Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2024 Financial Results and Highlights Recent Progress

February 27, 2025

Dosing ongoing in the Phase 2 pivotal study of RP-A501 for Danon disease; Program update expected in the first half of 2025

New England Journal of Medicine publication of Phase 1 study of RP-A501 to treat patients with Danon disease

Long-term data from the Phase 1 trial of RP-A501 for Danon disease presented at AHA on safety and meaningful efficacy

Enrollment completed in low dose cohort of Phase 1 study of RP-A601 for PKP2-ACM; Initial data expected in the first half of 2025

Regulatory reviews ongoing for KRESLADI (severe LAD-I) and RP-L102 (Fanconi Anemia)

Cash, cash equivalents and investments of approximately $372.3M; expected operational runway into the third quarter of 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Feb. 27, 2025-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of genetic therapies for rare disorders with high unmet need, today reported financial and recent operational results for the fourth quarter and year ended December 31, 2024.

"In 2024, we made strong progress in advancing our gene therapy pipeline, underscored by the *New England Journal of Medicine* publication of the Phase 1 study of RP-A501 for Danon disease and long-term data presented at AHA showing its safety and meaningful efficacy up to five years. Our momentum continues as we progress with the Phase 2 pivotal trial of RP-A501 and the Phase 1 trial of RP-A601 for PKP2-ACM, and we remain on track to submit the IND for BAG3-DCM in the first half of 2025," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. "Looking ahead to 2025, we will maintain our focus and resources on advancing our AAV cardiovascular programs while seeking to realize value in our full pipeline in a thoughtful manner, so we deliver the greatest value to our patients and shareholders."

Recent Pipeline and Operational Updates

- **Dosing in the Phase 2 pivotal study of RP-A501 for Danon disease is ongoing.**
  - Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
  - Program update anticipated in the first half of 2025.

- **Long-term data from the Phase 1 study of RP-A501 for Danon disease published in *The New England Journal of Medicine* and new data presented at the American Heart Association's 2024 Late-Breaking Science sessions.**
  - RP-A501 demonstrated safety and meaningful efficacy; all evaluable patients show cardiac *LAMP2* expression and ≥10% reduction in LV mass index at 12 months and sustained through most recent follow up (up to five years).
  - Evidence of sustained clinically meaningful improvement was observed in pediatric patients followed up to 24 months and adult/adolescent patients followed up to 60 months.
  - All evaluable patients had reductions in NYHA heart failure (from Class II to Class I; no longer displaying symptoms of heart failure), improvements in KCCQ (median 27-point increase), and substantial improvements in troponin (median reduction 84%) and BNP (median reduction 57%) observed 24-54 months after treatment.

- **Progressed the Phase 1 clinical study of RP-A601 for PKP2 arrhythmogenic cardiomyopathy (ACM).**
  - Completed enrollment of all patients in the low dose cohort, and initial data from the Phase 1 study is expected in the first half of 2025.
  - Ongoing internal estimates confirm that PKP2-ACM affects approximately 50,000 people in the U.S. and Europe, representing the largest market opportunity in Rocket's pipeline of disclosed programs.
  - Details of the Phase 1 study can be found at www.ClinicalTrials.gov under the NCT identifier NCT05885412.

- **Progressed BAG3-associated dilated cardiomyopathy preclinical program.**
  - Nonclinical, IND-enabling studies are ongoing.
  - Submission of the IND is anticipated in the first half of 2025.

- **Progressed RP-L102 investigational gene therapy for Fanconi Anemia (FA).**
  - Initiated rolling Biologics License Application (BLA) for RP-L102 and submission of the final module is anticipated in 2025.
  - Regulatory review for RP-L102 for the treatment of FA by the European Medicines Authority is ongoing.

- **U.S. Food and Drug Administration (FDA) review of limited additional Chemistry Manufacturing and Controls (CMC) information ongoing for KRESLADI™ (marnetegragene autotemcel; marne-cel) for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).**
  - Rocket previously disclosed that the FDA requested limited additional CMC information to complete its review of KRESLADI to treat severe LAD-I.
  - The Company continues to work with senior leaders and reviewers from the FDA's Center for Biologics Evaluation and Research.
  - Submission of complete BLA to resolve Complete Response Letter anticipated in 2025.

- **Raised net proceeds of $182.5M through public offering.**
  - On December 11, 2024, the Company completed a public offering of approximately 15.2 million shares of our common stock at a public offering price of $12.50 per share and pre-funded warrants to purchase 0.4 million shares of common stock at a price of $12.49 per warrant. The gross proceeds to Rocket from the public offering were approximately $194.7 million, net of $12.2 million of offering costs, commissions, legal and other expenses for net proceeds of $182.5 million.

