

## Regulus Expands its Biomarkers Platform and Establishes Regulus microMarkers™, a New R&D Division Focused on Identifying microRNAs as Biomarkers of Disease

*-Regulus microMarkers™ Designed to Support Regulus' Therapeutic Pipeline, its Collaborators and Strategic Partners-*

LA JOLLA, Calif., Jan. 13, 2014 [/PRNewswire/](#) -- Regulus Therapeutics Inc. (*NASDAQ:RGLS*), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today that it has established Regulus *microMarkers™*, a new research and development division of Regulus designed to support the expansion of its innovative biomarkers platform aimed at identifying microRNAs as biomarkers for disease. Regulus *microMarkers™* utilizes a clinically-validated, highly reproducible, proprietary technology platform to extract, profile, and analyze microRNAs as potential biomarkers for disease.

Additionally, Regulus controls key intellectual property and know-how related to the Regulus *microMarkers™* division.

"At Regulus, we continue to focus our efforts on truly innovative ideas that we believe will transform the field of drug discovery and Regulus *microMarkers™* was born from such innovation," said Kleanthis G. Xanthopoulos, Ph.D., President and CEO. "We believe that with our technical expertise and know-how, we can enhance early drug discovery and development processes by identifying microRNAs as biomarkers of disease and strengthening our therapeutic programs, and our collaborators' and strategic partners' programs."

Regulus believes that microRNA biomarkers are of significant value and may provide opportunities to develop companion diagnostics for its therapeutic candidates that may be developed, such as its program targeting microRNA-21 for kidney fibrosis, and any drugs developed by other companies. In 2012, Regulus formed a research collaboration with Biogen Idec focused on the discovery of microRNAs as biomarkers for multiple sclerosis. More recently, Regulus has entered into an arrangement with another leading, commercial-stage pharmaceutical company to explore microRNAs as biomarkers for specific patient populations. 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.

### **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.

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 rich 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 Therapeutics Inc.**

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 which it believes will allow it 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, the Company has established Regulus *microMarkers™*, a research and development division focused on identifying *microRNAs* as biomarkers of human disease, which is designed to support its therapeutic pipeline, collaborators and strategic partners.

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, or risks associated with discovering, developing and commercializing biomarkers and in the endeavor of building a business around such drugs and biomarkers. 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.

SOURCE Regulus Therapeutics Inc.

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