

Regulus Earns Milestone Payment from Biogen Idec Related to the Identification of Potential microRNA Biomarkers in Multiple Sclerosis

LA JOLLA, Calif., Feb. 2, 2015 /PRNewswire/ -- Regulus Therapeutics Inc., a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, announced today that it has earned a milestone payment from Biogen Idec (NASDAQ: BIIB) related to progress in the companies' research collaboration to identify microRNAs as biomarkers for multiple sclerosis ("MS"). To earn the milestone, Regulus' microMarkersSM division used its robust technology platform to successfully meet quality specifications for extracting and reproducibly profiling microRNAs from an initial set of whole blood samples of a recent Biogen Idec MS therapy trial. Regulus' microMarkersSM division is now working to identify a microRNA biomarker signature with the potential to differentiate relapse remitting MS patients from healthy volunteers and to compare with treatment response profiles.

"We are pleased with Regulus microMarkersSM continued progress and are hopeful that our innovative technology can enhance Biogen Idec's efforts to bring effective treatments to market for patients with multiple sclerosis," said David Szekeres, Chief Business Officer and General Counsel of Regulus.

Regulus and Biogen Idec began their collaboration in August 2012 and expanded their research in August 2014 to identify potential microRNA biomarkers in MS. Under the scope of the total research, Regulus has achieved two milestones and is eligible to receive future payments upon achievement of certain additional milestones related to the identification of microRNA signatures.

Key Regulus microMarkersSM Goals for 2015

In addition to the collaborative research being performed with Biogen Idec in 2015, Regulus microMarkersSM plans to support Regulus' microRNA therapeutics programs and its academic research partners by expanding the scope of the division's work. Specifically, Regulus microMarkersSM plans to (i) profile serum samples from the healthy volunteers and HCV patients in the ongoing clinical study of RG-101, a GalNAc-conjugated anti-miR targeting microRNA-122 for the treatment of HCV, to identify potential microRNA signatures, which may aid in accurately predicting a patient's response to RG-101 therapy; (ii) profile urine and blood samples from Alport syndrome patients enrolled in Regulus' ATHENA natural history of disease study to potentially identify a clinically useful microRNA signature; and (iii) profile and analyze microRNAs in multiple bodily fluids including plasma, serum, whole blood, urine and cerebrospinal fluid.

About microRNAs and microRNAs as Biomarkers

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 one-third 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. In addition to being clinically relevant therapeutic targets, microRNAs may be ideally suited as biomarkers for multiple diseases. Regulus believes that microRNA biomarkers are of significant value and may be used to select optimal patient segments in clinical trials and to monitor disease progression or relapse.

About Regulus microMarkersSM

Regulus microMarkersSM utilizes a highly reproducible, proprietary technology platform to extract, profile, and analyze microRNAs from small volumes of bodily fluids to differentiate disease from healthy patient samples and to identify microRNAs as potential biomarkers for disease. Regulus has profiled approximately 3,000 clinical samples in a wide variety of disease states including multiple sclerosis, chronic kidney disease, fatty liver, rheumatoid arthritis and others. Regulus has formed a research collaboration with Biogen Idec focused on the discovery of microRNAs as biomarkers for MS and has also entered into an arrangement with another leading, commercial-stage pharmaceutical company to explore microRNAs as biomarkers for specific patient populations. Regulus also maintains several academic research collaborations focused on the identification of microRNAs as biomarkers in multiple disease areas.

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 microMarkersSM biomarkers platform and a rich intellectual property estate to retain its domain dominant 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. 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 Idec 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 regarding the expected ability of Regulus to undertake certain activities and accomplish certain goals (including with respect to its Regulus' microMarkersSM goals in 2015), the projected timeline of clinical development activities, and expectations regarding future therapeutic and commercial potential of Regulus' business plans (including Regulus' expected future activities in 2015), 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 \(1\)](#)

<https://ir.regulusrx.com/2015-02-02-Regulus-Earns-Milestone-Payment-from-Biogen-Idec-Related-to-the-Identification-of-Potential-microRNA-Biomarkers-in-Multiple-Sclerosis>