

Regulus Presents Positive Preclinical Data on miR-21 and miR-221 Oncology Programs In Oral and Poster Presentations at AACR 2014 Annual Meeting

*- Data Advances microRNA Oncology Portfolio -
-Oncology Portfolio Protected by Regulus' Strong Intellectual Property Estate-*

LA JOLLA, Calif., April 7, 2014 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ:RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that three abstracts will be presented in poster presentation sessions during the American Association for Cancer Research (AACR) Annual Meeting, which will be held April 5-9, 2014 in San Diego, CA. An oral presentation will also be presented by Regulus' strategic alliance partner, Sanofi. Data to be presented include preclinical studies investigating the therapeutic potential of targeting microRNA-21 ("miR-21") and microRNA-221 ("miR-221") for the treatment of hepatocellular carcinoma ("HCC"), the most common form of liver cancer, and biomarker discovery studies evaluating the pharmacodynamics signature for assessing miR-221 inhibition and microRNA expression in tumor tissues in mouse models of HCC. Each poster, after the time of its presentation, can be accessed on the investor relations page of Regulus' website, <http://ir.regulusrx.com/>

- **Poster Session on miR-221 - April 7, 2014 8:00 a.m. - 12:00 p.m. PDT**

- *miR-221: a Potential Therapeutic Target for Hepatocellular Carcinoma*
- *A miR-221 Multigene Pharmacodynamics Signature for Assessing miR-221 Inhibition*

- **Oral Presentation by Sanofi on miR-21 - April 8, 2014 3:35 p.m. PDT**

- *Targeting microRNA-21 in Hepatocellular Carcinoma*

- **Poster Session on microRNA Expression - April 9, 2014 8:00 a.m. - 12:00 p.m. PDT**

- *Serum microRNAs Reflect microRNA Expression in Tumor Tissues as Well as Systemic Response to Disease in HRAS-driven Mouse Models of HCC*

"Utilizing both genetic and pharmacological approaches, the data to be presented at AACR provide further evidence that miR-21 and miR-221 are validated preclinical targets for the treatment of hepatocellular carcinoma," said Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus. "In addition, we've made progress to develop gene signatures that enable demonstration of target inhibition in tumor tissues. We believe these gene signatures and the overall approach enable Regulus to demonstrate proof of mechanism in preclinical studies which may be translated into useful biomarker signatures in potential future clinical trials."

Update on Regulus' Liver Cancer Intellectual Property Estate

As part of Regulus' rich intellectual property estate, Regulus controls patent rights related to the modulation of multiple different microRNAs for the treatment of liver cancer. Regulus has already received patents in the United States for targeting multiple different microRNAs, including miR-21 and miR-221/222, for the treatment of liver cancer. Also included in the Regulus portfolio are fundamental patent rights claiming methods of treating liver cancer with mimics of microRNA-34a, one of the most well-validated tumor suppressor microRNAs.

Today, Regulus is pleased to announce receipt of a Decision to Grant a Patent from the Japan Patent Office for the use of anti-miRs targeting multiple different microRNAs, including miR-21 and miR-222, for the treatment of liver cancer.

"We are pleased to see our liver cancer patent estate further bolstered with new claims in a major market such as Japan. We believe our claims broadly cover the use of pharmaceutically relevant compounds targeting any of these multiple different microRNAs for the treatment of liver cancer," said David L. Szekeres, Chief Business Officer and General Counsel of Regulus. "As evidenced by these new claims, we continue to extract maximum value from our broad intellectual property estate, solidifying Regulus' position as the recognized leader in the field of microRNA therapeutics."

About Regulus' Intellectual Property Estate

Regulus believes that it has a leading intellectual property position and substantial know-how relating to the development and commercialization of microRNA therapeutics, composed of approximately 200 patents and patent applications that the company owns or has in-licensed from academic institutions and third parties including its founding companies, Alnylam Pharmaceuticals, Inc. and Isis Pharmaceuticals, Inc., related to microRNA and microRNA drug products. Regulus also has access to approximately 850 patents and patent

applications exclusively related to RNA technologies, including patents and patent applications relating to chemical modification of oligonucleotides that are useful for the development of microRNA therapeutics.

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. Specifically, Regulus is developing RG-012, an anti-miR targeting microRNA-21 for the treatment of Alport Syndrome, a life-threatening kidney disease driven by genetic mutations with no approved therapy, and 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, 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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