Regulus Announces Significant Progress in Strategic Alliance with AstraZeneca and Selection of a New Pre-Clinically Validated microRNA Oncology Target

LA JOLLA, Calif., Oct. 28, 2013 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today the selection of an undisclosed oncology target by AstraZeneca under the strategic alliance to discover, develop, and commercialize microRNA therapeutics. Regulus and AstraZeneca continue to collaborate on three exclusive microRNA targets in cardiovascular and metabolic diseases and oncology.

"We are very pleased with the successful relationship we have built with AstraZeneca and their selection of a validated microRNA target for oncology indications," said Neil W. Gibson, Ph.D., Chief Scientific Officer of Regulus. "In the last year, we have made significant progress in advancing our drug discovery efforts against the portfolio of AstraZeneca targets. With the support of AstraZeneca's drug development expertise and commitment to the RNA therapeutics field, we continue to harness our microRNA expertise to develop innovative, first-in-class treatment options for patients in need."

In August 2012, Regulus and AstraZeneca formed a strategic alliance focused on three exclusive microRNA targets for cardiovascular and metabolic diseases and oncology, which includes Regulus' program targeting microRNA-33 for the treatment of atherosclerosis. Under the terms of the alliance, Regulus will lead preclinical development and, if successful, could receive preclinical milestone payments. AstraZeneca will lead and fund the clinical development and commercialization of these programs. In addition, Regulus could also receive clinical milestones based on the successful development of microRNA therapies in each target area. Regulus is also eligible to receive significant launch and commercial milestone payments and royalties contingent on the successful commercialization of microRNA therapeutic products by AstraZeneca.

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 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 Idea around its emerging microRNA biomarkers platform.

For more information, please visit http://www.regulusrx.com.

About AstraZeneca

AstraZeneca is a global, innovation-driven biopharmaceutical business that focuses on the discovery, development and commercialisation of prescription medicines, primarily for the treatment of cardiovascular, metabolic, respiratory, inflammation, autoimmune, oncology, infection and neuroscience diseases. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information please visit: www.astrazeneca.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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