Regulus to Present New RG-101 Data at The International Liver Congress™ 2016 (ILC 2016)

-Oral presentation during general session on interim RG-101 Phase II results & three posters to be presented-

LA JOLLA, Calif., March 16, 2016 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that new data on RG-101, the company's anti-miR-122 in Phase II development for the treatment of Hepatitis C Virus infection (HCV), will be presented as an oral presentation during the general session at The International Liver Congress™ (ILC) 2016, April 13-17 in Barcelona, Spain. Three poster presentations outlining additional RG-101 clinical and preclinical research will also be included as part of the meeting's poster sessions.

"Regulus and our clinical investigators are pleased to be making several presentations at this year's International Liver Congress, including an oral presentation during the general session elaborating on recent interim results from an ongoing Phase II combination study of RG-101 for the treatment of HCV," said Paul Grint, M.D., President and Chief Executive Officer of Regulus. "We believe the work presented further supports our novel approach to treating HCV as well as the clinical utility of RG-101 within the HCV landscape."

Oral Presentation

- Friday, April 15, 8:45am CET (General Session): RG-101 in Combination with 4 Weeks of Oral Direct Acting Antiviral Therapy Achieves High Virologic Response Rates in Treatment Naïve Genotype 1 and 4 Chronic Hepatitis C Patients: Interim Results from a Randomised, Multi-Center, Phase 2 Study (G. Horvath, Hungary).

Poster Presentations

- **Abstract THU-254**: A Single Dose of Anti-miR-122 Oligonucleotide RG-101 Results in a Less Activated Phenotype of NK Cells in Patients with Chronic Hepatitis C (F. Stelma, Netherlands).
- **Abstract THU-232**: Sequence Analysis for Resistance Monitoring Following A Single Dose of RG-101, an anti-miR Targeting microRNA-122, in Chronic Hepatitis C Patients (M. van der Ree, Netherlands).
- **Abstract THU-239**: RG-101 Demonstrates Favorable in Vitro Antiviral Activity and Cross Resistance Profile to Support Clinical Combination in HCV Patients (S. Neben, United States).

When available, the abstracts related to the presentations can be accessed through the ILC/EASL website at ilc-congress.eu.

About RG-101 for HCV

RG-101 is Regulus' wholly-owned, GalNAc-conjugated anti-miR targeting miR-122 for the treatment of HCV. In a completed Phase I human proof-of-concept study, Regulus demonstrated that treatment with a single subcutaneous dose of RG-101 as monotherapy resulted in significant and sustained viral load reductions in all treated HCV patients, including patients with difficult to treat genotypes, various liver fibrosis status and those who have experienced viral relapse after a prior IFN-containing regimen.

Recently, Regulus presented favorable interim data from an ongoing Phase II study evaluating the combination of RG-101 with multiple approved DAAs positioning RG-101 for both front-line and second-line commercial opportunities. Patients received a single subcutaneous injection of 2 mg/kg of RG-101 on Day 1, followed by 28 days of a once daily oral DAA (Harvoni®, Olysio®, or Daklinza™), followed by an additional subcutaneous injection of 2 mg/kg of RG-101 on Day 29. Regulus is planning to report primary endpoint results at 12 weeks following conclusion of treatment in late Q2 2016.

In collaboration with GSK, Regulus recently initiated a Phase II study evaluating the combination of RG-101 and GSK2878175, a non-nucleoside NS5B polymerase inhibitor, in treatment-naïve patients chronically infected with HCV genotypes 1 and 3. Additionally, enrollment is nearly complete in a multi-center, open label, non-randomized Phase I study to compare the safety, tolerability, pharmacokinetics, and pharmacodynamics of 2 mg/kg of RG-101 in subjects with severe renal insufficiency or end-stage renal disease (ESRD) to healthy control subjects, and further explore RG-101 in hepatitis C infected subjects with severe renal insufficiency or ESRD. Regulus anticipates reporting safety and efficacy data from the HCV/severe renal impairment or ESRD arm in the second half of 2016.

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. 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 entered Phase I clinical development through its strategic alliance with AstraZeneca. 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. 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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Additional assets available online: Photos (1)

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