Fourth Quarter and Full Year 2024 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of December 31, 2024, were $372.3 million.
- **R&D expenses.** Research and development expenses were $171.2 million for the twelve months ended December 31, 2024, compared to $186.3 million for the twelve months ended December 31, 2023. The decrease of $15.1 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $19.9 million. The decreases were partially offset by increases in the costs for professional fees and consultants of $4.0 million, non-cash stock compensation expense of $1.3 million and depreciation expense of $2.2 million.
- **G&A expenses.** General and administrative expenses were $102.0 million for the twelve months ended December 31, 2024, compared to $73.3 million for the twelve months ended December 31, 2023. The increase in G&A expenses was primarily driven by increases in commercial preparation related expenses which consisted of commercial strategy, medical affairs, market development and pricing analysis expenses of $17.6 million, legal expenses of $4.8 million, non-cash stock compensation expense of $3.2 million, and compensation and benefit expense of $2.1 million.
- **Net loss.** Net loss was $258.7 million or $2.73 per share (basic and diluted) for the twelve months ended December 31, 2024, compared to $245.6 million or $2.92 (basic and diluted) for the twelve months ended December 31, 2023.
- **Shares outstanding.** 106,453,818 shares of common stock were outstanding as of December 31, 2024.

Financial Guidance

- **Cash position.** As of December 31, 2024, Rocket had cash, cash equivalents and investments of $372.3 million. Rocket expects such resources will be sufficient to fund its operations into the third quarter of 2026, including producing AAV cGMP batches at the Company's Cranbury, N.J. R&D and manufacturing facility and continued development of its six clinical and/or preclinical programs.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. Rocket's innovative multi-platform approach allows us to design the optimal gene therapy for each indication, creating potentially transformative options that enable people living with devastating rare diseases to experience long and full lives.

Rocket's adeno-associated viral (AAV) vector-based cardiovascular portfolio includes a late-stage program for Danon Disease, a devastating heart failure condition resulting in thickening of the heart, an early-stage program in clinical trials for PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death, and a pre-clinical program targeting BAG3-associated dilated cardiomyopathy (DCM), a heart failure condition that causes enlarged ventricles.

Rocket's lentiviral (LV) vector-based hematology portfolio consists of late-stage programs for Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure (BMF) and potentially cancer, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that

causes recurrent and life-threatening infections which are frequently fatal, and Pyruvate Kinase Deficiency (PKD), a monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

For more information about Rocket, please visit www.rocketpharma.com and follow us on LinkedIn, YouTube, and X.

Rocket Cautionary Statement Regarding Forward-Looking Statements

This press release contains forward-looking statements concerning Rocket's future expectations, plans and prospects that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this release are forward-looking statements. You should not place reliance on these forward-looking statements, which often include words such as "could," "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. These forward-looking statements include, but are not limited to, statements concerning Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, including the timing and outcome of the FDA's review of the additional CMC information that Rocket will provide in response to the FDA's request, the safety, effectiveness and timing of pre-clinical studies and clinical trials, Rocket's ability to establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates, Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies, Rocket's ability to transition to a commercial stage pharmaceutical company, and Rocket's expectation that its cash, cash equivalents and investments will be sufficient to funds its operations into the third quarter of 2026. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket's competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket's ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, the integration of new executive team members and the effectiveness of the newly configured corporate leadership team, Rocket's ability to acquire additional businesses, form strategic alliances or create joint ventures and its ability to realize the benefit of such acquisitions, alliances or joint ventures, Rocket's ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2024, filed February 27, 2025 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 37,357	$ 41,744	$ 171,244	$ 186,342
General and administrative	25,337	21,535	101,961	73,317
Total operating expenses	62,694	63,279	273,205	259,659
Loss from operations	(62,694)	(63,279)	(273,205)	(259,659)
Interest expense	$ (473)	(470)	(1,886)	(1,875)
Interest and other income, net	1,617	814	8,267	5,288
Accretion of discount on investments, net	$ 1,223	3,275	8,078	10,651
Net loss	$ (60,327)	$ (59,660)	$ (258,746)	$ (245,595)
Net loss per share - basic and diluted	$ (0.62)	$ (0.64)	$ (2.73)	$ (2.92)
Weighted-average common shares outstanding - basic and diluted	97,530,032	93,336,541	94,807,773	84,009,004

	December 31, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 372,336	$ 407,495
Total assets	527,700	566,341
Total liabilities	64,466	73,767
Total stockholders' equity	463,234	492,574

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