Rallybio Initiates Dosing in RLYB116 Phase 1 Confirmatory Pharmacokinetic/Pharmacodynamic Study

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– Potential to Address a Broad Range of Complement-Mediated Diseases with Initial Focus on Immune Platelet Transfusion Refractoriness and Refractory Antiphospholipid Syndrome –

– Data Readouts Expected from Cohort 1 in 3Q 2025 and Cohort 2 in 4Q 2025 –

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jun. 12, 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 initiation of dosing in a Phase 1 confirmatory pharmacokinetic/pharmacodynamic (PK/PD) study evaluating RLYB116, the Company's innovative, once-weekly, small volume, subcutaneously injected C5 inhibitor.

Additionally, 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). Patients suffering from these potentially life-threatening conditions have no approved or effective therapeutic options.

"The initiation of dosing in our confirmatory PK/PD study of RLYB116 marks a significant step forward in our mission to transform care for patients with immune PTR and refractory APS," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "These are serious, underserved hematologic conditions that demand bold innovation. With RLYB116, we are advancing a potential first-in-class therapy that represents a combined market opportunity of $5 billion. Our goal is to set a new standard of care and bring real hope to patients who have no effective treatment options. We look forward to sharing study results later this year."

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 will evaluate a 4-week treatment duration that will include two cohorts of eight participants each, randomized 3 to 1 to receive either RLYB116 or placebo once weekly. Cohort 1 will evaluate 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.

About Platelet Transfusion Refractoriness

PTR is a clinical condition in which patients fail to achieve the expected rise in platelet count following two or more consecutive transfusions. PTR is an urgent clinical event that increases bleeding risks and can complicate the management of conditions including bone marrow failure, hematologic malignancies, and chemotherapy treatment. The cause of PTR may be either immune or non-immune, with an immune response implicated in up to 40% of cases[1]. In patients with immune PTR, antibodies against platelet antigens, most commonly HLA antigens, bind to transfused platelets and target them for clearance. Evidence suggests that the complement pathway can play an important role in the clearance of platelets in patients with immune PTR. Patients who have received multiple platelet transfusions, commonly as a result of a hematologic malignancy, chemotherapy treatment, and/or transplant, are at highest risk of developing immune PTR.

About Antiphospholipid Syndrome

APS is a rare autoimmune disease characterized by recurrent vascular thrombosis (arterial and/or venous) and/or pregnancy-related complications (such as fetal loss), in the presence of persistent antiphospholipid antibodies. APS can be classified as primary APS or secondary APS. Primary APS occurs without any other underlying autoimmune disease, while secondary APS arises in association with other autoimmune diseases, such as systemic lupus erythematosus. The disease is driven by the production of pathogenic antiphospholipid (aPL) antibodies, including lupus anticoagulant (LA), anticardiolipin (aCL) antibodies, and anti-β2 glycoprotein-I (aβ2GPI) antibodies. These antibodies promote thrombosis and can lead to serious complications, such as stroke, ischemic attacks, deep vein thrombosis, and pulmonary embolism. Increasing evidence implicates complement system overactivation as a key contributor to both the pathogenesis and clinical manifestations of APS[2-4]. It is estimated that approximately 10% of patients with APS are refractory and continue to experience thromboses despite anticoagulant treatment.

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.

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 potential of RLYB116 to address a broad range of complement-mediated diseases, including immune platelet transfusion refractoriness and refractory antiphospholipid syndrome, the expected timing of data readouts from the RLYB116 confirmatory PK/PD study, whether RLYB116 will be an effective treatment option for immune PTR and APS, whether the PK/PD confirmatory study will demonstrate improved tolerability and sustained inhibition of terminal complement, and the potential commercial opportunity for RLYB116 for the treatment of immune PTR and APS. 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 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 the Quarterly Report on Form 10-Q for the period ended March 31, 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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2. Chaturvedi, S., Brodsky, R.A., McCrae, K.R. Complement in the Pathophysiology of the Antiphospholipid Syndrome. Front. Immunol. 2019 Mar;10:449.
3. Chaturvedi, S., Braunstein, E.M., Yuan, X., et al. Complement activity and complement regulatory gene mutations are associated with thrombosis in APS and CAPS. Blood. 2020;135(4):239-251.
4. Chaturvedi, S., Braunstein, E.M., Brodsky, R.A. Antiphospholipid syndrome: Complement activation, complement gene mutations, and therapeutic implications. J Thromb Haemost. 2021 Mar;19(3):607-616.

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Investor Contacts
Samantha Tracy
Rallybio Corporation
(475) 47-RALLY (Ext. 282)
investors@rallybio.com

Kevin Lui
Precision AQ
(212) 698-8691
kevin.lui@precisionaq.com

Media Contact
media@rallybio.com

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