

Regulus Announces Publication in Nature Communications Identifying miR-17 as a Promising Drug Target for ADPKD

Published results from preclinical studies show inhibition of miR-17 increased survival and reduced disease burden in mice

LA JOLLA, Calif., Feb. 16, 2017 /PRNewswire/ -- [Regulus Therapeutics Inc.](http://www.regulusrx.com) (NASDAQ: RGLS), a biopharmaceutical company leading the discovery and development of innovative medicines targeting microRNAs, today announced that *Nature Communications* has published an article entitled "microRNA-17 family promotes polycystic kidney disease progression through the modulation of mitochondrial metabolism." The article highlights research that identifies miR-17 as a target for the treatment of autosomal dominant polycystic kidney disease (ADPKD) and can be found on-line at <http://www.nature.com/ncomms>.

"This successful collaboration between Regulus scientists and academic investigators, most importantly, Dr. Vishal Patel and his team at UT Southwestern Medical Center, represents a major advance in our understanding of the pathophysiology of ADPKD. These results provide strong evidence that targeting miR-17 may provide therapeutic benefit in humans and supports our development program for RGLS4326 (anti-miR-17) for the treatment of ADPKD," said Dr. Timothy Wright, Regulus' Chief R&D Officer. "Regulus expects to file an IND for RGLS4326 in the second half of 2017."

About Autosomal Dominant Polycystic Kidney Disease (ADPKD)

ADPKD, caused by the mutations of PKD1 or PKD2, is among the most common monogenetic disorders and a leading genetic cause of end-stage renal disease. The clinical hallmark of this disease is the presence of numerous renal tubule-derived cysts. Excessive proliferation, a central pathological feature, fuels the relentless expansion of cysts, ultimately causing end-stage renal disease. ADPKD affects approximately 600,000 people in the US and 12.5 million people worldwide.

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 rich intellectual property estate to retain its leadership in the microRNA field. Regulus is advancing several programs in renal, hepatic and central nervous systems diseases, both independently and with our strategic alliance partners, Sanofi and AstraZeneca. 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.

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