E. Williams, Ph.D. and Mark G. Foletta, CPA recently joined Regulus' Board of Directors, bringing significant commercial, financial and operational expertise in the biopharmaceutical industry to the Board.
- **Completed Significant Financing Activity.** Regulus raised \$80.9 million in gross proceeds, which included \$50.9 million from the sale of common stock in the Initial Public Offering ("IPO"), \$25.0 million from the sale of common stock in a private placement to new strategic partner AstraZeneca AB that closed concurrently with the IPO, and \$5.0 million from the issuance of a convertible note to new collaborator Biogen Idec MA Inc., which was converted into shares of common stock at the completion of the IPO.

Fourth Quarter and Year-End 2012 Financial Results and 2013 Financial Outlook

Regulus reported a net loss of \$6.9 million and \$17.4 million for the quarter and year ended December 31, 2012, respectively, compared to a net loss of \$2.0 million and \$7.6 million for the same periods in 2011. In 2012, net loss included non-cash charges resulting from a \$1.7 million loss on the extinguishment of debt associated with the amended and restated \$5.0 million convertible promissory note Regulus originally issued to GSK in 2010. Subsequent to the extinguishment, losses on the change in value of the amended and restated note resulted in additional non-cash charges of \$3.0 million, which was attributable to increases in value of the underlying common stock of the Company. Basic and diluted loss per share was \$0.22 and \$2.12 for the quarter and year ended December 31, 2012, compared to net loss per share of \$14.81 and \$85.82 for the same periods in 2011. Comparability 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 reported revenue of \$3.2 million and \$12.7 million for the quarter and year ended December 31, 2012, respectively, compared to \$3.4 million and \$13.8 million for the same periods in 2011. 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 \$5.6 million and \$20.3 million for the quarter and year ended December 31, 2012, respectively, compared to \$4.5 million and \$17.3 million for the same periods in 2011. The increase is attributable to continued investment in our research and development team, expertise and capabilities in response to our increased and expanded strategic partnerships and platform development initiatives.

General and administrative expenses were \$1.9 million and \$4.9 million for the quarter and year ended December 31, 2012, respectively, compared to \$0.8 million and \$3.6 million for the same periods in 2011. The increase is attributable to costs associated with the growth of the business and personnel infrastructure associated with the IPO in October 2012.

As of December 31, 2012, Regulus had \$98.1 million in cash, cash equivalents and short-term investments, debt with a principal balance of \$5.4 million and 35.8 million shares of common stock outstanding. The Company expects its current cash to be sufficient to fund operations into 2016 and to finish 2013 with at least \$60 million in cash, cash equivalents and short-term investments.

Conference Call & Webcast Information

Regulus will host a conference call and webcast at 5:00 pm Eastern Standard Time today to discuss its fourth quarter and year-end 2012 financial results, recent company highlights and its expectations for 2013. 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 10388595. The webcast and replay will be archived on the company's website for two weeks 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 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 advancing microRNA therapeutics toward clinical development in several areas, including oncology, fibrosis, hepatitis C 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.

Condensed Statements of Operations
(In thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2012	2011	2012	2011
(Unaudited)				
Revenues	3,238	3,363	12,700	13,789
Operating expenses:				
Research and development	5,607	4,466	20,342	17,289
General and administrative	1,934	773	4,932	3,637
Total operating expenses	7,541	5,239	25,274	20,926
Loss from operations	(4,303)	(1,876)	(12,574)	(7,137)
Other income (expense), net	(2,555)	(64)	(4,844)	(259)
Loss before income taxes	(6,858)	(1,940)	(17,418)	(7,396)
Income tax (benefit) expense	18	(75)	(10)	206
Net loss	\$ (6,876)	\$ (2,015)	\$ (17,408)	\$ (7,602)
Basic and diluted net loss per share	\$ (0.22)	\$ (14.81)	\$ (2.12)	\$ (85.82)
Shares used to compute basic and diluted net loss per share	31,907,152	136,041	8,212,538	88,582

Regulus Therapeutics Inc.
Condensed Balance Sheets
(In thousands)

	December 31, 2012	December 31, 2011
(Unaudited)		
Assets		
Cash, cash equivalents and short-term investments	\$ 98,100	\$ 38,144
Other current assets	829	522

Noncurrent assets	4,589	4,215
Total assets	\$ 103,518	\$ 42,881
Liabilities and stockholders' deficit		
Current liabilities	\$ 2,317	\$ 2,115
Deferred revenue	10,451	10,735
Convertible notes payable, at fair value	10,134	—
Other long-term obligations, less current portion	767	11,847
Deferred revenue, less current portion	17,756	16,987
Convertible preferred stock	—	42,691
Stockholders' equity (deficit)	62,093	(41,494)
Total liabilities and stockholders' equity (deficit)	\$ 103,518	\$ 42,881

SOURCE Regulus Therapeutics Inc.

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<https://ir.regulusrx.com/2013-02-20-Regulus-Reports-Fourth-Quarter-and-Year-End-2012-Financial-Results-and-Highlights>