Rallybio Reports Third Quarter 2024 Financial Results and Provides Business Updates

November 7, 2024 at 8:00 AM EST

—CTA Approvals Received for RLYB212 Phase 2 Clinical Trial; On Track to Initiate Screening in 4Q 2024 —

—$75.1 Million in Cash, Cash Equivalents, and Marketable Securities as of September 30, 2024 Provides Runway into Mid-2026 —

NEW HAVEN, Conn.--(BUSINESS WIRE)--Nov. 7, 2024-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, today reported third quarter financial results for the period ended September 30, 2024, and provided an update on recent company developments.

"Advancing RLYB212 into a Phase 2 trial marks a significant achievement for Rallybio. With clinical trial application (CTA) approvals in-hand, our team is activating clinical sites, and we expect to initiate screening for pregnant women at higher risk for alloimmunization and fetal and neonatal alloimmune thrombocytopenia (FNAIT) in the coming weeks," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "Additionally, the focused investments in our RLYB116 program have delivered important data that we look forward to sharing in December along with next steps for the program. As a result of our refocused operating plan announced earlier this year, we have reduced our cash burn and we are on track to achieve all of our 2024 pipeline milestones. We will continue our strategy of focused investments which positions Rallybio to deliver multiple key value inflection points in 2025 and long-term value creation."

Recent Business Highlights and Upcoming Milestones:

RLYB212 Program

- Rallybio announced the approval of its CTAs for the RLYB212 Phase 2 dose confirmation trial in pregnant women at higher risk for HPA-1a alloimmunization and FNAIT. With these approvals from the European Medicines Agency (EMA) and the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA), activation of clinical sites is underway and screening is on track to begin in the fourth quarter of 2024.
- Screening continues in the Company's FNAIT natural history study, with more than 13,000 pregnant women screened as of November 1, 2024. Rallybio has now transitioned European sites from the natural history study to the Phase 2 trial, where the sites will continue to collect natural history data in those women at higher risk for HPA-1a alloimmunization who do not receive RLYB212.
- Results from the Phase 1b proof-of-concept study were published in *Thrombosis and Haemostasis* in August 2024. Additional publications, including the rationale and modeling that support the RLYB212 Phase 2 dose regimen, are expected later this year.
- Data from a preclinical model demonstrating that prophylactic administration of RLYB212 safely and effectively prevents FNAIT in pregnant mice will be presented at the American Society of Hematology (ASH) Annual Meeting being held December 7 – 10 in San Diego, CA.

Title: *Prophylactic Administration of HPA-1a–Specific Antibody RLYB212 Safely Prevents Fetal/Neonatal Alloimmune Thrombocytopenia in Pregnant Mice*
Publication Number: *1185*
Session Name: *311. Disorders of Platelet Number or Function: Clinical and Epidemiological: Poster I*
Session Date: *Saturday, December 7, 2024*
Presentation Time: *5:30 PM - 7:30 PM*
Location: *San Diego Convention Center, Halls G-H*

This poster was also selected for the ASH Poster Walk on *Hemostasis and Thrombosis: from Basic Concepts to Clinical Applications Hosted by Blood Vessels, Thrombosis & Hemostasis,* which will take place on December 7 from 11:15 AM - 12:15 PM.

- Rallybio presented full data from an epidemiological analysis that provided the first robust evidence quantifying the proportion of women of non-Caucasian ancestries that carry the genetic markers for higher FNAIT risk (HPA-1a negative, HLA-DRB3*01:01 positive) at the NORD Rare Diseases and Orphan Products Breakthrough Summit, which was held October 20 – 22 in Washington D.C., and at the American Society of Human Genetics (ASHG) Annual Meeting, which is being held November 5 – 9 in Denver, CO. Based on the data, it is estimated that more than 30,000 pregnancies each year are at higher risk for FNAIT in North America and major European countries alone.

RLYB116 Program

- RLYB116 manufacturing work was successfully completed in the third quarter of 2024. Rallybio expects that the process

enhancements from this manufacturing work will improve the tolerability of RLYB116.

- Additional complement biomarker characterization has been completed. These results, when taken together with Phase 1 MAD data, indicate that RLYB116 led to greater sustained reductions in free C5 than initially understood. Based on these analyses, Rallybio believes that there is an opportunity to pursue indications beyond generalized myasthenia gravis, including paroxysmal nocturnal hemoglobinuria and antiphospholipid syndrome at doses tested in the Phase 1 MAD study.

- In December 2024, Rallybio expects to provide updates on the manufacturing process enhancements and biomarker characterization as well as future plans for RLYB116.

Preclinical Programs

In accordance with its 2024 operating plan, Rallybio is advancing its preclinical programs to important 2024 milestones. Beyond achievement of these milestones, Rallybio is seeking alternative options to further advance these programs, including partnerships and other forms of non-dilutive financing.

- **RLYB332:** Nonclinical data evaluating RLYB332, a long-acting version of the RLYB331 anti-matriptase-2 antibody, will be presented at the upcoming ASH Annual Meeting.

