# Rallybio Reports Third Quarter 2022 Financial Results

November 7, 2022

*-- Preliminary results from Phase 1b study of RLYB212 showed rapid and complete elimination of transfused platelets in all subjects to date; Proof-of-concept data expected in 1Q 2023 --*

*-- Positive 100 mg results of Phase 1 study of RLYB116 showed reduction of >99% in free C5 with the potential for weekly or less frequent dosing --*

NEW HAVEN, Conn.--(BUSINESS WIRE)--Nov. 7, 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 reported financial results for the third quarter ended September 30, 2022 and provided an update on recent program and corporate developments.

"We made tremendous progress across our portfolio in recent months, demonstrating the power of our business development platform to identify transformative science, as well as the ability of our team to efficiently advance clinical trials across diverse therapeutic areas," said Martin Mackay, Ph.D., Chief Executive Officer. "Earlier this morning, we announced positive topline data from our Phase 1 trial of RLYB116, which we believe validate RLYB116 as a highly innovative, differentiated inhibitor of complement component 5 (C5), with the potential to address clinical and commercial challenges that have limited the reach of first-generation programs. We look forward to initiating a multiple ascending dose Phase 1 study in the first quarter of 2023 and, ultimately, to developing RLYB116 for a broad range of complement-mediated diseases."

Dr. Mackay continued, "Moving to the rest of our pipeline, in September 2022, we announced preliminary platelet elimination results from our ongoing Phase 1b proof-of-concept study for RLYB212, which is in development for the prevention of fetal and neonatal alloimmune thrombocytopenia. These data showed rapid and complete elimination of transfused platelets in all subjects to date, with a greater than 90 percent reduction of the mean platelet elimination half-life compared to placebo. We expect to discuss the proof-of-concept data in the first quarter of 2023. Our other preclinical programs are also advancing according to our plan. We expect our existing capital will be sufficient to fund our operating expenses and capital expenditure requirements into the first quarter of 2024."

**Recent Business Highlights and Upcoming Milestones:**

***Maternal Fetal Blood Disorders***

In September 2022, Rallybio announced preliminary platelet elimination results from its ongoing Phase 1b proof-of-concept study for RLYB212. These data demonstrated that one week after a single subcutaneous dose, RLYB212 eliminated transfused HPA-1a positive platelets rapidly compared to placebo in a challenge model of a catastrophic fetal maternal hemorrhage. Further, these data showed rapid and complete elimination of transfused platelets in all subjects to date, with a greater than 90% reduction of the mean platelet elimination half-life compared to placebo, which is consistent with Rallybio's proof-of-concept criteria.

Rallybio also announced in September 2022 that dosing under the amended protocol has commenced, which increases the dose of RLYB212. The Company expects the broader range of pharmacokinetic and pharmacodynamic data to enable substantive modeling of the concentration-effect relationship that further informs dosing for a future registrational study. The Company expects to discuss these proof-of-concept data in the first quarter of 2023.

Rallybio continues to advance the FNAIT natural history alloimmunization study. This non-interventional study is designed to inform on the frequency of women at higher risk for FNAIT across a broad population of pregnant women of different racial and ethnic characteristics, and the occurrence of HPA-1a alloimmunization in these women. The Company expects that the data from this study will contribute to a control dataset for a future single armed registration trial for RLYB212.

***Complement Dysregulation***

Rallybio announced today positive topline results from its Phase 1 single ascending dose study of RLYB116, an innovative, potentially long-acting, subcutaneously administered inhibitor of C5, in development for the treatment of patients with complement-mediated diseases. In the ongoing Phase 1 study, all study participants that were administered a single 1 mL subcutaneous injection of 100 mg of RLYB116 (n=6) demonstrated a reduction in free C5 greater than 99% within 24 hours of dosing. The terminal elimination half-life of RLYB116 was greater than 300 hours. Subcutaneously administered RLYB116 was observed to be generally well-tolerated at the 100 mg dose, with mild or moderate adverse events and no drug-related serious adverse events reported.

Rallybio currently expects to initiate the multiple ascending dose Phase 1 study of RLYB116 in the first quarter of 2023.

Rallybio continues to advance development of RLYB114, formulated for intravitreal injection, for the treatment of ophthalmic disorders.

