Regulus Therapeutics Announces Completion of Enrollment in Phase 2 Clinical Trial of Lademirsen for Alport Syndrome

--Final data and potential for \$25m milestone anticipated in H1 2023--

SAN DIEGO, Feb. 24, 2022 /<u>PRNewswire</u>/ -- <u>Regulus Therapeutics Inc.</u> (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs (the "Company" or "Regulus"), today announced the completion of enrollment in the Phase 2 HERA clinical study evaluating lademirsen (RG-012) for the treatment of adult patients with Alport Syndrome under the Company's Collaboration and License Agreement with Sanofi.

"We value our collaboration with Sanofi and continue to be proud of the steady progress being made not only in this study, but also in our technology to treat kidney disease," said Jay Hagan, President and Chief Executive Officer of Regulus Therapeutics. "With final data for this Phase 2 trial expected in the first half of next year, this study is a critical step in our effort to improve the lives of patients suffering from Alport Syndrome, a lifethreatening, genetic rare nephrology disease for which there are no currently available treatments."

Lademirsen is a single stranded, chemically modified oligonucleotide that binds to and inhibits the function of miR-21. Initiated in September 2017, the HERA study is a global, randomized, placebo-controlled Phase 2 study designed to assess the safety and tolerability of lademirsen, as well as the efficacy in reducing the decline in renal function in adult patients with Alport Syndrome. The Company expects HERA results in the first half of 2023.

In 2020, Regulus achieved \$10 million in milestone payments associated with the completion of transfer and verification of certain materials as well as an interim enrollment milestone from Sanofi for its progress with the HERA study. Under the terms of the Collaboration and License Agreement, the Company is also eligible to receive an additional \$25 million upon successful completion of the ongoing HERA study.

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 San Diego, CA.

Forward-Looking Statements

Statements contained in this presentation 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 collaboration with Sanofi including the potential completion of studies concerning lademirsen and receipt of milestones by Regulus therefrom. 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, and the risk additional toxicology data may be negative. In addition, while Regulus expects the COVID-19 pandemic to adversely affect its business operations and financial results, the extent of the impact on Regulus' ability to achieve its preclinical and clinical development objectives and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, guarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. These and other risks are described in additional detail in Regulus' filings with the Securities and Exchange Commission, including under the "Risk Factors" heading of Regulus most recently quarterly report on Form 10-Q. 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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