Title: *Long-Acting Anti-Matriptase-2 Antibody as a Potentially Best-in-Class Therapy for Iron Overload Diseases*
Publication Number: *3854*
Session Name: *102. Iron Homeostasis and Biology: Poster III*
Session Date: *Monday, December 9, 2024*
Presentation Time: *6:00 PM - 8:00 PM*
Location: *San Diego Convention Center, Halls G-H*

- **ENPP1 Inhibitor, Exscientia Partnership:** Rallybio and Exscientia remain on track to nominate an ENPP1 inhibitor development candidate for the treatment of patients with hypophosphatasia (HPP) in December 2024. Data from an early lead ENPP1 inhibitor in a model of HPP was presented at the American Society for Bone and Mineral Research (ASBMR) meeting in September. The data demonstrated that oral dosing of REV101 to adult HPP mice lowered inorganic pyrophosphate (PPi) by 30%, leading to improvements in mineralization of long and vertebrate bones. Furthermore, data showed that ENPP1 inhibition was safe and well-tolerated. Data also showed, for the first time, that ENPP1 is a druggable target for later-onset HPP.

Corporate Updates

- Rallybio was named to the 2024 Fierce 50 List as an Innovation Honoree.

Third Quarter 2024 Financial Results

- **Revenue:** Revenue was $0.3 million for the third quarter of 2024, compared to no revenue in the same period in 2023. The increase was related to Rallybio's entrance into the collaboration agreement with Johnson & Johnson in the second quarter of 2024 and the recognition of revenue related to the collaboration's performance obligations.

- **Research & Development (R&D) Expenses:** R&D expenses were $8.2 million for the third quarter of 2024, compared to $13.3 million for the same period in 2023. The decrease in R&D expense was primarily due to a decrease in development costs related to RLYB116 and RLYB212, in addition to a decrease in payroll and personnel-related costs, largely related to the workforce reduction and lower ongoing headcount in 2024 as compared to 2023.

- **General & Administrative (G&A) Expenses:** G&A expenses were $4.1 million for the third quarter of 2024, compared to $6.1 million for the same period in 2023. The decrease in G&A expense was primarily related to lower payroll and personnel-related costs, largely related to the workforce reduction and lower ongoing headcount in 2024 as compared to 2023, and reductions in other related general and administrative expenses, including a reduction in consulting fees.

- **Net Loss and Net Loss Per Common Share:** Rallybio reported a net loss of $11.5 million, or $0.26 per common share, for the third quarter of 2024. This compares to a net loss of $18.4 million, or $0.45 per common share, for the third quarter of 2023.

- **Cash Position:** As of September 30, 2024, cash, cash equivalents, and marketable securities were $75.1 million.

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 clinical trial for RLYB212, whether the results of the FNAIT natural history study and the planned RLYB212 Phase 2 trial will be sufficient to support design and implementation of a Phase 3 registrational study for RLYB212, whether the manufacturing work for RLYB116 will result in improved tolerability, if and when tested in a clinical trial, our expectations regarding the usefulness of data from our clinical studies, our expectations regarding driving awareness of FNAIT through the Johnson & Johnson collaboration, our ability to more accurately identify the number of pregnant women at higher risk of FNAIT and our estimates of the number of women at higher risk for FNAIT in North America and major European countries alone, our estimates of the market opportunity for RLYB212, the timing of publications relating to FNAIT and RLYB212, whether our conclusions from RLYB116 biomarker characterization work will be informative, the timing of achieving milestones in 2024 for our preclinical programs, including for candidate nomination of an ENPP1 inhibitor, whether Rallybio will deliver multiple key value inflection points in 2025, if any, and long-term value creation, the likelihood that Rallybio will be successful in developing RLYB212, RLYB116, or any of our other product candidates, our ability to successfully identify and implement alternative and acceptable options to further advance our programs, our estimates of our capital requirements and the sufficiency thereof, 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 trials, including the FNAIT natural history study, and the Phase 2 trial for RLYB212, 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 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 June 30, 2024, 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.

Financial Tables

RALLYBIO CORPORATION
SELECTED CONDENSED CONSOLIDATED FINANCIAL INFORMATION

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

	FOR THE THREE MONTHS ENDED SEPTEMBER 30,		FOR THE NINE MONTHS ENDED SEPTEMBER 30,	
(in thousands, except share and per share amounts)	**2024**	**2023**	**2024**	**2023**
Revenue:				
Collaboration and license revenue	$ 299	$ —	$ 598	$ —
Total revenue	299	—	598	—
Operating expenses:				
Research and development	8,240	13,288	34,122	37,620
General and administrative	4,125	6,075	15,364	20,200
Total operating expenses	12,365	19,363	49,486	57,820
Loss from operations	(12,066)	(19,363)	(48,888)	(57,820)
Other income:				
Interest income	986	1,545	3,405	4,699

Other income	251	92	561	227
Total other income, net	1,237	1,637	3,966	4,926
Loss before equity in losses of joint venture	(10,829)	(17,726)	(44,922)	(52,894)
Loss on investment in joint venture	637	648	1,809	1,428
Net loss	$ (11,466)	$ (18,374)	$ (46,731)	$ (54,322)
Net loss per common share, basic and diluted	$ (0.26)	$ (0.45)	$ (1.08)	$ (1.35)
Weighted-average common shares outstanding, basic and diluted	44,593,221	40,531,497	43,170,177	40,382,625
Other comprehensive loss:				
Net unrealized gain (loss) on marketable securities	240	64	154	6
Other comprehensive gain (loss)	240	64	154	6
Comprehensive loss	$ (11,226)	$ (18,310)	$ (46,577)	$ (54,316)

Condensed Consolidated Balance Sheets
(Unaudited)

(in thousands)	**SEPTEMBER 30, 2024**	**DECEMBER 31, 2023**
Cash, cash equivalents and marketable securities	$ 75,139	$ 109,929
Total assets	79,007	115,620
Total liabilities	8,189	9,436
Total stockholders' equity	70,818	106,184

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