Rallybio Announces Preliminary Phase 1 Multiple Ascending Dose Data for RLYB116, an Innovative Subcutaneously Injected Inhibitor of Complement Component 5

December 20, 2023 at 8:00 AM EST

-- 100 mg Results Demonstrated a Mean Reduction of Greater than 93% in Free C5 with Low Volume Once-a-Week Subcutaneous Dosing --

-- Data Supports the Study of RLYB116 as a Differentiated Therapeutic for the Treatment of Generalized Myasthenia Gravis --

-- Company Announces Extension of Runway to 3Q 2025 As Part of Portfolio Prioritization --

-- Conference Call and Webcast Today at 8:30 AM Eastern Time --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Dec. 20, 2023-- Rallybio Corporation (Nasdaq: RLYB) today announced preliminary Phase 1 multiple ascending dose (MAD) data for RLYB116, an innovative, long-acting, low volume subcutaneously injected inhibitor of complement component 5 (C5), in development for patients with complement-mediated diseases.

The Phase 1 MAD study for RLYB116 evaluated the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of subcutaneous RLYB116 in healthy participants with multiple dose administration. The MAD study utilized an adaptive design and included four cohorts of 12 participants receiving doses of up to 200 mg per week of RLYB116 or placebo, with a four-week treatment duration and a 10-week follow-up period.

The preliminary results showed:

- A 100 mg low volume (1 mL) once-a-week dose of subcutaneously administered RLYB116 achieved sustained mean reductions in free C5 of greater than 93%, including at Day 29 with measurement prior to the last dose. The reduction in free C5 at 24 hours after the first dose of 100 mg was greater than 99%. These data and additional work we have conducted with RLYB116 reinforce our confidence that RLYB116 has the potential to be an effective treatment for patients with certain complement-mediated diseases, including generalized myasthenia gravis (gMG).
- RLYB116 also demonstrated low inter-subject variability and consistent increases in exposure relative to dose. The mean estimated elimination half-life for RLYB116 was >300 hours.
- In comparison to 100 mg weekly administration, higher concentrations of RLYB116 were observed in a cohort with 100 mg administered twice per week and were associated with a greater than 97% mean reduction in free C5.
- RLYB116 administered as a 100 mg once-a-week dose was observed to be generally well tolerated. The most common adverse event (AE) in the cohort was injection site reaction (ISR), which occurred in 60% of the participants in the cohort. All AEs during subcutaneous administration with the 100 mg weekly dose were mild in severity.
- The ISR rate for all participants in the 4 cohorts was 59% and all were mild in severity. There were no serious AEs reported for participants receiving study treatment.
- A participant with a history of hepatitis A receiving the 150 mg dose experienced liver enzyme test elevation that resulted in discontinuation and a reduction in the dose for the 3rd cohort from 150 mg to 125 mg.
- The measurement of anti-drug antibody (ADA) formation in the study did not demonstrate an effect on PK or PD parameters and did not appear to be associated with an effect on AE incidence or severity.

“The preliminary results from this Phase 1 multiple ascending dose study of RLYB116 support continued development in patients with gMG,” said Eric Watsky, M.D., RLYB116 Program Lead for Rallybio. “We are encouraged by the free C5 reductions demonstrated by RLYB116 as well as the exposures achieved with subcutaneous administration. Through enhancements in the manufacturing process, we believe we have the opportunity to increase the dose of RLYB116 and improve the tolerability thereby opening up the opportunity to treat a wider range of complement mediated diseases. Our market research is consistent with our belief that an effective, once-a-week, well-tolerated therapy that can be rapidly self-administered with an autoinjector would be an attractive alternative for patients.”

RLYB116 Near-Term Development Plans and Cash Runway

The preliminary data from the Phase 1 MAD study confirm improvements made to date in the manufacturing process will enable the Company to advance RLYB116 into studies in patients. Rather than immediately proceed to a Phase 2 study in gMG, the Company intends to prioritize near-term investments in RLYB116 in the manufacturing process. The Company expects that the 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. In addition, this will allow the Company to direct available cash resources to advance RLYB212, its product candidate for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT).

The Company is also updating its cash runway guidance and now expects its current cash, cash equivalents and marketable securities to extend the runway into the third quarter of 2025.

“We are pleased to see substantial reductions in free C5 with once weekly subcutaneous dosing of RLYB116,” said Stephen Uden, M.D., Chief Executive Officer of Rallybio. “The Phase 1 MAD data show us that RLYB116 can be a potential therapeutic to treat gMG and other complement

mediated diseases. In the spirit of managing our cash runway to realize the most value from our portfolio, we have decided to focus our RLYB116 investments on the manufacturing process with a goal of expanding the scope of therapeutic indications and addressing unmet medical need. In parallel, we continue to advance our lead program, RLYB212, and have extended our runway into the third quarter of 2025."

Conference Call Information

Rallybio will host a conference call and webcast today, December 20, 2023 at 8:30 a.m. Eastern Time to discuss the RLYB116 Phase 1 multiple ascending dose (MAD) study. The live webcast and replay may be accessed by visiting Rallybio's website at http://investors.rallybio.com. In addition, key slides from the RLYB116 Phase 1 MAD study will be discussed on the conference call and are posted to the "Events and Presentations" section of the Rallybio website. A replay 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, long-acting, subcutaneously injected inhibitor of C5 in development for the treatment of patients with complement-mediated diseases. Phase 1 in healthy participants included the study of RLYB116 as a single ascending dose and multiple ascending dose. The multiple ascending dose (MAD) study of RLYB116 included an adaptive single-blind design initiated in the first quarter of 2023 with a 4-week treatment duration to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of subcutaneous RLYB116 in healthy participants with multiple dose administration.

The MAD study of RLYB116 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).

Post-treatment / study follow-up continued for 10 weeks.

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 with an additional facility at the University of Connecticut's Technology Incubation Program in Farmington, 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 results from the Phase 1 MAD study of RLYB116; potential clinical effects and benefits of RLYB116, including for the treatment of gMG; the timing and initiation of future clinical studies for RLBY116; the success cost and timing of our clinical development of our product candidates, including RLYB212 and RLYB116; and statements concerning the Company's anticipated use of cash and 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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Source: Rallybio Corporation