

## Regulus Reports Fourth Quarter and Year-End 2015 Financial Results and Recent Highlights

LA JOLLA, Calif., Feb. 22, 2016 /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 fourth quarter and full-year ended December 31, 2015 and provided a summary of recent corporate highlights.

"2015 was a milestone year for the company with the filing of three investigational new drug applications in the United States, and two clinical trial applications in the European Union," said Paul Grint, M.D., President and CEO of Regulus. "Our priorities for 2016, based on the data seen to date, include acceleration of our clinical programs, advancing our pipeline and defining the regulatory path to approval for our lead programs."

### Fourth Quarter and Year-End 2015 Financial Results & Highlights

Regulus reported a net loss of \$7.2 million and \$55.7 million for the quarter and year ended December 31, 2015, respectively, compared to a net loss of \$22.2 million and \$56.7 million for the same periods in 2014. Basic and diluted net loss per share was \$0.14 for the quarter ended December 31, 2015, compared to \$0.47 for the same period in 2014. Basic and diluted net loss per share was \$1.08 for the year ended December 31, 2015, compared to \$1.29 for the same period in 2014.

Regulus recognized revenue of \$10.9 million and \$20.8 million for the quarter and year ended December 31, 2015, respectively, compared to \$4.2 million and \$7.7 million for the same periods in 2014. Revenue for the quarter and year ended December 31, 2015 included milestones earned under Regulus' strategic alliances and collaboration agreements of \$10.0 million and \$13.2 million, respectively, which included a \$10.0 million clinical milestone payment upon AstraZeneca's first patient dosing in a first-in-human Phase I clinical study of RG-125(AZD4076). Revenue from research services performed under Regulus' strategic alliances and collaborations was \$0.4 million and \$4.5 million for the quarter and year ended December 31, 2015, respectively. Other revenue during these periods consisted of amortization of up-front payments from Regulus' strategic alliances and collaborations, which is recognized over the estimated period of performance.

Research and development expenses were \$12.8 million and \$56.4 million for the quarter and year ended December 31, 2015, respectively, compared to \$10.5 million and \$41.0 million for the same periods in 2014. This increase was primarily driven by clinical trial costs for RG-101, pre-clinical study costs for RG-125 and an increase in salaries and related employee costs, including non-cash stock-based compensation.

General and administrative expenses were \$5.4 million and \$19.1 million for the quarter and year ended December 31, 2015, respectively, compared to \$3.3 million and \$11.5 million for the same periods in 2014. This increase was primarily driven by an increase in salaries and related employee costs, including non-cash stock-based compensation.

As of December 31, 2015, Regulus had \$115.3 million in cash, cash equivalents and short-term investments, including restricted cash of \$1.3 million, and 52,669,266 shares of common stock outstanding.

### Recent Highlights

#### **RG-101** (GalNAc-conjugated anti-miR122 for the treatment of Hepatitis C Virus)

- **Interim Results from Phase II Combination Study.** Regulus recently announced interim results from one of the company's ongoing Phase II studies of RG-101 for the treatment of Hepatitis C Virus infection (HCV). The study was designed to evaluate a shortened, four-week treatment regimen containing a subcutaneous administration of 2 mg/kg of RG-101 at Day 1 and Day 29, in combination with 4 weeks of once/daily approved anti-viral agents Harvoni®, Olysio®, or Daklinza™. The study enrolled 79 treatment naïve genotype 1 and 4 HCV patients (Harvoni® arm, n=27, Olysio® arm, n=27, Daklinza™ arm, n=25). Thirty-eight patients have been evaluated through 8 weeks of follow up. Ninety-seven percent of those patients (37/38) had HCV RNA viral load measurements below the limit of quantification. To date, RG-101 has been generally well tolerated with the majority of adverse events considered mild or moderate, and with no study discontinuations. For those patients through 12 weeks of follow-up, 100% remained below the limit of quantification (14/14). The primary endpoint analysis (12 week follow up) for all 79 patients in the study are anticipated to be reported in late Q2 2016.
- **Entered into Clinical Collaboration with GSK with Potential for Single Visit HCV Therapy.** In accordance with the GSK clinical collaboration, Regulus has initiated its Phase II clinical trial evaluating the combination of RG-101 and GSK2878175, a non-nucleoside NS5B polymerase inhibitor, in HCV patients. In parallel, GSK is working to develop a long-acting parenteral "LAP" formulation of GSK2878175 as a single intra-muscular injection, providing the potential for a single-visit therapeutic treatment for HCV. Regulus anticipates reporting interim safety and efficacy data from this study by the end of 2016.
- **Enrollment Nearly Complete in US Phase I Study.** Enrollment is nearly complete in a multi-center, open label, non-randomized Phase I study to compare the safety, tolerability, pharmacokinetics, and pharmacodynamics of RG-101 in subjects with severe renal insufficiency or end-stage renal disease ("ESRD") to healthy control subjects, and further explore RG-101 in hepatitis C infected subjects with severe renal insufficiency or ESRD. Regulus anticipates reporting safety and efficacy data from the HCV/severe renal impairment or ESRD arm in the second half of 2016.

