# Regulus Expands Development of RG-101 through Clinical Trial Collaboration with GSK

*- Long-Acting Parenteral Formulation of GSK2878175 Being Developed; Co-Administration with RG-101 May Enable Single Visit Therapy for HCV Patients*

LA JOLLA, Calif., Nov. 3, 2015 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ:RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs (miR), today announced that it has expanded development of RG-101, Regulus' wholly-owned, GalNAc-conjugated anti-miR that targets miR-122, through a clinical trial collaboration and formulation development agreement with GlaxoSmithKline ("GSK") (NYSE: GSK). The companies plan to conduct a Phase II study to evaluate the combination of RG-101 and GSK2878175, an investigational non-nucleoside NS5B polymerase inhibitor, for the treatment of HCV. Concurrently, GSK will work on developing a long-acting parenteral for injection ("LAP") formulation of GSK2878175 which could improve patient compliance through reduced dosing intervals and potentially extend opportunities for HCV therapeutic intervention. This LAP formulation of GSK2878175 may be used in additional clinical trials together with RG-101 following completion of the planned Phase II study, although any additional studies are not covered by the collaboration agreement.

"We are pleased to work with GSK to advance the scientific understanding of the potential for a combination regimen co-administered all at once to treat HCV," said Paul Grint, M.D., President and CEO of Regulus. "The study to be conducted under this clinical collaboration represents one of many approaches Regulus is actively pursuing with RG-101. We remain committed to realizing the full potential of RG-101 and we look forward to initiating the combination study in the first quarter of 2016."

Zhi Hong, Senior Vice President and Head of the Infectious Diseases Therapy Area, GSK commented, "Building on GSK's long-acting formulation expertise and know how, we are excited about the potential of this combination to provide people living with HCV a new treatment option that could be delivered in a single visit."

Regulus will be responsible for conducting a multi-center, open-label Phase II study to evaluate the potential to achieve sustained viral responses post treatment with a single subcutaneous administration of 4 mg/kg of RG-101 in combination with daily oral administrations of 20 mg of GSK2878175 for up to 12 weeks in treatment-naive patients chronically infected with HCV genotypes 1 and 3. This study will be conducted outside the United States and is planned to begin in the first quarter of 2016. Neither Regulus nor GSK has any further obligations or commitments beyond the contemplated study under the clinical collaboration agreement.

**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 Regulus**

Regulus Therapeutics Inc. (NASDAQ:RGLS) is a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a well-balanced microRNA therapeutics pipeline complemented by a maturing microMarkers$^{SM}$ biomarkers platform and a rich intellectual property estate to retain its leadership in the microRNA field. Under its 'Clinical Map Initiative', Regulus is developing RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of chronic hepatitis C virus infection, and 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. In addition, RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of NASH in patients with type 2 diabetes/pre-diabetes, has been selected for clinical development. Regulus is also advancing several programs toward clinical development in orphan disease indications, oncology and fibrosis. Regulus' commitment to innovation has resulted in multiple peer-reviewed publications in notable scientific journals and has resulted in the formation of strategic alliances with AstraZeneca and Sanofi and a research collaboration with Biogen focused on microRNA

biomarkers.  Regulus maintains its corporate headquarters in La Jolla, CA.  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 the expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to development and other activities related to RG-101), 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.

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