Rallybio Completes Dosing of First Cohort in RLYB116 Phase 1 Confirmatory Pharmacokinetic/Pharmacodynamic Study

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– Interim Data Support Advancing RLYB116 as a Differentiated Therapeutic and Enable Progression to Cohort 2 –

– Data Readouts for Completed Study Expected in 4Q 2025 –

NEW HAVEN, Conn.--(BUSINESS WIRE)--Sep. 25, 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 completion of dosing of the first cohort in the Phase 1 confirmatory pharmacokinetic/pharmacodynamic (PK/PD) study evaluating RLYB116, the Company's innovative, once-weekly, small volume, subcutaneously injected C5 inhibitor.

"We are encouraged by the data generated to date in this confirmatory multiple ascending dose study and these results reinforce our confidence in the RLYB116 program," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "We have completed the dosing of cohort 1 and the pharmacokinetic and pharmacodynamic profile is consistent with our predictions based on the previous Phase 1 study. More importantly, we have observed a significantly cleaner safety profile. We believe the improved tolerability is due to manufacturing enhancements and supports dose escalation to cohort 2. We look forward to sharing the data from this study in the fourth quarter of 2025."

The single-blind multiple ascending dose Phase 1 confirmatory PK/PD study of RLYB116 (NCT06797375) is designed to demonstrate complete and sustained complement inhibition with favorable tolerability in healthy volunteers. The study is evaluating a 4-week treatment duration that includes two cohorts of eight participants each, randomized 3 to 1 to receive either RLYB116 or placebo once weekly. Cohort 1 evaluated dosing of 150 mg and Cohort 2 will evaluate dosing of up to 300 mg. The study includes a 10-week follow-up period after the conclusion of treatment. In June 2025, Rallybio announced that the initial indication focus for RLYB116 will be on two hematologic conditions with significant unmet need: immune platelet transfusion refractoriness (PTR) and refractory antiphospholipid syndrome (APS). These indications represent a combined market opportunity of $5 billion.

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 and hematology. The Company's lead program, RLYB116, is a differentiated C5 inhibitor with the potential to treat diseases of complement dysregulation, with an initial focus on immune platelet transfusion refractoriness (PTR) and refractory antiphospholipid syndrome (APS). Rallybio's pipeline also includes RLYB332, a preclinical 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.

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 RLYB116 confirmatory PK/PD study, including the timing of topline data for the study, and the potential commercial opportunity for 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 conduct the RLYB116 PK/PD confirmatory study, and complete such study 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 those risks and uncertainties described in Rallybio's filings with the U.S. Securities and Exchange Commission (SEC), including the Quarterly Report on Form 10-Q for the period ended June 30, 2025, 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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