Rallybio Announces Proof-of-Concept Achieved for RLYB212, a Novel Monoclonal anti-HPA-1a Antibody to Prevent Fetal and Neonatal Alloimmune Thrombocytopenia

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-- RLYB212 Showed Rapid Elimination of Transfused, HPA-1a Positive Platelets in HPA-1a Negative Subjects --

-- Clinical Findings and Safety Profile Consistent with Previously Reported Data; Continue to Support the Potential for RLYB212 as a Prophylactic Treatment for FNAIT --

-- Company Expects to Present Results at a Scientific Conference in 2023 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Mar. 6, 2023-- Rallybio Corporation (Nasdaq: RLYB) today announced that clinical proof-of-concept has been achieved in a Phase 1b study for RLYB212, an anti-HPA-1a monoclonal antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT). Results show that one week after a single subcutaneous dose, RLYB212 was able to rapidly and completely eliminate transfused, HPA-1a positive platelets in HPA-1a negative subjects.

Additional findings from the study show:

- The reduction in mean platelet elimination half-life was greater than 90% in both RLYB212 dose groups compared to placebo and was dose related.
- The broad range of pharmacokinetic and pharmacodynamic data allows substantive modeling to inform dose selection for a future registrational study.
- RLYB212 was observed to be well-tolerated with no serious adverse events reported.

The Company expects to report data from the Phase 1b clinical study of RLYB212 at a scientific conference in 2023.

“Our FNAIT development program has consistently demonstrated the effectiveness of anti-HPA-1a antibodies to rapidly eliminate HPA-1a positive platelets from the circulation of HPA-1a negative subjects,” commented Róisín Armstrong, Ph.D., Rallybio’s RLYB212 Program Lead. “We’ve also established in published nonclinical studies the association between rapid platelet elimination and prevention of HPA-1a alloimmunization, which can lead to negative and potentially life-threatening outcomes in FNAIT. Collectively, these data reinforce our belief on the potential for an anti-HPA-1a antibody to be a viable approach for preventing FNAIT and we look forward to continued advancement of the RLYB212 development program.”

Rallybio also announced today that testing in the multi-dose cohort of its single-center Phase 1 trial in Europe began in the first quarter of 2023. This portion of the Phase 1 study will evaluate safety and pharmacokinetics of RLYB212 based on repeat dosing over 12 weeks in healthy male and female participants. The Company expects results from this cohort of subjects in the fourth quarter of 2023.

Martin Mackay, Ph.D., Chief Executive Officer of Rallybio, stated, “We are very pleased with the progress of our RLYB212 program. Throughout the program, we have carefully laid the groundwork to advance a product candidate that we believe can have a significant impact on the lives of expectant mothers and neonates. RLYB212 exemplifies Rallybio’s enduring commitment to transforming the treatment of rare diseases with little to no therapeutic options.”

Given the favorable development profile of RLYB212 to date, the data generated to date for RLYB212, and the expected manufacturing and supply efficiencies for RLYB212, the Company also announced today that RLYB211, a plasma-derived polyclonal anti-HPA-1a antibody, will not be advanced further in clinical development.

About the RLYB212 Phase 1b Study

Rallybio’s Phase 1b study is a single-blind, placebo-controlled proof-of-concept study designed to establish the ability of subcutaneous RLYB212 to rapidly accelerate the elimination of HPA-1a positive platelets transfused to HPA-1a negative healthy male participants. In this single-center, EU-based 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.

In August 2022, the Company amended the Phase 1b protocol to include a higher dose of RLYB212 and further broaden the range of pharmacokinetic and pharmacodynamic data for RLYB212, enabling substantive modeling of the concentration-effect relationship that can inform dosing for a future registrational study.

The Phase 1b study has been 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.

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 prenatal 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 substance, design and timing of our planned or ongoing studies for RLYB212, the timing of the availability of data from such studies, our expectations regarding reporting of data from such studies, our expectations regarding the usefulness such data, the success of modeling to inform dosing for a future registrational study, our ability to advance RLYB212 into future clinical studies, and the likelihood that Rallybio will be successful in developing RLYB212 as an approach to prevent FNAIT. 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 September 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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