***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 to identify a lead compound and generate mouse in vivo biomarker evidence to support modulation of on-target activity. Through our collaboration with a leading global HPP expert, in vivo efficacy data is expected in the second half of 2023. Following those results, both companies expect to commence investigational new drug (IND)-enabling studies.

***Hematological Disorders***

Rallybio continues to conduct IND-enabling activities for RLYB331, a preclinical potentially first-in-class therapeutic monoclonal antibody that inhibits Matriptase-2 (MTP-2), to support transition of the asset into clinical development. RLYB331 has the potential to treat multiple disorders characterized by severe anemias with ineffective erythropoiesis and iron overload, such as beta thalassemia and a subset of lower risk myelodysplastic syndromes.

**Third Quarter 2022 Financial Results:**

- **Research & Development (R&D) Expenses:** R&D expenses were $12.1 million for the third quarter of 2022, compared to $5.0 million for the same period in 2021. The increase in R&D expenses was primarily due to an increase in clinical development and manufacturing costs of RLYB212 and RLYB116. In addition, R&D personnel expenses increased as compared to the same period in 2021 due to headcount growth.
- **General & Administrative (G&A) Expenses:** G&A expenses were $6.8 million for the third quarter of 2022, compared to $5.0 million for the same period in 2021. The increase in G&A expenses was primarily due to an increase in payroll and personnel-related costs due to an increase in general and administrative related headcount, including an increase of $0.7 million in share-based compensation.
- **Net Loss and Net Loss Per Common Share:** Net loss was $18.4 million for the third quarter of 2022, or net loss per common share of $0.60 compared to a net loss of $10.2 million, or net loss per common share of $0.37 for the third quarter of 2021.
- **Cash Position:** As of September 30, 2022, cash, cash equivalents, and marketable securities were $132.4 million.

**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. 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, initiation and timing of our planned studies for RLYB212 and RLYB116, and the timing of the availability of data from such studies, 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, and the therapeutic effects of RLYB331. 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 1b clinical trials for RLYB212 and the Phase 1 study for 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, our ability to integrate RLYB331 into our pipeline, 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, 2022, 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)** | **2022** | **2021** | **2022** | **2021** |

| | | | | |
|---|---|---|---|---|
| Operating expenses: | | | | |
| Research and development | $ 12,110 | $ 4,960 | $ 29,896 | $ 20,815 |
| General and administrative | 6,750 | 5,021 | 20,897 | 12,520 |
| Total operating expenses | 18,860 | 9,981 | 50,793 | 33,335 |
| Loss from operations | (18,860) | (9,981) | (50,793) | (33,335) |
| Other income (expenses): | | | | |
| Interest income | 525 | 12 | 892 | 42 |
| Interest expense | — | — | — | (10) |
| Other income (expense) | 98 | 105 | 311 | (13) |
| Total other income, net | 623 | 117 | 1,203 | 19 |
| Loss from continuing operations | (18,237) | (9,864) | (49,590) | (33,316) |
| Loss on investment in joint venture | 133 | 332 | 861 | 1,282 |
| Net loss | $ (18,370) | $ (10,196) | $ (50,451) | $ (34,598) |
| Net loss per common share, basic and diluted | $ (0.60) | $ (0.37) | $ (1.65) | $ (1.44) |
| Weighted average common shares outstanding, basic and diluted | 30,777,797 | 27,527,770 | 30,562,723 | 24,011,862 |
| Other comprehensive loss: | | | | |
| Net unrealized loss on marketable securities | 63 | — | 434 | — |
| Other comprehensive loss | (63) | — | (434) | — |
| Comprehensive loss | $ (18,433) | $ (10,196) | $ (50,885) | $ (34,598) |

**Condensed Consolidated Balance Sheets (Unaudited)**

| **(in thousands)** | **SEPTEMBER 30, 2022** | **DECEMBER 31, 2021** |
|---|---|---|
| Cash, cash equivalents, and marketable securities | $ 132,420 | $ 175,334 |
| Total assets | 143,666 | 182,185 |

| | | |
|---|---|---|
| Total liabilities | 11,723 | 6,583 |
| Total stockholders' equity | 131,943 | 175,602 |

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Source: Rallybio Corporation