

## Regulus Reports Third Quarter 2012 Financial Results and Recent Highlights

*-Formed Multiple Strategic Relationships to Support our microRNA-based Therapeutics and Biomarker Platforms -  
- Raised \$80.9M in Total Gross Proceeds from IPO and Other Recent Financial Transactions -*

LA JOLLA, Calif., Nov. 19, 2012 /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 September 30, 2012 and provided a summary of recent corporate highlights.

"The third quarter and subsequent period were the most successful to date for Regulus as measured by the achievement of significant financial and strategic goals," said Kleanthis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "Regulus is now focused on the execution of our research and development plans that include selection of clinical candidates in all of our microRNA therapeutic programs, which we believe will enable us to file at least two INDs by 2014."

"We expect that the capital raised in the last several months will provide sufficient funding for operations through at least the end of 2015," said Garry E. Menzel, Ph.D., Chief Operating Officer and Executive Vice President of Finance. "We believe our disciplined financial strategy will enable us to advance multiple programs toward the clinic without the need for additional equity financing."

### Third Quarter 2012 and Recent Corporate Highlights

- **Completed Initial Public Offering ("IPO").** In October 2012, Regulus completed its IPO, raising \$50.9 million in gross proceeds, inclusive of the over-allotment option exercised by the underwriters and insider participation by Isis Pharmaceuticals, Inc., one of its founding companies, and two of its strategic partners, Sanofi SA ("Sanofi") and GlaxoSmithKline plc ("GSK"). Regulus' common stock began trading on the NASDAQ Global Market on October 4, 2012 under the symbol "RGLS."
- **Established Significant Strategic Alliance Focused Primarily on Metabolic Diseases.** In August 2012, Regulus entered into a strategic alliance with AstraZeneca AB ("AZ") to discover, develop, and commercialize microRNA therapeutics for three exclusive targets focused on cardiovascular and metabolic diseases and oncology. AZ made an upfront payment of \$3.0 million and purchased \$25.0 million of Regulus' common stock in a private placement that closed concurrently with the IPO. In addition, Regulus is eligible to receive preclinical, clinical and commercialization milestone payments of up to \$509.0 million, as well as royalties.
- **Established Research Collaboration to Explore microRNA Biomarkers for Multiple Sclerosis.** In August 2012, Regulus formed a collaboration with Biogen Idec Inc. ("Biogen Idec") to explore microRNA biomarkers for multiple sclerosis. Regulus received an upfront payment of \$750,000 and is eligible to receive milestone payments of up to \$1.3 million. Regulus also received \$5.0 million from the sale of a convertible note to Biogen Idec which converted into shares of Regulus' common stock at the closing of the IPO.
- **Re-Aligned Board of Directors.** In November 2012, Douglas E. Williams, Ph.D., Executive Vice President of Research and Development at Biogen Idec, joined Regulus' Board of Directors. Concurrently, Stanley T. Crooke, M.D., Ph.D., Chairman of the Board and CEO of Isis Pharmaceuticals, Inc., and Barry E. Greene, President and Chief Operating Officer of Alnylam Pharmaceuticals, Inc., stepped down from the Board and their respective Board committees.

### Third Quarter 2012 Financial Results & Highlights

As of September 30, 2012, Regulus had cash, cash equivalents and short-term investments of \$30.9 million compared to \$38.1 million at December 31, 2011. The decrease in cash, cash equivalents and short-term investments was the result of approximately \$17.7 million used to fund the Company during the first nine months of 2012, offset by \$5.0 million in R&D funding from Sanofi received in July 2012, and \$5.8 million from Biogen Idec related to a \$5.0 million convertible note and a \$750,000 upfront payment received in August 2012.

### Net Loss

The net loss for the three and nine months ended September 30, 2012 was \$5.7 million and \$10.5 million, respectively, compared to \$1.0 million and \$5.6 million for the same periods in 2011. The increase in our 2012 net losses was the result of \$1.0 million less in revenue recognized on our upfront payments from GSK, the result of amending our strategic alliance agreement with GSK in June 2012, and an increase of \$1.6 million and \$2.0 million in operating expenses for the three and nine months ended September 30, 2012, respectively. Included as a component of net loss was a non-cash charge of \$1.7 million resulting from a loss on extinguishment of debt associated with the modification of the \$5.0 million convertible promissory note we

issued to GSK in 2010. The change in the fair value of the convertible promissory note is primarily attributable to the change in the value of our common stock price during the period. Our net loss also included non-cash stock-based compensation expenses of \$414,000 and \$732,000 for the three and nine months ended September 30, 2012, respectively, and \$196,000 and \$609,000 for the same periods in 2011. Basic and diluted net loss per share for the three and nine months ended September 30, 2012 was \$15.98 per share and \$41.03 per share, respectively, compared to \$11.68 per share and \$76.97 per share for the same period in 2011.

