

Regulus Demonstrates Initial Progress in microRNA Biomarkers MS Collaboration and Receives Milestone Payment from Biogen Idec

LA JOLLA, Calif., Nov. 20, 2013 /PRNewswire/ -- [Regulus Therapeutics Inc.](#) (*NASDAQ:RGLS*), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that it has demonstrated initial progress in its research collaboration with Biogen Idec, M.A. ("Biogen Idec") to discover microRNAs as biomarkers for multiple sclerosis ("MS") and has received a milestone payment.

Regulus has developed a highly reproducible, proprietary platform for extracting, profiling, and analyzing microRNAs from small volumes of serum. Under its collaboration with Biogen Idec, Regulus applied this platform to profile over 400 serum samples from MS patients and compared these samples against those from healthy volunteers in the largest known MS serum profiling experiment performed to date. The results of this large and successful experiment have provided the foundation for identifying potential biomarker signatures for patients with MS. Based on the initial progress, Regulus and Biogen Idec will now work on the next phase of the research collaboration to validate further the microRNA biomarker signatures in patients with relapse-remitting MS, the most common form of the disease.

"We are very excited about our early success in this collaboration and are hopeful that our efforts advance Biogen Idec's work in treating MS," said Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus. "Further, we believe that achievement of this milestone assigns significant value to our proprietary technology and demonstrates the potential of our emerging biomarkers platform to support our microRNA therapeutic pipeline." Regulus believes that microRNA biomarkers may be used to select optimal patient segments in clinical trials, to develop companion diagnostics, and to monitor disease progression or relapse.

In August 2012, Regulus entered into a research collaboration with Biogen Idec to explore microRNAs as biomarkers for disease monitoring and response to therapy. In June 2013, Regulus and Biogen Idec amended the collaboration agreement to update the research plan and criteria for success. The collaboration is initially focused on the discovery of microRNAs as biomarkers for MS. Under the transaction, Regulus has received an upfront payment and has earned an initial milestone payment and is also eligible to receive additional milestone payments. Additionally, Biogen Idec made an investment in Regulus in the form of a promissory note that converted into shares of Regulus' common stock upon the closing of the Company's initial public offering in October 2012.

About microRNAs and microRNAs as Biomarkers

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 500 microRNAs have been identified in the human genome, and over one-third of all human genes are believed to be regulated by microRNAs. microRNA expression, or function, has been shown to be significantly altered or dysregulated in many disease states, including oncology, fibrosis and metabolic diseases. Regulus believes that microRNAs are clinically relevant therapeutic targets and may be ideally suited as biomarkers for these disease states and others, like MS.

microRNAs have been detected in bodily fluids such as blood, and emerging data has demonstrated that microRNA signatures in blood can mimic the expression profile observed in disease tissues. Regulus has a comprehensive intellectual property estate and oligonucleotide technology know-how and believes that microRNA biomarkers may be used to select optimal patient segments in clinical trials, to develop companion diagnostics, and to monitor disease progression or relapse.

About Regulus

Regulus Therapeutics Inc. (*NASDAQ:RGLS*) is a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus is uniquely positioned to leverage a mature therapeutic platform that harnesses the oligonucleotide drug discovery and development expertise of Alnylam Pharmaceuticals, Inc. and Isis Pharmaceuticals, Inc., which founded the company. Regulus has a well-balanced microRNA therapeutic pipeline entering clinical development, an emerging microRNA biomarkers platform to support its therapeutic programs, and a rich intellectual property estate to retain its leadership in the microRNA field. Regulus intends to focus its proprietary efforts on developing microRNA therapeutics for oncology indications and orphan diseases and is currently advancing several programs toward clinical development in oncology, fibrosis and metabolic diseases. Regulus is also developing RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122, for the treatment of chronic hepatitis C virus infection. Regulus' commitment to

innovation and its leadership in the microRNA field have enabled the formation of strategic alliances with AstraZeneca, GlaxoSmithKline and Sanofi. In addition, Regulus has formed a research collaboration with Biogen Idec around its emerging microRNA biomarkers platform.

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 Regulus' expectations regarding future therapeutic and commercial potential of Regulus' business plans, technologies and intellectual property related to microRNA therapeutics 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' programs are described in additional detail in Regulus' SEC filings. 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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