#### **RG-012** (anti-miR21 for the treatment of Alport syndrome)

- **Completed Phase I Study.** Regulus completed a single-ascending dose, first-in-human, Phase I study evaluating the safety, tolerability and pharmacokinetics of subcutaneous dosing of RG-012 in healthy volunteers. RG-012 was well-tolerated with no serious adverse events or discontinuations reported. Regulus is scheduled to meet with the FDA towards the end of the first quarter of 2016 to discuss the Phase II program.
- **Advanced ATHENA Natural History Study.** Regulus continues to advance the ATHENA natural history study in patients with Alport syndrome and anticipates reporting initial observations in the second quarter of 2016 at a medical meeting. Longitudinal data obtained in the ATHENA natural history study will help inform the design of the Phase II study.

#### **RG-125** (GalNAc-conjugated anti-miR103/107 for the treatment of NASH)

- **Entered Phase I Development; Received \$10.0M Milestone Payment from AstraZeneca.** Dosing commenced in a first-in-human Phase I study of RG-125(AZD4076) by Regulus' collaboration partner AstraZeneca. Regulus received a \$10.0 million milestone payment from AstraZeneca, who will assume all ongoing development of RG-125(AZD4076).

## Additional Highlights

- **Attracted Key Talent.** Regulus appointed Joseph "Jay" Hagan as Chief Operating Officer, principal financial officer and principal accounting officer. Mr. Hagan is responsible for leading the company's operations and corporate development and serves as a key member of the executive leadership team.
- **Received Key Patents for Lead Programs.** The U.S. Patent and Trademark Office granted patents related to Regulus' most advanced microRNA therapeutics, RG-101 and RG-012, furthering the company's ability to protect its microRNA candidates.
- **Achieved All Milestones in Biomarkers Collaboration with Biogen; Earned \$3.7M.** Regulus realized the full potential of its collaboration with Biogen to identify microRNAs as biomarkers for multiple sclerosis and earned \$3.7 million in payments. The scope of the research under the current collaboration agreement has concluded.

## Conference Call & Webcast Information

Regulus will host a conference call and webcast at 5:00 p.m. Eastern Standard Time today to discuss its fourth-quarter and year-end 2015 financial results and recent company highlights. 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 49930922. To access the telephone replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), passcode 49930922. The webcast and telephone replay will be archived on the company's website 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 has leveraged its oligonucleotide drug discovery and development expertise to develop a well-balanced microRNA therapeutics pipeline complemented by a maturing microMarkers<sup>SM</sup> biomarkers platform and a rich intellectual property estate to retain its leadership in the microRNA field. Regulus is developing RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection, and 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. In addition, RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of NASH in patients with type 2 diabetes/pre-diabetes, has entered Phase I clinical development through its strategic alliance with AstraZeneca. Regulus is also advancing several programs toward clinical development in orphan disease indications, oncology and fibrosis. Regulus' commitment to innovation has resulted in multiple peer-reviewed publications in notable scientific journals and has resulted in the formation of strategic alliances with AstraZeneca and Sanofi. Regulus maintains its corporate headquarters in La Jolla, CA. 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 (including with respect to development and other activities related to RG-101, RG-012, RG-125), 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 or the Regulus microMarkers<sup>SM</sup> division 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 December 31,		Year ended December 31,	
	2015	2014	2015	2014
(Unaudited)				
Revenues:				
Revenue under strategic alliances	\$ 10,860	\$ 4,219	\$ 20,759	\$ 7,669
Operating expenses:				

Research and development	12,794	10,474	56,387	41,046
General and administrative	5,427	3,278	19,130	11,533
Total operating expenses	18,221	13,752	75,517	52,579
Loss from operations	(7,361)	(9,533)	(54,758)	(44,910)
Other income (expense), net	139	(12,635)	(1,008)	(11,769)
Loss before income taxes	(7,222)	(22,168)	(55,766)	(56,679)
Income tax (expense) benefit	(4)	—	18	(1)
Net loss	\$ (7,226)	\$ (22,168)	\$ (55,748)	\$ (56,680)

Net loss per share, basic and diluted	\$ (0.14)	\$ (0.47)	\$ (1.08)	\$ (1.29)
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Weighted average shares used to compute basic and diluted net loss per share	52,477,489	46,863,382	51,411,353	44,090,165
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**Regulus Therapeutics Inc.  
Condensed Balance Sheets  
(In thousands)**

**December 31,  
2015**      **December 31,  
2014**

**Assets**

Cash, cash equivalents, short-term investments and restricted cash	\$	115,319	\$	159,743
Other current assets		18,939		5,208
Non-current assets		6,825		6,529
Total assets	\$	141,083	\$	171,480

**Liabilities and stockholders' equity**

Current liabilities	\$	11,438	\$	8,698
Total deferred revenue		3,259		6,349
Convertible notes payable, at fair value		—		23,397
Other long-term liabilities		2,308		1,022
Stockholders' equity		124,078		132,014
Total liabilities and stockholders' equity	\$	141,083	\$	171,480

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/regulus-reports-fourth-quarter-and-year-end-2015-financial-results-and-recent-highlights-300223854.html>

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<https://ir.regulusrx.com/2016-02-22-Regulus-Reports-Fourth-Quarter-and-Year-End-2015-Financial-Results-and-Recent-Highlights>