Rallybio Reports Positive Data in Its Clinical Program for the Prevention of Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT)

December 1, 2021

RLYB211, an IV-administered anti-HPA-1a antibody, demonstrates ability to rapidly and safely eliminate HPA-1a mismatched platelets up to 7 days after administration

NEW HAVEN, Conn.--(BUSINESS WIRE)--Dec. 1, 2021-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare diseases, today announced data from its ongoing Phase 1/2 study of RLYB211 showing significant benefit over placebo. RLYB211, a plasma-derived polyclonal anti-HPA-1a antibody, is being evaluated for the prevention of Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT), a potentially life-threatening rare disease that can cause uncontrolled bleeding in fetuses and newborns. There is currently no approved therapy for the prevention or treatment of FNAIT.

Extending previous findings, the new data show that administration of RLYB211 accelerated the elimination of HPA-1ab-positive platelets through 7 days following administration compared with placebo. The study simulated a real-world treatment approach to preventing FNAIT.

“This result reinforces our preventative approach to FNAIT for mothers and babies at-risk for this potentially life-threatening-disease,” said Martin Mackay, Chief Executive Officer of Rallybio. “We are both pleased with the RLYB211 findings and excited to move into the next phase of our FNAIT program by advancing RLYB212.”

These data support Rallybio’s first-in-human clinical trial of its lead candidate RLYB212, a novel human monoclonal anti-HPA-1a antibody which has the same mechanism of action as RLYB211. Data from the Phase 1/2 RLYB212 trial are anticipated mid-year 2022.

“These new data for RLYB211 add to our understanding of how we might best safely prevent FNAIT by rapidly and completely eliminating antigen from circulation before alloimmunization can occur,” said Dr. Christof Geisen, Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service, key collaborator in the RLYB211 study. “With this data to inform future trials this is an important step forward for at-risk families who currently have no approved treatments or therapies to prevent maternal alloimmunization, and therefore the occurrence of FNAIT in babies.”

Consistent with previously reported data, the results from the Phase 1/2 study of RLYB211 cohort 1B showed acceptable safety and tolerability with no serious adverse events. Collectively, the RLYB211 clinical data demonstrate the sustained treatment capacity of anti-HPA-1a antibodies to cause rapid and complete elimination of mismatched platelets in study volunteers.

RLYB211 PHASE 1/2 STUDY DESIGN

The ongoing Phase 1/2 study is a single-blind, placebo-controlled proof-of-concept study designed to establish the dose of RLYB211 that will rapidly clear 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.

In cohort 1B of the ongoing placebo-controlled Phase 1/2 study, HPA-1a negative healthy male participants (n=4) were randomized 3:1 to receive either 1000 IU RLYB211 or placebo (single blind) 7 days prior to administration of HPA-1a positive platelets ($10x10^9$ dose) on Day 1. Platelet elimination was assessed at Days 1, 3, and 7.

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.

Additional information on the RLYB211 Phase 1/2 study is available at clinicaltrialsregister.eu with EudraCT Number 2019-003459-12. Learn more about FNAIT and RLYB211 here. Rallybio intends to report additional data and analyses in a future publication or other peer-reviewed forum.

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.

Our FNAIT Program

RLYB212 and RLYB211 are investigational anti-HPA-1a antibodies in development for the prevention of FNAIT. RLYB211 is a plasma-derived

polyclonal anti-HPA-1a antibody administered via intravenous bolus injection. RLYB212 is a novel human monoclonal anti-HPA-1a antibody administered via subcutaneous injection. Both RLYB211 and RLYB212 are designed to rapidly eliminate fetal HPA-1a positive platelets from the circulation of a mother who is HPA-1a negative and prevent the occurrence of maternal alloimmunization, thereby eliminating the risk of FNAIT in the fetus.

Both product candidates have received Orphan Drug Designations from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency, and Rare Pediatric Disease designations from the FDA.

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, the timing of our clinical trials for RLYB211 or RLYB212, the period during which the results of the trials will become available or announced, and the medical benefits of RLYB211 and RLYB212. 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 1/2 clinical trials for RLYB211 or RLYB212, 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, 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 Quarterly Report on Form 10-Q for the period ended September 30, 2021, and any 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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