Rallybio to Discontinue Development of RLYB212 for Prevention of FNAIT

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– RLYB212 Phase 2 PK Results Did Not Achieve Target Concentrations, Including Minimum Target Concentration Required for Efficacy –

– RLYB116 Confirmatory PK/PD Study to Initiate in 2Q 2025, with Data in 2H 2025 –

NEW HAVEN, Conn.--(BUSINESS WIRE)--Apr. 8, 2025-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, today announced the discontinuation of the RLYB212 program for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT). The Company's decision to discontinue RLYB212 development was based on pharmacokinetic (PK) data from the Phase 2 clinical trial demonstrating the inability of the RLYB212 dose regimen to achieve predicted target concentrations, as well as the minimum target concentration required for efficacy. Rallybio remains focused on advancing RLYB116, a once-weekly low volume C5 inhibitor for the treatment of complement-driven diseases, as well as its emerging preclinical programs.

"We are disappointed by the PK results of the RLYB212 Phase 2 trial," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "Given that the results significantly deviated from the predicted range and the absence of empiric data to further inform dose adjustment, the risk/benefit no longer supports continued dosing, and we will discontinue RLYB212 development. We are grateful to the participants, investigators, and study staff for their partnership and dedication to this program."

Dr. Uden continued, "Rallybio remains steadfast in our mission to develop transformative therapies. We are focused on creating shareholder value by advancing our portfolio of potentially best-in-class assets for patients with rare diseases, which includes RLYB116 and REV102, an ENPP1 inhibitor for patients with hypophosphatasia, as well as RLYB332, a long-acting matriptase-2 antibody for diseases of iron overload."

RLYB212 Phase 2 Trial

The single-arm Phase 2 dose confirmation trial was designed to assess the PK and safety of RLYB212 in pregnant women at higher risk for HPA-1a alloimmunization and FNAIT. Secondary objectives included the assessment of pregnancy and neonatal/infant outcomes, and the occurrence of emergent HPA-1a alloimmunization.

Second trimester PK results from the sentinel participant demonstrated an inability of RLYB212 to achieve predicted target concentrations of 6 ng/mL to 10 ng/mL, as well as the minimum target concentration required for efficacy of 3 ng/mL, with values near or below the assay's lower limit of quantitation. Dose adjustment is not deemed feasible given that PK levels are meaningfully outside the predicted range and the absence of empiric data to inform an adjustment. It is hypothesized that HPA-1a antigen expression on the placenta may be impacting plasma concentrations of RLYB212. No further enrollment in the trial is planned and all screening of participants has been stopped. The Company will continue safety follow-up of the sentinel participant as specified in the clinical trial protocol.

Promising Rare Disease Pipeline to Deliver Data in 2H 2025

RLYB116 Program

Rallybio remains on track to initiate dosing in the RLYB116 confirmatory clinical pharmacokinetic/ pharmacodynamic (PK/PD) study in the second quarter of 2025, with data readouts from Cohorts 1 and 2 expected in the third and fourth quarter of 2025, respectively. The study is expected to demonstrate complete and sustained complement inhibition with improved tolerability of RLYB116.

RLYB116 is a novel antibody mimetic fusion protein designed to inhibit C5 and to provide a once-weekly, small volume, subcutaneously injected therapy to meet patient demand for a convenient, self-administered at-home solution. RLYB116 has the potential to address significant unmet need for patients across a number of complement mediated diseases, including paroxysmal nocturnal hemoglobinuria (PNH), antiphospholipid syndrome (APS), and generalized myasthenia gravis (gMG), which together represent a commercial opportunity of more than $6 billion. Importantly, this strategic focus on complement-mediated diseases presents a unique opportunity for Rallybio to utilize its team's deep domain experience in successfully designing, developing, and delivering complement inhibitors for patients with rare diseases.

Preclinical Programs

REV102, an ENPP1 inhibitor for the treatment of patients with HPP under development through a joint venture with Recursion Pharmaceuticals, entered investigational new drug application (IND)-enabling studies in the first quarter of 2025 to support the initiation of a Phase 1 study in 2026. Data evaluating REV102 in a preclinical model of later-onset HPP is expected in the second half of 2025.

Rallybio's portfolio also includes RLYB332, a long-acting, monoclonal anti-matriptase-2 antibody that has the potential to be a best-in-class treatment for diseases of iron overload. Preclinical data has demonstrated superior impact on PD parameters relative to comparator molecules, including on serum iron, unsaturated iron binding capacity (UIBC), and transferrin saturation (TSAT). The Company is preparing plans for future development of RLYB332.

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 pipeline of promising product candidates aimed at addressing diseases with unmet medical need in areas of complement dysregulation, hematology, and metabolic disorders. The Company's lead program, RLYB116, is a differentiated C5 inhibitor with the potential to treat diseases of complement dysregulation. Rallybio also has two programs in preclinical development, including REV102, an ENPP1 inhibitor for the treatment of patients with hypophosphatasia (HPP), and RLYB332, a long-acting matriptase-2 antibody for the

treatment of diseases of iron overload. 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 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 timing of initiating the RLYB116 confirmatory PK/PD study and the date when data is available, including data for Cohorts 1 and 2, whether the PK/PD confirmatory study will demonstrate improved tolerability and sustained inhibition of terminal complement, whether RLYB116 will be effective in treating a broad range of complement-mediated diseases, the potential commercial opportunity for RLYB116, and the timing of initiation of a REV102 Phase 1 study. 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 RLYB116 PK/PD confirmatory study, 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, 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 Annual Report on Form 10-K for the period ended December 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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