Rallybio Announces First-in-Human Dosing in RLYB211 Phase 1/2 Study

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RLYB211 is in Clinical Development for the Prevention of FNAIT, a Potentially Life-Threatening Rare Disorder that Impacts Fetuses and Newborns

NEW HAVEN, Conn., November 10, 2020 – Rallybio, a biopharmaceutical company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare disorders, today announced that dosing has commenced in a Phase 1/2 study evaluating RLYB211, a plasma-derived hyperimmune globulin, in healthy male participants. RLYB211 is in development for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT), a potentially life-threatening rare disorder that can cause uncontrolled bleeding in fetuses and newborns.

FNAIT can occur during pregnancy and is caused by a mismatch between mother and fetus of a specific human platelet antigen (HPA), most commonly HPA-1. This incompatibility can cause an expectant mother to develop antibodies that attack and destroy the platelets of her fetus, which can result in severe thrombocytopenia in the baby, potentially leading to intracranial hemorrhage (ICH), severe life-long neurological disability, and loss of the fetus or newborn. RLYB211 is designed to prevent FNAIT through a mechanism known as antibody-mediated immune suppression (AMIS).

“The initiation of the RLYB211 Phase 1/2 study represents a significant milestone as we work diligently to bring a life-transforming preventative therapy to expectant mothers whose fetuses and newborns are at risk of developing FNAIT,” said Martin Mackay, co-founder and Chief Executive Officer of Rallybio. “Our hearts break for the mothers, babies and families who have been impacted by this terrible disorder, and with RLYB211, we hope to prevent this disease from ever occurring.”

The 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 RLYB211 to elicit AMIS, thereby preventing HPA-1a maternal alloimmunization and the occurrence of FNAIT in the fetuses and the newborns.

The study consists of three cohorts, each investigating a different dose of RLYB211. In Cohorts 1 and 2, platelet transfusion precedes administration with either RLYB211 or placebo. Data from Cohorts 1 and 2 will be used to support the study’s primary endpoint of time to platelet clearance. Data from Cohort 3 will be used to establish the RLYB211 pharmacokinetic profile. Safety will be assessed across all cohorts. The study is being conducted at the Clinical Research department of Fraunhofer Institute for Molecular Biology and Applied Ecology IME, Branch for Translational Medicine and Pharmacology (TMP) in Frankfurt/Main, Germany in collaboration with the German Red Cross (Deutsches Rotes Kreuz) Blood Service Baden-Württemberg-Hessen 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.

About FNAIT

FNAIT is a disorder that can occur during pregnancy and is caused by a mismatch between mother and fetus of a specific human platelet antigen (HPA), most commonly HPA-1. When fetal platelets enter the mother’s circulation, the mother recognizes the fetal HPA-1a as foreign and generates an antibody response, known as alloimmunization. These maternal HPA-1a antibodies then cross the placenta where they attack and destroy the platelets of the fetus, which can result in thrombocytopenia, potentially leading to intracranial hemorrhage (ICH). The consequences of ICH can be devastating, and include miscarriage, stillbirth, or loss of the newborn, as well as severe lifelong neurological disability in those babies who survive. There is currently no approved therapy for the prevention or treatment of FNAIT.

About RLYB211

RLYB211 is an investigational drug in development for the prevention of FNAIT resulting from HPA-1a maternal alloimmunization. RLYB211 is a human hyperimmune globulin derived from the plasma of women who were previously alloimmunized to HPA-1a. This product candidate is designed to prevent FNAIT from occurring in the fetus through antibody-mediated immune suppression of the expectant mother. RLYB211 has received Orphan Drug Designation from both the U.S. Food and Drug Administration (FDA) and from the European Medicines Agency (EMA), and a Rare Pediatric Disease designation by the FDA.

About Rallybio

Rallybio is a biopharmaceutical 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.