



Regulus Presents Preclinical Survival Data Highlighting Potential of Novel Treatment for Glioblastoma Multiforme

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Single Dose of Lead anti-miR-10b Compound in Combination with temozolomide Resulted in over 150% Increase in Median Survival

LA JOLLA, Calif., Nov. 19, 2018 /PRNewswire/ -- [Regulus Therapeutics Inc.](http://www.regulusrx.com) (NASDAQ: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs, today announced that it presented new data on the therapeutic potential of its lead anti-miR candidate for the treatment of glioblastoma multiforme (GBM), at the Society for Neuro-Oncology meeting held November 15-18, 2018 in New Orleans, LA. The Company presented data highlighting the following:

- Confirmation of the differential upregulation of miR-10b in GBM tumor cells compared to normal brain tissue and a panel of other normal and malignant cell types.
- Identification of highly potent and specific anti-miR-10b oligonucleotide compounds that inhibited the growth of human GBM cell lines *in vitro* and *in vivo*.
- Demonstration of significant synergy benefit with the Company's lead anti-miR-10b compound. Results showed efficacy as monotherapy (18% increase in median survival vs. control) comparable to standard of care temozolomide (27% increase in median survival) and synergy in combination with temozolomide (>150% increased median survival; $p = 0.0001$) in an orthotopic tumor model in which human GBM cells were surgically implanted into mouse brains. Further details are available in the poster provided on Regulus' website.



"These results provide strong evidence that targeting miR-10b may provide therapeutic benefit in patients with GBM and supports the continued advancement of our program to address this cancer which has no highly effective therapy," said Dr. Timothy Wright, Regulus' Chief R&D Officer. "Regulus anticipates selection of a clinical candidate by year-end."

About Regulus

Regulus Therapeutics Inc. (Nasdaq: RGLS) is a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a pipeline complemented by a rich intellectual property estate in the microRNA field. 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 and objectives (including with respect to development and other activities related to its miR-10b program in GBM). 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.

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