



December 6, 2016

## Regulus Announces New Drug Development Candidates

LA JOLLA, Calif., Dec. 6, 2016 /PRNewswire/ -- Regulus Therapeutics Inc. (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced two new drug development candidates at its R&D day, held in New York. The first candidate, RGLS5040, is an anti-miR targeting microRNA-27 (miR-27) for the treatment of cholestatic diseases. The second candidate, RGLS4326, is an anti-miR targeting microRNA-17 (miR-17) for the treatment of autosomal dominant polycystic kidney disease (ADPKD).



"We are delighted to announce the addition of two new drug candidates to our growing portfolio of potential microRNA therapeutics. We believe both RGLS5040 and RGLS4326 have very favorable preclinical profiles with the potential to fulfill meaningful unmet medical needs," said Paul C Grint, M.D., President and Chief Executive Officer of Regulus. "We are currently conducting IND-enabling work and finalizing development plans for RGLS5040 and RGLS4326 and expect to file INDs for both drug candidates in the second half of 2017. Today's announcement of two new drug development candidates underlines the confidence we have in our rigorous targeting and validation process, which we believe should enable us to identify at least one new drug development candidate per year moving forward."

For additional details on Regulus' two new drug development candidates as well as other information detailed at Regulus' R&D day, please visit the investor relations section of Regulus' website [www.regulusrx.com](http://www.regulusrx.com) where a replay of the webcast presentation is available for a limited time.

### 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<sup>SM</sup> 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/IIa clinical development through its strategic alliance with AstraZeneca. Regulus is also advancing several programs toward clinical development in renal, hepatic and central nervous systems diseases. 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, 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.

Logo - <http://photos.prnewswire.com/prnh/20160519/370141LOGO>

To view the original version on PR Newswire, visit:<http://www.prnewswire.com/news-releases/regulus-announces-new-drug-development-candidates-300373935.html>

SOURCE Regulus Therapeutics Inc.

News Provided by Acquire Media