Rallybio Announces Clinical Proof-of-Concept Results for RLYB211, an Anti-HPA-1a Polyclonal Antibody for the Prevention of Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT), Published in the Journal of Thrombosis and Haemostasis

April 6, 2023

-- Data Support Potential Use of an Anti-HPA-1a Antibody as Prophylaxis for FNAIT --
-- In March 2023, Rallybio Announced Clinical Proof-of-Concept Achieved for its Lead Product Candidate, RLYB212, a Monoclonal Anti-HPA-1a Antibody, in the First Quarter of 2023 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Apr. 6, 2023-- Rallybio Corporation (Nasdaq: RLYB) today announced publication of Phase 1 / 2 proof-of-concept results for RLYB211, a polyclonal anti-HPA-1a antibody derived from human plasma for the prevention of FNAIT. FNAIT is a rare and life-threatening bleeding disorder in which maternal alloantibodies directed against fetal platelets can lead to devastating outcomes for the fetus/neonate. There are currently no approved therapies for the prevention or treatment of FNAIT. The findings were published in the Journal of Thrombosis and Haemostasis, the official journal of the International Society on Thrombosis and Haemostasis.

Rallybio's randomized, single-blind, placebo-controlled, study investigated the ability of a single dose of intravenous RLYB211 to eliminate HPA-1a-positive platelets transfused into HPA-1a-negative healthy subjects. The platelet transfusion in this study was selected based on the precedent of historical clinical trial designs to assess the efficacy of anti-D prophylaxis, in which the transfusion of antigen positive cells is designed to mimic a catastrophic fetal maternal hemorrhage. As has been shown with anti-D prophylactic agents, the ability of RLYB211 to drive the rapid and complete elimination of antigen positive cells is a surrogate indicator of its potential to prevent maternal alloimmunization, and therefore FNAIT. In Cohort 1, subjects received RLYB211 or placebo one hour after transfusion of HPA-1a positive platelets and in Cohort 1B subjects received RLYB211 or placebo followed by platelet transfusion one week later. Proof-of-concept was defined as ≥90% reduction in platelet elimination half-life compared to placebo.

The publication stated:

- RLYB211 accelerated the elimination of HPA-1a positive platelets versus placebo, with all subjects meeting proof-of-concept criteria for greater than 90% reduction in platelet elimination half-life compared to placebo.
- Rapid elimination of transfused platelets was evident 7 days after RLYB211 administration, simulating prophylactic administration of an anti-HPA-1a antibody prior to a fetal-maternal bleed.
- RLYB211 was well tolerated and no serious adverse events were reported.

"We are pleased that these clinical results were published in the Journal of Thrombosis and Haemostasis, indicating that RLYB211 markedly accelerated the elimination of HPA-1a-positive platelets. We believe that these data demonstrate the prophylactic potential of anti-HPA-1a antibodies to prevent HPA-1a alloimmunization and occurrence of FNAIT," said Róisín Armstrong, Ph.D., Rallybio's FNAIT Program Lead. "Our FNAIT program continues to advance and as previously announced, we are pleased that an abstract reporting results from the Phase 1b proof-of-concept study for RLYB212, our monoclonal candidate, has been accepted for presentation at the upcoming 31st Congress of International Society on Thrombosis and Haemostasis in June."

Dr. Christof Geisen, Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service, and a key collaborator in the RLYB211 study, stated, "In addition to the rapid elimination of transfused platelets in all subjects, the effect of RLYB211 was also demonstrated when it was administered one week prior to platelet transfusion, suggesting a durable potential treatment effect for HPA-1a-specific antibodies and that FNAIT might safely be prevented by rapidly eliminating platelet antigen from maternal circulation before alloimmunization can occur."

The RLYB211 Phase 1 / 2 results were published in the April issue (Volume 21, Issue 4, Pages 838-849) of the *Journal of Thrombosis and Haemostasis*, https://doi.org/10.1016/j.jtha.2022.11.041.

Given the advantages of small volume subcutaneous dosing of RLYB212 as compared to RLYB211 and the expected manufacturing and supply efficiencies for RLYB212, Rallybio announced in March 2023 that the Company will not continue development of RLYB211.

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 (NASDAQ: RLYB) is a clinical-stage biotechnology company with a mission to develop and commercialize life-transforming therapies for

patients with severe and rare diseases. Rallybio has built a broad pipeline of promising product candidates aimed at addressing diseases with unmet medical need in areas of maternal fetal health, complement dysregulation, hematology, and metabolic disorders. The company has two clinical stage programs: RLYB212, an anti-HPA-1a antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT) and RLYB116, a C5 complement inhibitor with the potential to treat several diseases of complement dysregulation, as well as additional programs in preclinical development.

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 and follow us on LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements that are based on our management's beliefs and assumptions and on information currently available to management. 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 the ability of RLYB212 and RLYB211, and the prophylactic administration of HPA-1a-specific antibodies, to clear platelets and prevent alloimmunization in pregnant women, and the anticipated threshold effect for rapid and complete elimination of HPA-1a positive platelets, the expected progress, results, and plans for RLYB212 and RLYB211, the initiation, 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, and our expectations regarding the usefulness 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 ongoing and future clinical trials for RLYB212 for the prevention of FNAIT, 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, 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-K for the period ended December 31, 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. Moreover, we operate in an evolving environment. New risks and uncertainties may emerge from time to time, and it is not possible for management to predict all risks and uncertainties. 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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