Rallybio Presents Natural History Study for Fetal and Neonatal Alloimmune Thrombocytopenia at the 65th American Society of Hematology Annual Meeting

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-- FNAIT Natural History Study to Inform Frequency of FNAIT Risk in a Broad and Diverse Population of Pregnant Women --

-- Study Designed to Provide a Contemporary Control Dataset to Support a Future Registrational Trial in Pregnant Women --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Dec. 9, 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 presented details from the Rallybio Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) natural history study in a poster presentation at the 65th American Society of Hematology (ASH) Annual Meeting, in San Diego, California. The FNAIT natural history study is a prospective, non-interventional, multinational alloimmunization study to determine the frequency of expectant mothers of diverse races and ethnicities at higher FNAIT risk.

“There is a significant unmet need to establish routine maternal blood screening to identify women at higher risk of FNAIT and provide a well-tolerated, effective prophylactic treatment. Screening mothers for FNAIT during pregnancy is not routinely performed and currently most at-risk pregnancies are not identified prior to birth,” said Emilie Vander Haar, M.D., Weill Cornell Medicine, New York-Presbyterian Hospital and co-author. “By analogy, the introduction of antenatal screening in Rh disease combined with an effective prophylactic therapy has resulted in the virtual elimination of that disease and is one of the most significant medical advances ever achieved. Achieving the same in FNAIT would have lasting implications for expectant moms and their babies.”

Previous FNAIT studies primarily conducted in Caucasian populations suggest that approximately 2% of expectant women are HPA-1a negative and, therefore, at risk for FNAIT. This FNAIT natural history study is the first prospective study that seeks to characterize risk for FNAIT in a racially and ethnically diverse population of pregnant women.

The study is presently being conducted in the United States and across multiple European countries including Germany, Netherlands, Norway, Sweden, and the United Kingdom. Expectant mothers are screened at gestational weeks 10 to 14, enabling early identification and follow-up of women at higher risk of alloimmunization. Rallybio expects to screen up to 30,000 expectant mothers in the natural history study, with an estimated 7,600 women screened by the end of 2023.

Screening for the natural history study is expected to continue in parallel with Rallybio’s Phase 2 study of RLYB212, an anti-HPA-1a monoclonal antibody for the prevention of FNAIT. The Phase 2 study is expected to initiate in the second half of 2024. Data from the natural history study is designed to provide a contemporary control for the planned single-arm Phase 3 registrational study.

The poster can be viewed on the Publications & Presentations page of the Company’s website www.rallybio.com.

Additional information about the ASH Annual Meeting is available at: https://www.hematology.org/meetings/annual-meeting.

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 results from the FNAIT natural history study, expectations regarding the use of such results, the estimated number of expectant mothers screened in the natural history study, and whether a prophylactic treatment for FNAIT can be successfully developed. 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 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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