## **Revenue**

We recognized revenue of \$2.8 million and \$9.5 million in the three and nine months ended September 30, 2012, respectively, and \$3.8 million and \$10.4 million for the same periods in 2011. Our revenue during these periods consisted primarily of amortization of upfront payments received from GSK and Sanofi which we amortize monthly on a straight-line basis over our period of performance. The total amortization attributable to payments from Sanofi was \$2.5 million and \$7.5 million for each of the three and nine months ended September 30, 2012 and 2011, respectively. The total amortization attributable to upfront payments from GSK was \$186,000 and \$1.3 million for the three months ended September 30, 2012 and 2011, respectively, and \$1.8 million and \$2.9 million for the nine months ended September 30, 2012 and 2011, respectively. The decrease in the amount amortized for GSK in 2012 compared to 2011 is the result of our June 2012 amendment to the collaboration agreement which extended our period of performance.

## **Research and Development Expenses**

Research and development expenses were \$5.2 million and \$14.7 million for the three and nine months ended September 30, 2012, respectively, compared to \$3.9 million and \$12.8 million for the same periods in 2011. The increase of \$1.4 million and \$1.9 million for the three and nine month periods ended September 30, 2012 was primarily related to increases in payroll and related benefits, laboratory supplies and external services to advance our preclinical programs.

## **General and Administrative Expenses**

General and administrative expenses were \$1.1 million and \$3.0 million for the three and nine months ended September 30, 2012, respectively, compared to \$907,000 and \$2.9 million for the same periods in 2011. The increases primarily represent legal services related to our transactions with AZ and Biogen Idec completed in August 2012.

## **Conference Call & Webcast Information**

Regulus will host a conference call and webcast at 5:00 pm Eastern Standard Time today to discuss its third quarter 2012 financial results and highlights. A live webcast of the call will be available online at [www.regulusrx.com](http://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 52577487. 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 (NASDAQ:ALNY) and Isis Pharmaceuticals (NASDAQ:ISIS). 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 regulatory approval, 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' 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 per share data)**

	<b>Three months ended September 30,</b>		<b>Nine months ended September 30,</b>	
	<b>2012</b>	<b>2011</b>	<b>2012</b>	<b>2011</b>
<b>(Unaudited)</b>				
Revenues:				
Revenue under strategic alliances	\$ 2,809	\$ 3,809	\$ 9,462	\$ 10,426
Total revenues	2,809	3,809	9,462	10,426
Operating expenses:				
Research and development	5,248	3,875	14,735	12,823
General and administrative	1,093	907	2,998	2,864
Total operating expenses	6,341	4,782	17,733	15,687
Loss from operations	(3,532)	(973)	(8,271)	(5,261)
Other income (expense), net	(2,159)	(68)	(2,289)	(195)
Loss before income taxes	(5,691)	(1,041)	(10,560)	(5,456)
Income tax (benefit) expense	(6)	4	(28)	131
Net loss	\$ (5,685)	\$ (1,045)	\$ (10,532)	\$ (5,587)
Basic and diluted net loss per share	\$ (15.98)	\$ (11.68)	\$ (41.03)	\$ (76.97)
Shares used to compute basic and diluted net loss per share	355,735	89,438	256,682	72,588

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

**September 30,  
2012**                      **December 31,  
2011**

**(Unaudited)**

**Assets**

Cash, cash equivalents and short-term investments	\$ 30,893	\$ 38,144
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Other current assets	3,466	522
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Noncurrent assets	6,364	4,215
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Total assets	\$ 40,723	\$ 42,881
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**Liabilities and stockholders' deficit**

Current liabilities	\$ 2,814	\$ 2,115
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Deferred revenue	10,593	10,735
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Convertible notes payable	10,000	—
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Convertible notes payable, at fair value	7,069	—
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Other long-term obligations, less current portion	875	11,847
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Deferred revenue, less current portion	16,602	16,987
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Convertible preferred stock	42,691	42,691
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Stockholders' deficit	(51,148)	(41,494)
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Total liabilities and stockholders' deficit	\$ 40,723	\$ 42,881
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SOURCE Regulus Therapeutics Inc.

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<https://ir.regulusrx.com/2012-11-19-Regulus-Reports-Third-Quarter-2012-Financial-Results-and-Recent-Highlights>