Rallybio Announces Positive Preliminary Results for RLYB212, an anti-HPA-1a Monoclonal Antibody for the Prevention of Fetal and Neonatal Alloimmune Thrombocytopenia

September 28, 2022

-- Preliminary data from the ongoing Phase 1b study shows RLYB212 rapidly and completely eliminates transfused HPA-1a positive platelets --
-- Proof-of-Concept Data Expected in 1Q 2023 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Sep. 28, 2022-- Rallybio Corporation (Nasdaq: RLYB) today announced positive preliminary results from its ongoing Phase 1b proof-of-concept study of RLYB212, an anti-HPA-1a monoclonal antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT).

These data demonstrate that one week after a single subcutaneous dose, RLYB212 was able to eliminate transfused HPA-1a positive platelets rapidly compared to placebo in a challenge model of a catastrophic fetal maternal hemorrhage. Additional pharmacokinetic data suggests the opportunity for less frequent dosing.

"These preliminary results continue to support our projected effective target therapeutic concentrations for the prevention of maternal HPA-1a alloimmunization by RLYB212. We are pleased to see rapid and complete elimination of transfused platelets in all subjects to date, with a greater than 90% reduction of the mean platelet elimination half-life compared to placebo, consistent with our proof-of-concept criteria," said Martin Mackay, Ph.D., Chief Executive Officer of Rallybio.

Dr. Mackay added, "We have now commenced dosing under an amended protocol that increases the dose of RLYB212. We expect the broader range of pharmacokinetic and pharmacodynamic data to enable substantive modeling of the concentration-effect relationship that further informs dosing for a future registrational study. We look forward to discussing these proof-of-concept data in the first quarter of 2023."

Consistent with previously reported data, these preliminary results showed acceptable safety and tolerability with no serious adverse events.

About the RLYB212 Phase 1b Study

The ongoing Phase 1b study is a single-blind, placebo-controlled proof-of-concept study designed to establish the ability of subcutaneous RLYB212 to markedly accelerate the elimination of HPA-1a positive platelets transfused to HPA-1a negative healthy male participants. In this study, the elimination of transfused platelets serves as a surrogate for assessing the ability of an anti-HPA-1a antibody to drive rapid elimination of HPA-1a positive fetal platelets from an expectant mother's circulation, thereby potentially preventing HPA-1a maternal alloimmunization and the occurrence of FNAIT in fetuses and newborns. The platelet challenge in this model represents an equivalent fetal maternal hemorrhage of 30 mL, a rare and catastrophic scenario during pregnancy.

Rallybio announced in August 2022 that the Company was amending the protocol to add additional sampling time points and expand the dose range in order to further characterize the absorption and concentration-effect relationship of RLYB212 to platelet elimination. Concomitant PK assessments are included to generate a robust data set of dose-concentration-effect parameters to enable substantive PK/PD modeling for dosing in a future registrational trial.

The study is being conducted at the Clinical Research department of the Fraunhofer Institute for Translational Medicine and Pharmacology ITMP, in Frankfurt/Main, Germany, in collaboration with the Institute of Transfusion Medicine and Immunohaematology, German Red Cross (Deutsches Rotes Kreuz) Blood Transfusion Service Baden-Württemberg-Hessen gGmbH in Frankfurt/Main, Germany.

Rallybio expects to report the Phase 1b proof-of-concept data in the first quarter of 2023.

About FNAIT
Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) is a potentially life-threatening rare disease that can cause uncontrolled bleeding in fetuses and newborns. FNAIT can arise during pregnancy due to an immune incompatibility between an expectant mother and her fetus in a specific platelet antigen called human platelet antigen 1, or HPA-1. There are two predominant forms of HPA-1, known as HPA-1a and HPA-1b, which are expressed on the surface of platelets. Individuals who are homozygous for HPA-1b, meaning that they have two copies of the HPA-1b allele and no copies of the HPA-1a allele, are also known as HPA-1a negative. Upon exposure to the HPA-1a antigen, these individuals can develop antibodies to that antigen in a process known as alloimmunization. In expectant mothers, alloimmunization can occur upon mixing of fetal blood with maternal blood. When alloimmunization occurs in an expectant mother, the anti-HPA-1a antibodies that develop in the mother can cross the placenta and destroy platelets in the fetus. The destruction of platelets in the fetus can result in severely low platelet counts, or thrombocytopenia, and potentially lead to devastating consequences including miscarriage, stillbirth, death of the newborn, or severe lifelong neurological disability in those babies who survive. There is currently no approved therapy for the prevention or treatment of FNAIT.

About Rallybio
Rallybio is a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare diseases. Since its launch in January 2018, Rallybio has built a portfolio of promising product candidates, which are now in development to address rare diseases in the areas of hematology, immuno-inflammation, maternal fetal health, and metabolic disorders. The Company's mission is being advanced by a team of highly experienced biopharma industry leaders with extensive research, development, and rare disease expertise. Rallybio is headquartered in New Haven, Connecticut, with an additional facility at the University of Connecticut's Technology Incubation Program in Farmington, Connecticut. For more information, please visit **www.rallybio.com**.

Forward-Looking Statements

This press release contains forward-looking statements that are based on our management's beliefs and assumptions and on currently available information. In some cases, forward-looking statements can be identified by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements in this press release include, but are not limited to, statements concerning timing of completion of our planned studies for RLYB212, and the timing of the availability of data from such studies. The forward-looking statements in this press release are only predictions and are based largely on management's current expectations and projections about future events and financial trends that management believes may affect Rallybio's business, financial condition and results of operations. These forward-looking statements speak only as of the date of this press release and are subject to a number of known and unknown risks, uncertainties and assumptions, including, but not limited to, our ability to successfully initiate and conduct our planned clinical trials, including the FNAIT natural history study, and the Phase 1 and 1b clinical trials for RLYB212 and the Phase 1 study for RLYB116, and complete such clinical trials and obtain results on our expected timelines, or at all, whether our cash resources will be sufficient to fund our operating expenses and capital expenditure requirements and whether we will be successful raising additional capital, our ability to identify new product candidates and successfully acquire such product candidates from third parties, our ability to integrate RLYB331 into our pipeline, competition from other biotechnology and pharmaceutical companies, and those risks and uncertainties described in Rallybio's filings with the U.S. Securities and Exchange Commission (SEC), including Rallybio's Annual Report on Form 10-Q for the period ended June 30, 2022, and subsequent filings with the SEC. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we are not obligated to publicly update or revise any forward-looking statements contained in this press release, whether as a result of any new information, future events, changed circumstances or otherwise.

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