Rallybio Announces Clinical Proof of Concept for RLYB211, an Anti-HPA-1a Antibody for the Prevention of Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT)

July 2, 2021

— Data to be Presented at the International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress

—Demonstrates ability of an anti-HPA-1a antibody to rapidly and completely clear HPA-1a mismatched platelets in a human model of FNAIT

— The mean half-life of mismatched platelets was 0.32 hours in RLYB211-treated participants compared to 65.29 hours in placebo-treated participants (p-value <0.001)

— Data support advancement of Rallybio's lead product candidate, RLYB212, a monoclonal anti-HPA-1a antibody, into a Phase 1 study

NEW HAVEN, Conn., July 2, 2021 – Rallybio, a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare disorders, today announced that clinical proof of concept has been established in the ongoing Phase 1/2 study of RLYB211. RLYB211, a 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. Data from the first cohort of participants demonstrate the ability of an anti-HPA-1a antibody to rapidly and completely clear HPA-1a positive platelets from the circulation of HPA-1a negative participants, providing evidence that administration of an anti-HPA-1a antibody could be a viable approach for the prevention of FNAIT.

Rallybio's FNAIT prevention portfolio includes both RLYB211 as well as the Company's lead product candidate, RLYB212, a subcutaneously administered novel human monoclonal anti-HPA-1a antibody that is expected to enter the clinic in 1Q 2022.

"We are thrilled with the clear signal demonstrated by RLYB211 in this first cohort of participants," said Martin Mackay, Co-Founder and Chief Executive Officer of Rallybio. "These data reinforce our conviction in our approach as we prepare to move RLYB212 into the clinic. We will continue to work expeditiously with our partners, including the Fraunhofer Institute for Translational Medicine and Pharmacology and German Red Cross, to advance a potential preventive treatment to benefit the mothers and babies at high risk of this devastating disease."

In the first cohort of the ongoing Phase 1/2 study, HPA-1a negative healthy male participants were administered either 1000 IU RLYB211 (n=6) or placebo (n=2) 60 minutes after administration of HPA-1a positive platelets. Analysis showed that administration of RLYB211 markedly accelerated the clearance of HPA-1a positive platelets compared with placebo, with a mean half-life of 0.32 hours vs. 65.29 hours, respectively (p-value <0.001). Additionally, RLYB211 was safe and well tolerated, and no serious adverse events were observed. The detailed data will be presented at the International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress taking place July 17-21, 2021.

Abstract #PB0969: Rapid and Complete Clearance of HPA-1a Mismatched Platelets in a Human Model of Fetal and Neonatal Alloimmune Thrombocytopenia by a Hyperimmune Plasma Derived Polyclonal Anti- HPA-1a Antibody
Presenting Author: Dr. Christof Geisen, Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service, Baden-Württemberg-Hessen gGmbh, Frankfurt am Main, Germany

"In this elegant human model of FNAIT, administration of a low dose of an anti-HPA-1a antibody can rapidly and completely clear mismatched platelets from circulation," said Dr. Christoph Geisen, Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service, lead author on the ISTH poster and key collaborator in the RLYB211 study. "If we can clear mismatched fetal platelets from a mother's circulation, then we believe we should be able to prevent maternal alloimmunization and therefore, the occurrence of FNAIT in babies. This would represent a significant step forward for at-risk families where no approved preventive therapy or treatment presently exist."

Administration of RLYB211 and RLYB212 to expectant at-risk mothers is designed to rapidly eliminate fetal HPA-1a positive platelets from a mother's circulation and prevent maternal alloimmunization, thereby eliminating the risk of FNAIT in the fetus.

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.

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.

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 RLYB212 AND RLYB211

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.

Rallybio acquired RLYB211 and RLYB212 from Prophylix AS. 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 focused on identifying and accelerating the development of life-transforming therapies for patients with severe and rare disorders. 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, and metabolism. 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.