Rallybio Reports Fourth Quarter and Full Year 2022 Financial Results

March 6, 2023 at 8:00 AM EST

-- Proof-of-Concept Achieved for RLYB212; Showed Rapid and Complete Elimination of Transfused HPA-1a Positive Platelets in HPA-1a Negative Subjects --

-- Phase 1 Multiple Dose Cohort RLYB212 Study Initiated --

-- Phase 1 Multiple Ascending Dose Study of RLYB116 Continues to Progress; Safety, PK and PD Data Expected in 4Q 2023 --

-- $169.0 million cash, cash equivalents and marketable securities as of December 31, 2022; Provides Runway into 1Q 2025 --

NEW HAVEN, Conn.--(BUSINESS WIRE)--Mar. 6, 2023-- 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 fourth quarter and full year ended December 31, 2022 and provided an update on recent program and corporate developments.

"In 2023, we will fortify our position as a leader in the development of transformative medicines for underserved rare diseases," said Martin Mackay, Ph.D., Chief Executive Officer of Rallybio. "Earlier today, we announced proof-of-concept for RLYB212, our product candidate for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT). These data show that RLYB212 was able to markedly accelerate the elimination of transfused HPA-1a positive platelets in HPA-1a negative subjects, supporting our development of RLYB212 as a potential first-in-class medicine to prevent HPA-1a alloimmunization and, ultimately, eliminate FNAIT. We are now enrolling the multiple dose cohort of our Phase 1 trial and look forward to sharing additional data in the fourth quarter of 2023."

Dr. Mackay continued, "In parallel, we are advancing our efforts across complement dysregulation, hematology and metabolic disorders. In the fourth quarter of 2022, we initiated a multiple ascending dose Phase 1 study of RLYB116, our inhibitor of complement component 5 (C5), and we look forward to sharing initial data from this study in the fourth quarter of 2023. In addition, we entered into a collaboration with AbCellera, under which we will co-develop programs for up to five rare disease targets. Following our offering of common stock and pre-funded warrants in November 2022, we believe our capital will enable us to advance our growing portfolio through upcoming milestones, and opportunistically invest in strategic business development transactions that may allow us to broaden our impact and deliver life-transforming therapies to patients globally."

Recent Business Highlights and Upcoming Milestones:

Maternal Fetal Blood Disorders
Rallybio announced today that proof-of-concept has been achieved in a Phase 1b study for RLYB212, an anti-HPA-1a monoclonal antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia. Results showed that one week after a single subcutaneous dose, RLYB212 was able to rapidly and completely eliminate transfused, HPA-1a positive platelets in HPA-1a negative subjects. RLYB212 was observed to be well-tolerated with no serious adverse events reported.

The Company believes that the broad range of pharmacokinetic and pharmacodynamic data in the Phase 1b study will allow for substantive modeling to inform dose selection for a future registrational study.

The Company expects to report data from the Phase 1b clinical study of RLYB212 at a scientific conference in 2023.

Rallybio also announced today that testing in the multiple dose cohort of its Phase 1 trial in Europe initiated in the first quarter of 2023. This portion of the Phase 1 study will evaluate safety and pharmacokinetics of RLYB212 based on repeat dosing over 12 weeks in healthy male and female participants. The Company expects results from this cohort of subjects in the fourth quarter of 2023.

The Company 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.

Given the favorable development profile of RLYB212 as compared to RLYB211 to date, the data generated to date for RLYB212, and the expected manufacturing and supply efficiencies for RLYB212, the Company will not continue development of RLYB211, a plasma-derived polyclonal anti-HPA-1a antibody.

Complement Dysregulation
Rallybio initiated dosing in the first multiple ascending dose cohort of a Phase 1 study of RLYB116 in the fourth quarter of 2022. RLYB116 is a novel, potentially long-acting, subcutaneously administered inhibitor of C5 in development for the treatment of patients with complement-related diseases. The single-blind, dose escalation, placebo-controlled study is designed to evaluate the safety, pharmacokinetics, and pharmacodynamics of RLYB116 in healthy participants.

The Company expects initial data from the multiple ascending dose cohort in the fourth quarter of 2023.

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

Rallybio and EyePoint Pharmaceuticals, Inc. announced in February 2023 a research collaboration to explore and assess the viability of utilizing Rallybio's inhibitor of complement component 5 (C5) in EyePoint's Durasert ®technology for sustained intraocular delivery. The initial focus will be on

geographic atrophy, an advanced form of age-related macular degeneration that leads to irreversible vision loss.

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). In vivo efficacy data is expected in the second half of 2023. Following those results, we expect to commence investigational new drug (IND)-enabling studies.

In December 2022, Rallybio announced a strategic alliance with AbCellera to discover, develop, and commercialize novel antibody-based therapeutics for rare diseases. Under the terms of the agreement, AbCellera and Rallybio will co-develop up to five rare disease therapeutic targets, which will be chosen together by both companies. The partnership's first program will focus on addressing the significant unmet therapeutic needs of patients with rare metabolic diseases.

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 the transition of this asset into clinical development.

