Rallybio Announces Preliminary Multiple Dose Data from the Completed Phase 1 Safety and Pharmacokinetics Study for RLYB212, an Anti-HPA-1a Monoclonal Antibody for the Prevention of Fetal and Neonatal Alloimmune Thrombocytopenia

November 28, 2023 at 7:00 AM EST

-- Preliminary Data Support RLYB212 Once Monthly Subcutaneous Dosing in Phase 2 Study --

-- RLYB212 Toxicology Package Complete, Including Maternal-Fetal Toxicology; Key Activity to Support Phase 2 and Phase 3 Studies in Pregnant Women --

-- RLYB212 Phase 2 Study Expected To Commence in 2H 2024 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Nov. 28, 2023-- Rallybio Corporation (Nasdaq: RLYB) today announced preliminary data from the completed multiple dose cohort of the Phase 1 safety and pharmacokinetics (PK) study for RLYB212, an anti-HPA-1a monoclonal antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia.

The Phase 1 multiple dose cohort for RLYB212 was initiated in the first quarter of 2023 to evaluate the safety and PK of subcutaneous (SC) RLYB212 based on repeat dosing over 12 weeks. Eight HPA-1a negative subjects participated in the multiple dose cohort; 6 subjects received RLYB212 every 2 weeks and 2 received placebo every 2 weeks.

The preliminary data demonstrated that multiple dose PK were consistent both within and between subjects. The preliminary data and the Company's clinical pharmacology modeling predictions support a once monthly dosing regimen for the planned Phase 2 study. Consistent with previously reported data, RLYB212 was observed to be generally well-tolerated with no reports of injection site reactions or serious adverse events.

Rallybio also announced today that the RLYB212 toxicology package to support the planned Phase 2 and Phase 3 studies, including the maternal fetal toxicology program, is complete.

"These data support our belief in the potential use of subcutaneous RLYB212 as a prophylactic therapeutic for the prevention of HPA-1a alloimmunization and FNAIT," commented Róisín Armstrong, Ph.D., Rallybio's RLYB212 Program Lead. "We are pleased that the RLYB212 toxicology package is complete, including the maternal fetal toxicology program. The program, which involved a novel animal model, is a key step for Phase 2 and Phase 3 clinical studies in pregnant women. We are now focused on initiating the Phase 2 dose confirmation study in pregnant women at higher risk of FNAIT in the second half of 2024. We thank all of our collaborators and particularly, the Fraunhofer Institute for Translational Medicine and Pharmacology and the German Red Cross, for their support and partnership to complete the Phase 1 study."

Rallybio expects to submit both the RLYB212 clinical pharmacology model and the Phase 2 dosing approach, including supportive clinical and nonclinical data, to a peer reviewed journal in 2024.

About RLYB212 Phase 1 Safety and PK Study

Rallybio's Phase 1 study is a single-blind, placebo-controlled study that investigated the safety and PK of SC RLYB212 in HPA-1a negative healthy participants. The study included two cohorts: a previously completed single dose cohort and a multiple dose cohort. In the multiple dose cohort, subjects received SC RLYB212 or placebo every 2 weeks for 12 weeks.

The Phase 1 study was 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 (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, an inhibitor of complement component 5 (C5), 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 and Twitter.

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. All statements, other than statements of historical facts contained in this press release are forward-looking statements. 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 preliminary multiple dose data from the Phase 1 safety and pharmacokinetics study for RLYB212, the expected dosing regimen for the planned Phase 2 study, whether the completed toxicology package for RLYB212 will be adequate to initiate the planned Phase 2 study, statements concerning the substance, design and timing of our planned or ongoing studies for RLYB212, including the planned Phase 2 and Phase 3 studies, the timing of the availability of data from such studies, our expectations regarding the usefulness of such data, the success of modeling that informs dosing for future studies, whether the RLYB212 clinical pharmacology model and the Phase 2 dosing approach will be published in a peer reviewed journal, 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 studies, and complete such clinical studies 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 enter into strategic partnerships or other arrangements, 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, 2023, 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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Source: Rallybio Corporation