

Regulus Therapeutics Announces Update on Preclinical Studies in Amyotrophic Lateral Sclerosis in Collaboration with Researchers from Harvard's Brigham and Women's Hospital

Program advances to lead compound evaluation in preclinical in vivo models

Preliminary data for in vivo studies are expected in second half of 2023

SAN DIEGO, June 20, 2023 /PRNewswire/ -- [Regulus Therapeutics Inc.](#) (Nasdaq: RGLS), a biopharmaceutical company focused on the discovery and development of innovative medicines targeting microRNAs (the "Company" or "Regulus"), today announced an update on its preclinical studies evaluating a library of oligonucleotides designed to inhibit miR-155 for the treatment of Amyotrophic Lateral Sclerosis (ALS, or Lou Gehrig's disease). Regulus is pursuing this effort in collaboration with the laboratories of Oleg Butovsky, Ph.D., and Howard L. Weiner, M.D., at Brigham and Women's Hospital (BWH) and the Foundation for Neurologic Diseases (Boston, MA). The early-stage collaboration began in September 2021, and has now advanced to evaluation of compounds of interest in ALS preclinical in vivo models.

This milestone follows the successful completion of an oligonucleotide library and microglia cell-based screening campaign which identified compelling inhibitors of miR-155. Regulus and BWH are now evaluating the pharmacologic effects of selected anti-miR-155 inhibitors in preclinical ALS animal models, with an ultimate goal of achieving therapeutic efficacy and clinical translation for ALS patients. Preliminary data for these in vivo preclinical studies are expected in the second half of 2023.

"We are very excited about the latest progress in our productive partnership with Drs. Butovsky and Weiner at BWH, which brings us one step closer to translating our preclinical research into a potentially meaningful treatment for patients with ALS, a devastating disease of the central nervous system," said Jay Hagan, President and Chief Executive Officer of Regulus Therapeutics. "We look forward to reviewing preliminary data later this year, and to further explore the impact of our anti-miR-155 inhibitors in this devastating neurologic disease."

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 Company's preclinical programs, including the expected timing for initiating studies, the expected timing for reporting topline data, and the timing and future occurrence of other preclinical and clinical activities. 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, quarantines, 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 filed annual 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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