Corporate Updates
In January 2023, Rallybio announced the appointment of Jonathan I. Lieber as Chief Financial Officer (CFO), effective February 1, 2023. Mr. Lieber succeeds Jeffrey Fryer, CPA, Rallybio's Co-Founder and former CFO. The Company announced Mr. Fryer's retirement in June 2022, and following a transition period with Mr. Lieber, Mr. Fryer departed the company on February 15, 2023.

In November 2022, Rallybio consummated a follow-on offering of common stock and pre-funded warrants that raised approximately $55.0 million in gross proceeds.

Fourth Quarter and Full Year 2021 Financial Results:

- **Research & Development (R&D) Expenses:** R&D expenses were $10.8 million for the fourth quarter of 2022, compared to $6.1 million for the same period in 2021. R&D expenses for the fourth quarter 2022 increased primarily due to an increase in costs to advance RLYB212 and an increase in R&D related headcount costs as compared to the prior year. R&D expenses were $40.7 million for the year ended December 31, 2022 compared to $26.9 million for the year ended December 31, 2021. R&D expenses for the year ended December 31, 2022 increased primarily due to an increase in costs to advance RLYB212, the asset acquisition in-process research and development expense and development expenses of RLYB331 and additional R&D related headcount costs as compared to the prior year including an increase of $2.4 million in share-based compensation expense.
- **General & Administrative (G&A) Expenses:** G&A expenses were $6.3 million for the fourth quarter of 2022, compared to $6.2 million for the same period in 2021. G&A expenses were $27.2 million for the year ended December 31, 2022 compared to $18.7 million for the year ended December 31, 2021. The increase in general and administrative expenses for the full year was primarily due to additional G&A related payroll and personnel-related costs, including an increase of $3.5 million in share-based compensation expense, other professional fees, and costs associated with operating as a public company for full fiscal year as compared to 2021.
- **Net Loss and Net Loss Per Common Share:** Net loss was $16.2 million for the fourth quarter of 2022, or net loss per common share of $0.46 compared to a net loss of $12.4 million, or net loss per common share of $0.42 for the fourth quarter of 2021. Net loss was $66.7 million, or net loss per share of $2.09, for the year ended December 31, 2022 compared to $47.0 million, or net loss per share of $1.84, for the year ended December 31, 2021.
- **Cash Position:** As of December 31, 2022, cash, cash equivalents and marketable securities were $169.0 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, substance, design and timing of our planned or ongoing studies for RLYB212 and RLYB116, the timing of the availability of data from such studies, our expectations regarding reporting of data from such studies, and our expectations regarding the usefulness of data from such studies, the potential markets for RLYB212, RLYB114, RLYB116 and RLYB331, our estimates of our capital requirements and the sufficiency thereof, our ability to advance our portfolio, our ability to invest in business development activities, our ability to reach and potential impact on patients globally, our plans for development of RLYB114 for the treatment of ophthalmic disorders, our plans for development activities with our strategic collaboration partners, including Exscientia and Abcellera, the initiation and timing of and plans for our pre-IND enabling studies for our ENPP1 inhibitor, the timing of the availability of data from such studies, the initiation and timing of and plans for our pre-IND enabling studies of RLYB331, 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 enter into strategic partnerships or other arrangements, including the development of RLYB114, 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, 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 DECEMBER 31,		FOR THE YEAR ENDED DECEMBER 31,	
(in thousands, except share and per share amounts)	**2022**	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	$ 10,793	$ 6,094	$ 40,689	$ 26,909
General and administrative	6,298	6,219	27,195	18,739
Total operating expenses	17,091	12,313	67,884	45,648
Loss from operations	(17,091)	(12,313)	(67,884)	(45,648)
Other income (expenses):				
Interest income	1,071	12	1,963	54
Interest expense	—	—	—	(10)
Other income	31	109	342	96
Total other income, net	1,102	121	2,305	140
Loss from continuing operations	(15,989)	(12,192)	(65,579)	(45,508)
Loss on investment in joint venture	214	223	1,075	1,505
Net loss	$ (16,203)	$ (12,415)	$ (66,654)	$ (47,013)
Net loss per common share, basic and diluted	$ (0.46)	$ (0.42)	$ (2.09)	$ (1.84)
Weighted-average common shares outstanding, basic and diluted	35,516,630	29,789,974	31,821,311	25,519,114

Other comprehensive loss (income):				
Net unrealized loss (income) on marketable securities	(220)	—	214	—
Other comprehensive (loss) income	220	—	(214)	—
Comprehensive loss	$ (15,983)	$ (12,415)	$ (66,868)	$ (47,013)

Condensed Consolidated Balance Sheets
(Unaudited)

(in thousands)	**DECEMBER 31, 2022**	**DECEMBER 31, 2021**
Cash, cash equivalents and marketable securities	$ 168,994	$ 175,334
Total assets	180,435	182,185
Total liabilities	11,118	6,583
Total stockholders' equity	169,317	175,602

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Investor Contacts
Ami Bavishi
Head of Investor Relations and Corporate Communications
(475) 47-RALLY (Ext. 282)
abavishi@rallybio.com

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

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

Source: Rallybio Corporation