Rallybio to Initiate RLYB116 Confirmatory Clinical PK/PD Study in Second Quarter 2025

December 2, 2024 at 8:00 AM EST

-- New Biomarker Characterization Analyses Indicate RLYB116 Produced Complete and Sustained Complement Inhibition in Previous Phase 1 MAD Study –

-- Manufacturing Process Enhancements Expected to Further Improve RLYB116 Tolerability --

-- Webcast Today at 8:30 AM Eastern Time --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Dec. 2, 2024-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, today announced results of new biomarker characterization analyses and data from the recently completed manufacturing process enhancements for RLYB116, the Company's innovative, once-weekly, small volume, subcutaneously injected C5 inhibitor in development for the treatment of patients with complement-mediated diseases. Based on this recently completed work, Rallybio believes that RLYB116 has the potential to be a best-in-class C5 inhibitor and intends to initiate a confirmatory clinical pharmacokinetic/pharmacodynamic (PK/PD) study in the second quarter of 2025.

"Recently completed efforts to better understand the disconnect between the compelling preclinical potency data of RLYB116 and the results of the Phase 1 study indicate that RLYB116 achieved greater complement inhibition in the Phase 1 study than initially reported," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "This, together with successful manufacturing process enhancements to further improve the tolerability profile, uniquely positions RLYB116 as a potentially best-in-class C5 inhibitor that can meet patient demand for a once-weekly, small volume, subcutaneous, self-administered therapeutic. We look forward to bringing RLYB116 into a confirmatory clinical PK/PD study in the second quarter of 2025 and expect RLYB116 to demonstrate complete and sustained complement inhibition and an improved tolerability profile."

Following the completion of the RLYB116 Phase 1 single- and multiple-ascending dose (SAD/MAD) study in the fourth quarter of 2023, Rallybio conducted a series of biomarker characterization analyses. These analyses indicate the RLYB116 assay used to measure free C5 overestimated the levels of free C5 by approximately ten-fold, indicating that RLYB116 produced greater complement inhibition than initially reported.

Additionally, Rallybio completed manufacturing process enhancements in the third quarter of 2024 that are expected to further improve the tolerability of RLYB116. Based on the results of enhanced analytical techniques, including mass spectrometry, process enhancements have successfully further purified the RLYB116 drug substance. As a result, RLYB116 is expected to have a favorable tolerability profile at doses at and above those evaluated in the Phase 1 MAD study.

Rallybio plans to initiate a RLYB116 confirmatory clinical PK/PD study in the second quarter of 2025 to demonstrate improved tolerability as well as complete and sustained complement inhibition. This single-blind multiple ascending dose study will evaluate a 4-week treatment duration that will include two cohorts of 8 participants each. It is planned that Cohort 1 will evaluate weekly dosing of 150 mg and Cohort 2 will evaluate weekly dosing of 225 mg. Follow-up will continue for 10 weeks after the conclusion of treatment.

Webcast Information
The live webcast will be accessible through the Events and Presentations section of Rallybio's investor relations website at https://investors.rallybio.com. A replay and accompanying slides of the webcast will be available on the Rallybio website for 30 days following the event.

About RLYB116 Phase 1 Study
RLYB116 is an innovative, once-weekly, small volume, subcutaneously injected C5 inhibitor in development for the treatment of patients with complement-mediated diseases. A Phase 1 single- and multiple-ascending dose (SAD/MAD) study was conducted in healthy participants. The MAD portion of the study had an adaptive single-blind design with a 4-week treatment duration and 10-week follow-up period. The study was designed to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of RLYB116 and included 4 cohorts: Cohort 1 (weekly dosing of 100 mg), Cohort 2 (3 doses of 100 mg the first week followed by weekly dosing), Cohort 3 (150 mg weekly dosing reduced to 125 mg weekly dosing), and Cohort 4 (75 mg twice the first week followed by 100 mg twice per week). The study was completed in the fourth quarter of 2023.

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 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 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 timing of initiation of the planned RLYB116 confirmatory clinical PK/PD study, the expected dosing for RLYB116 in future clinical trials and whether RLYB116 will produce complete and sustained inhibition of C5, the frequency of administration of RLYB116, whether RLYB116 will be a

best-in-class C5 inhibitor, whether the RLYB116 manufacturing process enhancements will improve tolerability or be successful at the desired doses, and our characterization of the actual level of complement inhibition delivered by RLYB116. 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 confirmatory clinical PK/PD study, 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 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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