Rallybio Highlights 2024 Accomplishments and Anticipated Milestones for 2025

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– Dosing of Sentinel (First) Participant in RLYB212 Phase 2 Clinical Trial Expected in 2Q 2025 –

– Initiation of RLYB116 Confirmatory PK/PD Study Expected in 2Q 2025, with Data Anticipated in 2H 2025 –

– REV102, an ENPP1 Inhibitor for the Treatment of Hypophosphatasia, to Enter IND-Enabling Studies in 2025 –

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jan. 10, 2025-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, today highlighted its 2024 accomplishments and announced its anticipated key milestones for 2025.

“With our team’s innovation and execution throughout 2024, Rallybio is well positioned to create meaningful value in 2025,” said Stephen Uden, M.D., Chief Executive Officer of Rallybio. “We are planning for key readouts from our RLYB212 and RLYB116 clinical programs and expecting important advancements in our REV102 program throughout the year. We believe these developments will provide further evidence of the value our programs can ultimately bring to patients. With these accomplishments and continued financial discipline, the Company is poised for significant momentum in 2025 and even greater success in the years to come.”

2024 Accomplishments

RLYB212

- Obtained clinical trial application approvals and initiated the first-ever Phase 2 dose confirmation trial in pregnant women at higher risk for HPA-1a alloimmunization and fetal and neonatal alloimmune thrombocytopenia (FNAIT)
- Screened more than 14,000 pregnant women through January 1, 2025 in the Company’s ongoing FNAIT natural history study
- Presented results of an epidemiological analysis demonstrating FNAIT risk across racially and ethnically diverse populations, indicating that more than 30,000 pregnancies each year are at higher risk for FNAIT, at the NORD Summit and ASHG
- Published Phase 1b proof-of-concept results, in addition to the modeling and simulations that support the RLYB212 dose regimen for the Phase 2 trial

RLYB116

- Successfully completed manufacturing process enhancements, which are expected to further improve the tolerability of RLYB116
- Presented biomarker characterization analyses indicating that RLYB116 led to a greater degree of complement inhibition in the Phase 1 MAD study than initially reported

REV102

- Advanced REV102, an ENPP1 inhibitor for the treatment of patients with hypophosphatasia (HPP) which was discovered in partnership with Recursion Pharmaceuticals
- Presented data at ASBMR from an early lead ENPP1 inhibitor, REV101, in a mouse model of later-onset HPP demonstrating a 30% reduction in inorganic pyrophosphate (PPi), a key biomarker that is elevated in HPP and contributes to poor bone mineralization

RLYB332

- Presented preclinical data for RLYB332 at ASH, including favorable pharmacodynamic (PD) data, supporting RLYB332 as a long-acting, potentially best-in-class therapy for the treatment of diseases of iron overload

FNAIT Natural History Study Update

Rallybio plans to conclude screening in the FNAIT natural history study in the United States and Canada as of January 31, 2025. The Company will focus resources on advancing the RLYB212 Phase 2 trial across sites in Europe, including initiation of dosing in the sentinel participant and collection of natural history data in a sub-study of the Phase 2 trial. Participants identified as having higher risk for HPA-1a alloimmunization and FNAIT in the ongoing Phase 2 trial who do not receive RLYB212 are eligible to enroll in the natural history sub-study of the Phase 2 trial.

Anticipated 2025 Key Milestones

RLYB212

- Initiate dosing of sentinel participant in the Phase 2 trial in the second quarter of 2025
- Present interim data from the FNAIT natural history study in mid-2025
- Report interim safety and pharmacokinetic (PK) data from the Phase 2 trial sentinel participant in the third quarter of 2025
- Completion of pregnancy, with safety and PK data readout, from the Phase 2 trial sentinel participant in the fourth quarter of 2025

RLYB116

- Initiate confirmatory clinical PK/PD study in the second quarter of 2025
- Cohort 1 data readout from clinical PK/PD study in the third quarter of 2025
- Cohort 2 data readout from clinical PK/PD study in the fourth quarter of 2025

REV102

- Initiate investigational new drug application (IND)-enabling studies in 2025 to support the initiation of a Phase 1 study in 2026
- Report REV102 data from a preclinical model of later-onset HPP in the second half of 2025

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 needs 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 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. 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 dosing the sentinel participant in the RLYB212 Phase 2 trial, the timing of initiating the RLYB116 confirmatory PK/PD study and the date when data is available, including data for Cohorts 1 and 2, the timing of initiation of IND-enabling activities for REV102, whether anticipated 2025 milestones will result in meaningful value appreciation, whether RLYB116 will demonstrate improved tolerability at higher doses with complete and sustained complement inhibition, the timing of disclosure and characterization of preclinical data for REV102, the timing of completion of pregnancy, and data readout, for the sentinel participant for the RLYB212 Phase 2 trial, the timing of data releases for the Company's programs, including interim data from the FNAIT natural history study, interim safety and pharmacokinetic data from the sentinel participant in the RLYB212 Phase 2 trial, and REV102 data from a preclinical model of later-onset HPP. 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 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 September 30, 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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