Regulus Announces Successful Restructuring of Sanofi Collaboration

Sanofi Will Assume Development of RG-012 for Alport syndrome

LA JOLLA, Calif., Nov. 6, 2018 /PRNewswire/ -- Regulus Therapeutics Inc. (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs, today announced that it has amended and restructured its Collaboration and License Agreement with Sanofi (the "Amendment"). Under the terms of the Amendment, Regulus has granted Sanofi a worldwide exclusive license to develop and commercialize its investigational drug, targeting miR-21 for all indications, including Alport syndrome. Under the Amendment, Sanofi will assume all future costs and development activities associated with the advancement of RG-012, currently in Phase 2 for Alport syndrome.

Under the terms of the Amendment, Regulus is eligible to receive approximately $7 million in upfront and material transfer payments. Regulus is also eligible to receive up to $40 million in development milestone payments. In addition, Sanofi will reimburse Regulus for certain out-of-pocket transition activities and assume Regulus' upstream license royalty obligations.

"We are pleased with Sanofi's election to assume ongoing and future development of the RG-012 program in order to apply their expertise in rare diseases and to bring it forward as a potential new therapy for patients with Alport syndrome," said Jay Hagan, President and Chief Executive Officer of Regulus. "We look forward to a rapid transition of the program to Sanofi and their ongoing clinical development, while we continue to focus on our stated near-term objectives. Specifically, we believe this restructuring will improve our net cash position, yielding near term cash proceeds, eliminating RG-012-related spend over the next several years, and will enable us to focus our resources on our other promising programs targeting significant unmet medical needs in Autosomal Dominant Polycystic Kidney Disease and Hepatitis B virus."

Webcast and Conference Call

Regulus will host a conference call and webcast regarding this announcement at 8:30 a.m. Eastern Time today. A live webcast of the call will be available online at www.regulusrx.com. To access the call, please dial (877) 257-8599 (domestic) or (970) 315-0459 (international) and refer to conference ID 5085398. To access the replay of the call, dial (855) 859-2056 (domestic) or (404) 537-3406 (international), conference ID 5085398. The webcast and telephone replay will be archived on the Company's website following the call.

About Alport Syndrome

Alport syndrome is an inherited form of kidney disease caused by mutations in the type IV collagen genes (Col4A3, Col4A4 and Col4A5). Type IV collagen is important for maintaining the integrity of the glomerular basement membrane (GBM), a vital component in the kidney structure and filtration process. The genetic mutation in the collagen gene results in thickening in the GBM and impairment of glomerular filtration. Alport syndrome patients experience a progressive loss of kidney function, which ultimately leads to end stage renal disease requiring dialysis or kidney transplantation, or may even lead to death. Alport syndrome can also cause hearing loss and eye abnormalities during late childhood or early adolescence. ACE (angiotensin-converting enzyme) inhibitors, although not approved for the indication, are emerging as standard of care in patients with Alport syndrome to treat proteinuria, or abnormal amounts of protein in the urine, an indicator of chronic kidney disease. Alport syndrome represents a high unmet medical need with no approved therapy. miR-21 is believed to play a role in the disease progression and is up-regulated in Col4A3 deficient mouse models of Alport syndrome, other renal fibrosis models and human CKD patients. The role of miR-21 has been validated through genetic knock-out models and anti-miRs targeting miR-21 have reduced the severity of fibrosis in two distinct preclinical rodent models.

About RG-012

RG-012 is an investigational, single stranded, chemically modified oligonucleotide that binds to and inhibits the function of miR-21 for the treatment of Alport syndrome. In preclinical studies, RG-012 has demonstrated potent inhibition of miR-21 *in vitro* and *in vivo,* a decrease in the rate of progression of renal fibrosis, an increase in the lifespan of the Col4A3 deficient mice by up to fifty percent. In May 2017, Regulus completed a Phase I multiple-ascending dose, or MAD, study in 24 healthy volunteers (six-week repeat dosing) to determine safety, tolerability and PK of RG-012 prior to chronic dosing in patients. In Phase I clinical studies to date there were no serious adverse events, or SAEs, reported. Preliminary data from the first patients through a renal biopsy study followed by an open-label extension were encouraging, with kidney tissue concentrations achieved that would be predictive of therapeutic benefit based on animal disease models. In addition, modulation of the target, miR-21, was observed. RG-012 has received orphan designation in both the U.S. and Europe.

About Regulus

Regulus Therapeutics Inc. (Nasdaq: RGLS) is a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs. Regulus has leveraged its oligonucleotide drug discovery and development expertise to develop a pipeline complemented by a rich intellectual property estate in the microRNA field. 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 and objectives (including with respect to long term sustainability, the reduction of its cash burn, and advancement of its pipeline and programs). 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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