Rallybio Highlights Portfolio Advances and Outlines Expected Upcoming Milestones for 2022

January 6, 2022

-- First subjects dosed in Phase 1 study of RLYB212; proof-of-concept data from subsequent Phase 1b study expected 3Q 2022 --

-- On-track to initiate Phase 1 study of RLYB116 in 1Q 2022; single dose safety, PK and PD data expected 2H 2022 --

-- Advancing preclinical development of ENPP1 inhibitor; IND-enabling studies expected to initiate in 2H 2022 --

-- Rallybio to present these updates at 40th Annual J.P. Morgan Healthcare Conference --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jan. 6, 2022-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare diseases, today provided an update on recent accomplishments and announced expected upcoming milestones. The Company will present these updates at the 40th Annual J.P. Morgan Healthcare Conference on Thursday, January 13, 2022 at 11:15 a.m. ET.

"Our mission is to build Rallybio into a leading biotechnology company, with a broad and sustainable pipeline of product candidates that can transform the lives of people with severe and rare disorders and shatter expectations of what's possible," said Martin Mackay, Ph.D., Chief Executive Officer of Rallybio. "In 2021, we made meaningful progress against this goal. We announced initial data that reinforced the potential of our preventative approach to FNAIT and advanced our broader portfolio, including the C5 and ENPP1 inhibitor programs, toward the clinic."

Dr. Mackay continued, "As we enter the new year, we are poised to build on this momentum. We recently began enrolling subjects in our Phase 1 study of RLYB212, our lead product candidate for the prevention of FNAIT, and expect to initiate a Phase 1 study of RLYB116, our C5 inhibitor for the treatment of patients with PNH and gMG, in the first quarter, with initial data from both studies expected in 2022. In parallel, we continue to seek transformative science around the globe and we look forward to further expanding our portfolio with new assets and partners that have the potential to deliver dramatically improved outcomes for patients."

Recent Portfolio Milestones and Expected Upcoming Milestones:

Maternal Fetal Blood Disorders

Rallybio today announced that the first subjects have been dosed in its Phase 1 study of RLYB212, a novel human monoclonal anti-HPA-1a antibody in development for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT). The single-blind, placebo-controlled Phase 1 study is designed to evaluate the safety and pharmacokinetics (PK) of single and repeat subcutaneous doses of RLYB212 in HPA-1a negative healthy volunteers. A subsequent Phase 1b proof-of-concept study, expected to initiate in the second quarter of 2022, is designed to establish the ability of RLYB212 to rapidly eliminate transfused HPA-1a positive platelets from the circulation of HPA-1a negative healthy male volunteers. Potential proof-of-concept data from the Phase 1b study are expected in the third quarter of 2022.

Complement Dysregulation

Rallybio expects to initiate a Phase 1 study of RLYB116 in the first quarter of 2022. RLYB116 is a novel, potentially long-acting, subcutaneously administered inhibitor of complement factor 5, or C5, in development for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) and generalized Myasthenia Gravis (gMG). Single dose safety, PK and pharmacodynamic data from this Phase 1 study are expected in the second half of 2022.

Metabolic Disorders

Together with its partner Exscientia, Rallybio has created and is advancing a set of potentially best-in-class development candidates targeting ENPP1 for the treatment of hypophosphatasia (HPP). The companies have completed the drug design phase and are working to select a development candidate to advance into the clinic, with investigational new drug (IND)-enabling studies expected to commence in the second half of 2022.

Presentation at the 40th Annual J.P. Morgan Healthcare Conference

Rallybio will webcast its presentation at the 40th Annual J.P. Morgan Healthcare Conference on Thursday, January 13, 2022, at 11:15 a.m. ET. A live webcast of the presentation and subsequent question and answer session will be accessible through the Events and Presentations section of Rallybio's website. An archived replay of the webcast will be available for 30 days following the presentation.

About Rallybio

Rallybio is a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare diseases. Since its launch in January 2018, Rallybio has built a portfolio of promising product candidates, which are now in development to address rare diseases in the areas of hematology, immuno-inflammation, maternal fetal health, and metabolic disorders. The Company's mission is being advanced by a team of highly experienced biopharma industry leaders with extensive research, development, and rare disease expertise. Rallybio is headquartered in New Haven, Connecticut, with an additional facility at the University of Connecticut's Technology Incubation Program in Farmington, Connecticut. For more information, please visit www.rallybio.com.

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. 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, initiation and timing of our planned clinical trials for RLYB212 and RLYB116, and the timing of the availability of data from such clinical trials, the initiation and timing of our pre-IND enabling studies for our ENPP1 inhibitor, and the timing of the availability of data from such studies. 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 1 and or 1b clinical trials for RLYB212 and RLYB116, 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 identify new product candidates and successfully acquire such product candidates from third parties, 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, 2021, 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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Investor
Steven Tuch
Head of Corporate Development
415-218-0697
stuch@rallybio.com

Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200
hannah.deresiewicz@sternir.com

Media
Tara DiMilia
908-369-7168
tara.dimilia@tmstrat.com

Source: Rallybio Corporation