# Regulus Announces Continuation of RG-101 Clinical Hold

*FDA requests longer-term follow-up data from ongoing studies*

LA JOLLA, Calif., Jan. 27, 2017 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that it received written communication from the U.S. Food and Drug Administration (FDA) that the clinical development program for RG-101 remains on clinical hold. In June 2016, RG-101 was placed on clinical hold following the Company's submission of a second serious adverse event (SAE) of jaundice.

Late last year, Regulus submitted a complete response to the FDA's initial request for information, which included identification of a potential mechanism of hyperbilirubinemia. The Company also submitted a proposal to mitigate this risk. Subsequently, the FDA has requested the final safety and efficacy data from on-going RG-101 clinical and pre-clinical studies before reconsidering the clinical hold. These data will be available once the current study protocols are complete through 48 weeks of follow up, which is anticipated in the fourth quarter. The FDA also requested additional expert review of liver safety data in light of the proposed mechanism of hyperbilirubinemia.

"While we are disappointed that the clinical hold was not lifted at this time, we plan to continue to work with the FDA to address their additional requests as we seek the removal of the clinical hold," said Dr. Timothy Wright, Chief R&D Officer of Regulus.

**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 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, both independently and with our strategic alliance partners, Sanofi and AstraZeneca. Regulus maintains its corporate headquarters in La Jolla, CA. For more information, please visit http://www.regulusrx.com.

**About microRNAs**

The discovery of microRNAs in humans during the last decade is one of the most exciting scientific breakthroughs in recent history. microRNAs are small RNA molecules, typically 20 to 25 nucleotides in length, that do not encode proteins but instead regulate gene expression. More than 800 microRNAs have been identified in the human genome, and over two-thirds of all human genes are believed to be regulated by microRNAs. A single microRNA can regulate entire networks of genes. As such, these molecules are considered master regulators of the human genome. microRNA expression, or function, has been shown to be significantly altered or dysregulated in many disease states, including oncology, fibrosis, metabolic diseases, immune-inflammatory diseases and HCV. Targeting microRNAs with anti-miRs, chemically modified, single-stranded oligonucleotides, offers a unique approach to treating disease by modulating entire biological pathways and may become a new and major class of drugs with broad therapeutic application.

**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-101), 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.

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