



## Regulus Reports First Quarter 2018 Financial Results and Pipeline Progress

May 10, 2018

### Two pre-clinical programs advance towards clinical candidates Conference call today at 5:00 p.m. ET

LA JOLLA, Calif., May 10, 2018 /PRNewswire/ -- [Regulus Therapeutics Inc.](http://www.regulusrx.com) (Nasdaq: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today reported financial results for the first quarter ended March 31, 2018 and provided a pipeline update.

Regulus Therapeutics Inc. Logo

"We are very pleased with the progress being made on advancing our pipeline, including the recent initiation of the multiple ascending dose (MAD) study for RGLS4326; the advancement of two new pre-clinical programs in important areas of unmet need; and the continued advancement of the RG-012 program," said Jay Hagan, President and Chief Executive Officer of Regulus. "These two new pre-clinical programs represent attractive areas of development for Regulus beyond our two chronic kidney disease programs."

#### Pipeline Update

- **RGLS4326 for autosomal dominant polycystic kidney disease (ADPKD):** As previously announced, a Phase 1 MAD study was recently initiated in healthy volunteers. This trial was initiated based on data from the ongoing Phase 1 single ascending dose (SAD) trial, in which RGLS4326 has been determined to be well tolerated to date. The Phase 1 SAD study has completed dose escalation and continues in the planned follow-up phase, which is on-track for completion in the second half of 2018. Data from both studies will provide pharmacokinetics and safety data in advance of the Phase 2 proof-of-concept (POC) study estimated for initiation in the second half of 2019.
- **Pre-clinical programs:** Based on robust human in vitro data and murine in vivo data, the Company announced today it is advancing programs in Hepatitis B virus and immunology (targets undisclosed).
- **RG-012 for Alport syndrome:** The Phase 2 HERA study is ongoing and data from the Phase 1 renal biopsy study is anticipated by year-end 2018.

#### Financial Results

**Cash Position:** As of March 31, 2018, Regulus had cash, cash equivalents and short-term investments of \$45.1 million.

**Research and Development (R&D) Expenses:** R&D expenses were \$11.8 million for the quarter ended March 31, 2018, compared to \$15.8 million for the quarter ended March 31, 2017. The decrease was primarily the result of a reduction in personnel-related costs subsequent to our May 2017 corporate restructuring and the wind-down of clinical activities related to the RG-101 program.

**General and Administrative (G&A) Expenses:** G&A expenses were \$3.8 million for the quarter ended March 31, 2018, compared to \$4.0 million for the quarter ended March 31, 2017.

**Revenue:** Revenue was less than \$0.1 million for the quarters ended March 31, 2018 and 2017.

**Net Loss:** Net loss was \$16.0 million, or \$0.15 per share (basic and diluted), for the quarter ended March 31, 2018, compared to a net loss of \$20.0 million, or \$0.38 per share (basic and diluted), for the quarter ended March 31, 2017.

#### Conference Call Details

Regulus will host a conference call and webcast today at 5:00 p.m. Eastern Time to discuss first quarter financial results and provide a general business update. 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 8993969. To access the replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), conference ID 8993969. 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 clinical stage 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 rich intellectual property estate to retain its leadership in the microRNA field. Regulus is advancing several programs in renal, hepatic and central nervous systems diseases. 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 the expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-012 or RGLS4326), 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 and biomarkers 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 |             |
|---|--------------------|-------------|
|   | 2018               | 2017        |
| Revenues:   |                    |             |
| Revenue under strategic alliances   | \$ 18              | \$ 18       |
| Operating expenses:   |                    |             |
| Research and development  | 11,828             | 15,752      |
| General and administrative  | 3,773              | 3,959       |
| Total operating expenses  | 15,601             | 19,711      |
| Loss from operations  | (15,583)           | (19,693)    |
| Other expense, net  | (441)              | (332)       |
| Loss before income taxes  | (16,024)           | (20,025)    |
| Income tax (expense) benefit  | (1)                | 4           |
| Net loss  | \$ (16,025)        | \$ (20,021) |
| Net loss per share, basic and diluted   | \$ (0.15)          | \$ (0.38)   |
| Weighted average shares used to compute basic and diluted net loss per share: | 104,018,273        | 52,990,383  |

|   | March 31,<br>2018 | December 31,<br>2017 |
|---|-------------------|----------------------|
| Cash, cash equivalents and short-term investments | \$ 45,134         | \$ 60,074            |
| Total assets                                      | 62,079            | 77,809               |
| Term loan, less debt issuance costs               | 19,874            | 19,859               |
| Stockholders' equity                              | 22,876            | 35,216               |

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