Regulus and Alport Syndrome Foundation Raise Awareness of Life-Threatening, Genetic Kidney Disease

LA JOLLA, Calif., March 25, 2014 /PRNewswire/ -- Regulus Therapeutics Inc. (*NASDAQ:RGLS*), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, and the Alport Syndrome Foundation ("ASF"), a non-profit corporation, announced today they are working together to raise awareness of Alport Syndrome, a life-threatening genetic kidney disease driven by genetic mutations with no approved therapy.

This month, Regulus will join the Alport Syndrome Foundation in observing National Kidney Month and the first ever Alport Syndrome Awareness Month. As part of that effort, Regulus hosted an Alport Syndrome awareness day at Regulus' corporate headquarters in San Diego, CA. Alport Syndrome patients Sharon Lagas and Ryan Landwehr were invited to provide Regulus employees with patient perspectives on the impacts of living with Alport Syndrome. Additionally, Regulus will serve as a Spirit of Action sponsor of the Alport Syndrome Foundation 5K for Healthy Kidneys on Sunday, March 30 th, 2014 in Tempe, Arizona. For more information, please http://www.alportsyndrome.org/how-can-i-help/fundraising-new#awareness.

"We are grateful for the support from Regulus and know that it will take patients working with companies like Regulus to advance the search for potential Alport Syndrome treatment options," said Sharon Lagas, President of the Alport Syndrome Foundation.

"Partnerships are critical to advancing scientific research for orphan diseases such as Alport Syndrome," said Kleanthis G. Xanthopoulos, Ph.D., President and CEO of Regulus. "We are hopeful that our relationship with the Alport Syndrome Foundation will advance our understanding of the disease and believe that ASF will serve as an invaluable resource to us as we develop RG-012, an anti-miR targeting microRNA-21 for the treatment of Alport Syndrome. We are excited to initiate a natural history of disease study for RG-012 in the third quarter this year and expect to initiate a Phase I clinical study of RG-012 in the first half of 2015."

Alport Syndrome is a genetic condition caused by mutations in the COL4A3, COL4A4 or COL4A5 genes which impacts the body's ability to create a specific type of collagen highly expressed in the kidney and essential to normal kidney structure. In the absence of this type of collagen, the kidneys are unable to effectively filter toxins and waste products, resulting in end-stage renal disease. Lack of this special type of collagen can also result in hearing loss or effects on vision. Alport Syndrome is considered an orphan disease with no approved therapy.

Regulus is currently developing an innovative new therapy for the treatment of Alport Syndrome. The company recently nominated a microRNA candidate to enter clinical development, RG-012, an anti-miR targeting microRNA-21 ("miR-21") for the treatment of Alport Syndrome. Preclinical studies have demonstrated that RG-012 is a potent inhibitor of miR-21 in both *in vitro* and *in vivo* models and that subcutaneous administration of RG-012 has significantly decreased the rate of renal fibrosis and increased the lifespan of the mice in the studies up to 50%. RG-012 has been well tolerated to date with a favorable pharmacokinetic profile that supports the potential for a once/week dosing regimen.

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.

About Alport Syndrome Foundation

The Alport Syndrome Foundation is an all-volunteer-led, non-profit organization founded by a group of families affected by the disease and guided by a Medical Advisory Committee of renowned Nephrologists. The Foundation was built on the past efforts of the late Dr. Curtis Atkin (who himself had Alport Syndrome) of the University of Utah and the Hereditary Nephritis Foundation. ASF is the leading independent non-profit organization in the United States serving and giving a voice to the Alport Syndrome community.

The ASF MISSION is to improve the lives of those affected by Alport Syndrome through education, empowerment, advocacy, and funding research. The ASF VISION is to make Alport Syndrome a treatable disease and ultimately find a cure.

For more information, please visit http://www.alportsyndrome.org/home/.

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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