Rallybio Announces Results of Epidemiological Analysis Demonstrating FNAIT Risk Across Racially and Ethnically Diverse Populations

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– First Reported Large-Scale Analysis of FNAIT Risk Across Broad Population of Diverse Ancestries –

– Topline Results Suggest a Significant Increase in the Number of Pregnancies at Higher Risk for FNAIT Annually and the RLYB212 Addressable Market –

– Rallybio On Track to Initiate RLYB212 Phase 2 Dose Confirmation Study in Pregnant Women at Higher Risk for FNAIT in 2H 2024 –

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jun. 17, 2024-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, today reported topline results from an epidemiological analysis of large genomic datasets evaluating the frequency of fetal and neonatal alloimmune thrombocytopenia (FNAIT) risk in diverse ancestries. Data from this analysis confirmed Caucasian populations as having the greatest proportion of women with the genetic markers for higher FNAIT risk (HPA-1a negative, HLA-DRB3*01:01 positive). Additionally, data from this analysis provides, for the first time, robust evidence quantifying the proportion of women in non-Caucasian ancestries that carry the genetic markers for higher FNAIT risk.

These data indicate that the proportion of pregnant women at higher risk for FNAIT annually has been significantly underestimated. For example, in key geographies of North America and major European countries, it is estimated that more than 30,000 pregnancies each year are at higher risk for FNAIT, representing a 40% increase from prior estimates. Full data from the epidemiological analysis are expected to be presented at a scientific conference in the fourth quarter of 2024.

“This epidemiological analysis leverages the availability of large-scale, diverse genomic datasets, and provides the first clear evidence of the extent to which ancestries beyond the Caucasian population can carry a higher risk for FNAIT,” said Stephen Uden, MD, Chief Executive Officer of Rallybio. “These data indicate that FNAIT risk is more prevalent than previously estimated and highlights the importance of screening. Unlike many other rare diseases, we believe that screening for FNAIT risk could be seamlessly incorporated into standard prenatal care for all mothers. This would ensure all women at higher risk for FNAIT could then be offered prophylactic treatment with RLYB212, if approved.”

Dr. Uden continued, “Based on this new information, we now believe RLYB212 represents a commercial opportunity of greater than $1.6 billion. We thank HealthLumen for their partnership in this endeavor to expand our understanding of FNAIT risk across diverse ancestries.”

Topline results from this analysis demonstrate that the proportion of women at higher risk of alloimmunization and FNAIT was greatest in European Caucasian populations, consistent with published literature. Additionally, the results demonstrate a comparable proportion of women at higher risk in Hispanic White populations, as well as a relatively moderate proportion of women at higher risk in Hispanic Black and Non-Hispanic Black populations. Proportions of women at higher risk in South Asian, East Asian, and Amerindigenous populations were found to be low relative to the European Caucasian populations.

The analysis was conducted by Rallybio in partnership with HealthLumen, a leader in epidemiological modeling of rare genetic diseases. Allele frequencies for HPA-1a were obtained from gnomAD v4, which contains exome and genome sequencing data from approximately 810,000 individuals. Allele frequencies for HLA DRB3*01:01, which is associated with approximately 25-fold higher risk of alloimmunization, were obtained from the US National Marrow Donor Registry (NMDR), which includes data from approximately 2,700,000 individuals.

Rallybio is developing RLYB212, a novel human monoclonal anti-HPA-1a antibody, to prevent alloimmunization in pregnant women and thereby, eliminate the risk of FNAIT and its potentially devastating consequences in their fetuses and newborns. Rallybio expects to initiate a Phase 2 dose confirmation study of RLYB212 in pregnant women at higher risk for HPA-1a alloimmunization and FNAIT in the second half of 2024. The Company is also conducting a non-interventional FNAIT natural history study that is designed to provide a contemporary dataset for HPA-1a alloimmunization frequency in a racially and ethnically diverse population. As of May 1, 2024, Rallybio had screened approximately 10,000 pregnant women.

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 HPA-1a-negative expectant mothers bearing a HPA-1a-positive fetus, 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. 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 diversity of ancestries that carry the genetic markers for FNAIT, the increase in the number of pregnancies estimated to be at high risk of FNAIT each year based on the epidemiological analysis and our estimates of the number of pregnancies at higher risk of FNAIT, our ability to identify the number of pregnant women at higher risk of FNAIT based on the results of the analysis, our ability to ensure routine prenatal screening, our estimates of the market opportunity for RLYB212, the timing of initiation of the Phase 2 dose confirmation study for RLYB212, our expectations regarding the usefulness of data from our clinical studies, and the timing of publications relating to FNAIT and RLYB212, . 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 trials, including the FNAIT natural history study, and the Phase 2 clinical trial for RLYB212, and complete such clinical trials 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, 2024, 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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