Regulus Announces Clinical Candidate Nomination for the Treatment of Glioblastoma Multiforme

January 7, 2019

LA JOLLA, Calif., Jan. 7, 2019 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs, today announced the nomination of RGLS5579 as a clinical candidate for the treatment of glioblastoma multiforme ("GBM").

As recently disclosed at a scientific meeting, a single dose of RGLS5579 demonstrated increased survival as monotherapy vs. control in an orthotopic GBM animal model (18% increase in median survival vs. control) and in combination with temozolomide, a single dose of RGLS5579 significantly increased median survival (159% increased median survival vs. control; p = 0.0001). Standard of care temozolomide as a single agent increased median survival by 27% vs. control, therefore the combination of RGLS5579 plus temozolomide demonstrated synergistic efficacy in this model. RGLS5579 has completed the clinical candidate screening process demonstrating appropriate preliminary safety and drug-like properties.

"We are excited to announce our clinical candidate for the treatment of GBM, a devastating form of brain cancer, for which therapeutic options are extremely limited. The combination data we have generated, and repeated, may suggest that we can significantly extend expectations for survival for patients with GBM over the current standard of care," said Jay Hagan, President and Chief Executive Officer of Regulus. "RGLS5579 represents a novel approach to targeting a microRNA that has been shown to be important for the proliferation of GBM tumors."

About GBM

GBM is a highly malignant form of brain tumor. miR-10b is highly expressed in all GBM molecular subtypes, and its expression in normal brain cells is nearly undetectable. Its cells reproduce quickly and are nourished by a large network of blood vessels, fueling rapid growth and making it one of the most aggressive forms of brain cancer. Because of its aggressiveness, GBM is categorized as a high grade glioma (HGG). GBM is one of the most common forms of HGG, with new diagnoses estimated to be approximately 12,500 in 2018 in the United States. It accounts for about half of all brain and central nervous system cancers and is reported to have a five-year survival rate of approximately five percent.

About RGLS5579

RGLS5579 is a novel oligonucleotide designed to inhibit miR-10b. Preclinical studies with RGLS5579 have demonstrated direct anti-tumor effects in vitro and in vivo. RGLS5579 was selected from a library of over 200 anti-miRs targeting miR-10b based on its potency, selectivity and safety profile. In an orthotopic mouse model in which human GMB tumor cells were implanted into the brains of immunocompromised mice, RGLS5579 increased median survival as a monotherapy, and markedly extended median survival time in combination with TMZ.

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 (including with respect to the advancement of RGLS5579 for the treatment of glioblastoma multiforme), 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 and 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.

View original content to download multimedia:http://www.prnewswire.com/news-releases/regulus-announces-clinical-candidate-nomination-for-the-treatment-of-glioblastoma-multiforme-300773622.html

SOURCE Regulus Therapeutics Inc.

Investor Relations Contact: Dan Chevallard, Chief Financial Officer, 858-202-6376, dchevallard@regulusrx.com