Rallybio Announces Proof-of-Concept Results and Development Updates for RLYB212, a Novel Monoclonal anti-HPA-1a Antibody to Prevent Fetal and Neonatal Alloimmune Thrombocytopenia

June 24, 2023

-- RLYB212 Demonstrated Dose-Dependent, Rapid and Complete Elimination of Transfused HPA-1a Positive Platelets in HPA-1a Negative Subjects --

-- Mean Reduction in Platelet Elimination Half-Life After Subcutaneous Administration of RLYB212 was ≥90% in both RLYB212 Dose Groups, Achieving Proof-of-Concept Criteria --

-- Phase 2 Study in Pregnant Women at Higher Risk of HPA-1a Alloimmunization to be Initiated in 2H 2024 --

-- Phase 1 Multiple Dose Data and Maternal-Fetal Toxicology Data On-track for 4Q 2023 --

-- Company to Host Investor and Analyst Meeting Webcast with Corresponding Slides at 4:00pm ET --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jun. 24, 2023-- 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 reported data from the Phase 1b proof-of-concept study of RLYB212, a novel monoclonal anti-HPA-1a antibody in development for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT). The data, delivered in an oral presentation by Christof Geisen, M.D., at the 31st Congress of the International Society on Thrombosis and Haemostasis (ISTH), showed that subcutaneous (SC) RLYB212 administration produced a dose-dependent, rapid and complete elimination of transfused HPA-1a positive platelets in HPA-1a negative subjects, with both doses meeting the prespecified proof-of-concept criteria of ≥90% reduction in mean platelet elimination half-life. Mean platelet elimination half-life was 5.8 hours (0.09mg) and 1.5 hours (0.29mg) for RLYB212 compared to 71.7 hours for placebo.

Christof Geisen, M.D., Institute of Transfusion Medicine and Immunohaematology, German Red Cross Blood Transfusion Service, and the lead author for the RLYB212 abstract stated, “These proof-of-concept results provide further understanding as to how FNAIT may have the potential to be prevented through the rapid and complete elimination of fetal platelet antigens prior to maternal alloimmunization. These data are a critical step forward to address a significant unmet need with no currently approved treatments or therapies to prevent maternal alloimmunization as well as the occurrence of FNAIT in babies.”

Phase 1b Proof-of-Concept (POC) Study of RLYB212 in FNAIT

The Phase 1b single-blind, placebo-controlled proof-of-concept study was designed to establish the ability of SC RLYB212 to rapidly eliminate HPA-1a positive platelets transfused to HPA-1a negative healthy subjects. The study included 11 males aged 18 to 65 years, randomized to RLYB212 0.09mg (n=4), RLYB212 0.29mg (n=5), or placebo (n=2).

Summary of Proof-of-Concept Results

- RLYB212 demonstrated a dose-dependent, rapid and complete elimination of transfused HPA-1a positive platelets in HPA-1a negative subjects.
- Mean platelet elimination half-life was 5.8 hours (0.09mg) and 1.5 hours (0.29mg) for RLYB212 compared to 71.7 hours for placebo, meeting the study’s prespecified criteria for proof-of-concept in both dose groups (≥ 90% reduction in mean platelet elimination half-life).
- The study’s broad range of pharmacodynamic and pharmacokinetic (PK) data will allow substantive modeling of the RLYB212 concentration-effect relationship and inform the target dose regimen for the planned future studies.
- Platelet elimination profiles after SC administration of RLYB212 were consistent with those of Rhesus factor D (RhD)-positive erythrocytes after intramuscular administration of anti-RhD agents, which are well-established to safely and effectively prevent RhD alloimmunization during pregnancy.
- Consistent with previously reported data, RLYB212 was observed to be well-tolerated with no reports of serious or severe adverse events.

“The data reported today at ISTH continue to support our belief in the potential use of subcutaneous RLYB212 as a prophylactic therapeutic for the prevention of HPA-1a alloimmunization and FNAIT. We are pleased to see rapid and complete platelet elimination in our target concentration range, with both dose groups meeting the proof-of-concept criteria of at least 90% reduction in mean platelet elimination half-life,” commented Róisín Armstrong, Ph.D., Rallybio’s RLYB212 Program Lead. “With no currently approved therapies for the prevention of FNAIT, there remains a substantial unmet need for a treatment that can effectively eliminate fetal platelet antigen and, as a result, significantly decrease the risk of severe bleeding in the fetus and neonate, including intracranial hemorrhage and its devastating consequences.”

Dr. Armstrong continued, “We continue to focus our efforts on completing 12-week repeat dosing in the Phase 1 safety and PK study of RLYB212, along with the ongoing toxicology program, both of which are on track for the fourth quarter of 2023. In the second half of 2024 we plan to commence a Phase 2 study in pregnant women at higher risk of HPA-1a alloimmunization, designed to confirm the RLYB212 dose regimen for our Phase 3 registrational study. We are also planning for discussions with regulators later in 2023 or in the first half of 2024 in advance of the Phase 2 study.”

Clinical Development Update for RLYB212

Rallybio will host an investor and analyst meeting beginning at 4:00 p.m. ET to discuss clinical development plans for RLYB212.

RLYB212 Catalysts for 2023

- Rallybio remains on track to complete the following RLYB212 milestones in the fourth quarter of 2023:
  - Comprehensive toxicology program, including maternal-fetal toxicology
  - Multiple dose cohort of Phase 1 safety and PK study

Phase 2 Dose Confirmation Study

- The Company plans to initiate a Phase 2 dose confirmation study in the second half of 2024, designed to confirm the RLYB212 dose regimen in pregnant women at higher risk of FNAIT prior to initiation of a larger Phase 3 registrational study.
- This study will employ a sentinel, sequenced cohort design to allow for any required adjustments to the dose regimen, prior to advancing the confirmed dose regimen into the registrational study.

Natural History Study

- The Natural History Study is designed to provide a contemporary dataset for HPA-1a alloimmunization frequency in a racially and ethnically diverse population that can serve as a control for the planned single-arm registrational study, along with establishing the operational framework for seamless initiation of the Phase 2 study and future Phase 3 registrational study.
- The Natural History Study has screened approximately 4,500 women to date and an estimated 7,600 women are planned to be screened by end of 2023.
- Screening for the Natural History Study is expected to continue simultaneously with execution of the Phase 2 study; data from both studies will be used for end of Phase 2 regulatory discussions with the U.S. Food and Drug Administration and the European Medicines Agency to support design and initiation of the Phase 3 registrational study.

Phase 3 Registrational Study

- Following completion of the Phase 2 dose confirmation study, Rallybio expects to commence a Phase 3 registrational study, after consultation with regulatory authorities.

Investor and Analyst Meeting Webcast

Rallybio will host an investor and analyst meeting on Saturday, June 24, 2023 from 4:00 to 6:00 p.m. Eastern Time. The webcast and corresponding slides can be accessed through the Events and Presentations section of Rallybio's website at http://www.rallybio.com.

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, 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 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 results from the RLYB212 Phase 1b proof-of-concept study, statements concerning the substance, design and timing of our planned or ongoing studies for RLYB212, including the planned Phase 2 study and Phase 3 registrational study, the timing of the availability of data from such studies, our expectations regarding reporting of data from such studies, our expectations regarding the usefulness of such data, the success of modeling to inform dosing for a future registrational study, our ability to advance RLYB212 into future clinical studies, the outcomes of discussions with healthcare authorities, including the FDA, 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, including the FNAIT natural history study, the Phase 1b clinical study for RLYB212, and our planned Phase 2 and Phase 3 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 March 31, 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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