# Regulus Announces Notice of Allowance from U.S. Patent Office Related to microRNA-103/107 Program in Metabolic Disorders

*-Newly Allowed Claims Demonstrate Regulus' Leadership in the microRNA Field-*

LA JOLLA, Calif., Sept. 24, 2014 /PRNewswire/ -- Regulus Therapeutics Inc. (*NASDAQ:RGLS*), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today that the U.S. Patent and Trademark Office ("USPTO") has issued a Notice of Allowance in the company's exclusively licensed ETH Zurich patent family, for claims that cover methods of reducing blood glucose with modified oligonucleotides targeting microRNA-103/107 ("miR-103/107"). In addition to this recently allowed application, the patent family includes applications pending in other major markets including Australia, Canada, China, Europe and Japan.

The exclusively licensed technology relates to the discovery that inhibition of miR-103/107 with proprietary, chemically modified oligonucleotides improved glucose homeostasis and insulin sensitivity in mouse models of diabetes and obesity (Trajkovski et al., Nature, 2011). These findings, which originated from the laboratory of Dr. Markus Stoffel, Professor of the Institute of Molecular Health Sciences at ETH Zurich and member of Regulus' Scientific Advisory Board, established miR-103/107 as a potential therapeutic target for the treatment of metabolic disorders, including type 2 diabetes and obesity.

"Regulus continues to build and strengthen its intellectual property position, which includes over one thousand patents and patent applications, and supports each of our therapeutic programs as part of our overall development and commercialization strategy," said David Szekeres, Chief Business Officer and General Counsel. "The Notice of Allowance is for claims directed to methods of lowering blood glucose in subjects including those having a variety of metabolic disorders, with anti-miRs targeting miR-103/107, including anti-miRs in development by Regulus."

Neil W. Gibson, Ph.D., Regulus' Chief Scientific Officer, added, "In preclinical models of diabetes, we have demonstrated that inhibition of miR-103/107 with our anti-miRs leads to a sustained reduction in fasting glucose and fasting insulin levels. Currently, Regulus and AstraZeneca are working to develop an anti-miR-103/107 to treat metabolic disorders and we expect to present additional preclinical data on this exciting program at upcoming scientific conferences."

**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 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.

**About the 'Clinical Map Initiative'**

Launched in February 2014, Regulus' 'Clinical Map Initiative' outlines certain corporate goals to advance its microRNA therapeutics pipeline over the next several years. Regulus expects to report human proof-of-concept results in the Phase I clinical study of RG-101 for the treatment of HCV by the end of 2014, initiate a Phase I clinical study of RG-012 for the treatment of Alport syndrome in the first half of 2015, nominate a third microRNA candidate for clinical development by the end of 2014, and maintain a strong financial position and end 2014 with at least $75.0 million in cash, cash equivalents and short-term investments.

**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 therapeutics 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 and a research collaboration with Biogen Idec focused on microRNA biomarkers. In addition, the Company has established Regulus microMarkers™, a 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 financial estimates (including Regulus' projected cash at the end of 2014), the projected sufficiency of Regulus' capital position for future periods, the expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-012 and RG-101 and with respect to the nomination of a third microRNA candidate for clinical development), 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.

SOURCE Regulus Therapeutics Inc.

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