Rallybio Highlights Portfolio Advances and Outlines Expected Milestones in 2024

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-- Phase 2 Study for RLYB212 Expected to Initiate in 2H 2024 --

-- Company to Provide Update on Phase 2 Discussions with European Medicines Agency for RLYB212 in 1H 2024 --

-- Cash Runway Extended into 3Q 2025 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Jan. 4, 2024-- 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 2024 milestones. The Company will present these updates at the 42nd Annual J.P. Morgan Health Care Conference in San Francisco, California on Wednesday, January 10, 2024, at 8:15 a.m. PT (11:15 a.m. ET).

“Over the past twelve months we made significant progress towards building a broad and sustainable pipeline of transformative therapeutics for rare diseases with severe unmet needs,” said Stephen Uden, M.D., Chief Executive Officer of Rallybio. “In 2023, we announced encouraging Phase 1 results for our lead program, RLYB212, for the prevention of fetal and neonatal alloimmune thrombocytopenia, as well as for RLYB116, our C5 inhibitor. In parallel, we completed critical work on our earlier stage pipeline programs while carefully managing our expenses.”

Dr. Uden continued, “We are committed to building on this momentum in 2024. We remain on track to initiate a Phase 2 study for RLYB212 in the second half of 2024 to confirm the dose regimen for RLYB212 in pregnant women at higher risk for FNAIT. Furthermore, we believe RLYB116 is a promising drug candidate that has the potential to address an unmet need for patients by delivering a more convenient once-a-week self-administered therapy, and our earlier stage programs are expected to reach important milestones in 2024. We are also pleased that through careful management of our cash, our current cash runway guidance has been extended into the third quarter of 2025. We expect to have additional updates to our plans and cash runway before the end of the first quarter.”

Recent Portfolio Milestones and Expected Upcoming Milestones

Maternal Fetal Blood Disorders

Rallybio expects to provide an update on Phase 2 discussions with the European Medicines Agency (EMA) for RLYB212, a novel human monoclonal anti-HPA-1a antibody in development for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT), in the first half of 2024.

Rallybio expects to initiate its Phase 2 dose confirmation study for RLYB212 in the second half of 2024. The study is designed to confirm the dose regimen for RLYB212 in pregnant women at higher risk for FNAIT, prior to initiation of a Phase 3 registrational study.

In addition, Rallybio continues to advance its FNAIT natural history alloimmunization study. The FNAIT natural history study is a non-interventional study designed to provide a contemporary dataset for HPA-1a alloimmunization frequency in a racially and ethnically diverse population that can serve as a control arm for the planned single-arm Phase 3 registrational study. In addition, the natural history study is intended to establish the operational framework for the Phase 2 study and future Phase 3 registrational study. Screening for the natural history study is expected to continue simultaneously with execution of the Phase 2 study. Data from both studies will be used for the end of Phase 2 regulatory discussions with the U.S. Food and Drug Administration and the EMA to support design and initiation of the Phase 3 registrational study.

The natural history study has screened more than 7,600 women to date and the Company expects to provide an update on screening for 2024 in the first quarter.

Complement Dysregulation

In December 2023, Rallybio announced preliminary Phase 1 multiple ascending dose data for RLYB116. Preliminary results showed that a 100 mg low volume once-a-week dose of subcutaneously administered RLYB116 achieved sustained mean reductions in free C5 of greater than 93%. In addition, RLYB116 administered as a 100 mg once-a-week dose was observed to be generally well tolerated with injection site reaction as the most common adverse event (AE) in the cohort, occurring in 60% of the participants in the cohort. All AEs with the 100 mg weekly dose were mild in severity.

While the data support advancement of RLYB116 for patients with certain complement-mediated diseases, including generalized myasthenia gravis (gMG), the Company is prioritizing near-term investments in RLYB116 manufacturing and expects that additional manufacturing work will improve tolerability at higher doses with a low injection volume and infrequent subcutaneous administration. The Company believes such enhancements will enable higher exposure to RLYB116 and potentially increase C5 reduction, which can result in treating a broader range of complement-mediated diseases, including paroxysmal nocturnal hemoglobinuria and antiphospholipid syndrome. Rallybio expects to complete this manufacturing work and provide an update on the development plan for RLYB116 in the second half of 2024.

Rallybio, together with its partner EyePoint Pharmaceuticals, Inc., continue to evaluate sustained delivery of Rallybio’s inhibitor of C5 using EyePoint’s proprietary Durasert® technology for sustained intraocular drug delivery, with the initial focus on geographic atrophy, an advanced form of age-related macular degeneration that leads to irreversible vision loss. Rallybio and EyePoint expect to provide an update on this collaboration in the first half of 2024.

Hematological Disorders

Rallybio continues to advance preclinical activities for RLYB331, a preclinical, therapeutic monoclonal antibody that inhibits Matriptase-2 (MTP-2), to

support the transition of this asset into clinical development. Rallybio expects to report additional animal data from this program in the first half of 2024.

Metabolic Disorders

Rallybio, together with its partner Exscientia, continues to work toward the selection of a small molecule development candidate to advance into the clinic targeting ENPP1 for the treatment of patients with hypophosphatasia (HPP). Significant progress has been made and proof of mechanism studies are in progress with a leading global HPP expert. Rallybio and Exscientia plan to provide an update on the progress of the program in the second half of 2024.

Rallybio, together with its partner AbCellera, are focused on the discovery, development, and commercialization of novel antibody-based therapeutics for rare diseases. The partnership's first discovery program is focused on identifying a novel treatment for patients with rare metabolic diseases.

Financial Update

Cash, cash equivalents, and marketable securities were $121.4 million as of September 30, 2023. The Company currently expects its cash runway to extend into the third quarter of 2025.

Webcast of Presentation at the 42nd Annual J.P. Morgan Health Care Conference

Rallybio is scheduled to present at the 42nd Annual J.P. Morgan Health Care Conference on Wednesday, January 10, 2024, at 8:15 a.m. PT (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 (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, an inhibitor of complement component 5 (C5), 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 Phase 2 dose confirmation study for RLYB212, the progress and number of women screened in the natural history study, whether the results of the natural history study and the planned Phase 2 dose confirmation study will be sufficient to support design and implementation of a Phase 3 registrational study for RLYB212, whether the manufacturing work for RLYB116 will be timely completed or successful, our expectations regarding the usefulness of data from our clinical studies, the timing of reporting results from our collaboration with EyePoint, the timing of reporting results of the animal studies with RLYB331, the timing of providing an update of our joint venture with Exscientia, the likelihood that Rallybio will be successful in developing RLYB212, RLYB116, or any of our other product candidates, and our cash runway. 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 studies, and complete such clinical studies 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, 2023, 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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