Rocket Pharmaceuticals Reports First Quarter 2025 Financial Results and Highlights Recent Progress

May 8, 2025

Phase 2 pivotal study of RP-A501 for Danon disease is ongoing; Program update expected mid-year 2025

Initial data from the Phase 1 study of RP-A601 for PKP2-ACM anticipated May 2025

IND submission for BAG3-DCM program expected mid-year 2025

Sarbani Chaudhuri appointed Chief Commercial & Medical Affairs Officer

Cash, cash equivalents and investments of approximately $318.2M; expected operational runway into the fourth quarter of 2026

CRANBURY, N.J.--(BUSINESS WIRE)--May 8, 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 first quarter ending March 31, 2025.

"Rocket maintained strong momentum during the first quarter, and we look forward to sharing updates of our recent progress starting with initial data from the Phase 1 study of RP-A601 for PKP2-ACM this month, followed by an update on our Danon disease program in mid-year 2025. We also remain on track to submit an IND for BAG3-DCM. We continue to prioritize our current resources towards our AAV cardiovascular programs, which we see as holding the greatest potential to deliver transformative therapies to patients and provide the strongest value to shareholders," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. “As we continue into 2025, we remain steadfast in preserving resources and safeguarding financial health to keep on advancing our mission of seeking gene therapy cures for patients with devastating diseases, and as result have expanded our runway into the fourth quarter of 2026.”

Recent Pipeline and Operational Updates

- **Phase 2 pivotal study of RP-A501 for Danon disease is ongoing.**
  - Program update anticipated in mid-year 2025 and a clinical data readout expected in mid-year 2026. Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
  - In March, the largest longitudinal natural history study of Danon disease to date was published in the *Journal of the American Heart Association (JAHA)*, revealing key insights into the distinct cardiac patterns of Danon disease patients, showing earlier, more severe heart issues in male patients, while also noting that many females develop progressive cardiomyopathy and heart failure in adolescence or early adulthood.

- **Initial data from the low dose cohort of the Phase 1 clinical study of RP-A601 for PKP2 arrhythmogenic cardiomyopathy (ACM) expected in May 2025.**
  - Initial data from the Phase 1 study will be presented at an upcoming medical/scientific conference.
  - 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.**
  - Submission of the IND is anticipated mid-year 2025.

- **Regulatory progression of RP-L102 investigational gene therapy for Fanconi Anemia (FA).**
  - Initiated rolling Biologics License Application (BLA) for RP-L102, anticipating final module submission late 2025/early 2026.

- Regulatory review for RP-L102 for the treatment of FA by the European Medicines Authority (EMA) 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).**
  - In May, clinical outcomes data from the nine severe LAD-I patients treated in the global Phase I/II study of KRESLADI™ was published in the *New England Journal of Medicine (NEJM)*, highlighting the transformational effect of KRESLADI™ towards the treatment of severe LAD-I and sustained durability with all patients remaining symptom-free at two years post-treatment without the need of an allogeneic HSCT.
  - 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.

- **In April, Rocket appointed Sarbani Chaudhuri as Chief Commercial & Medical Affairs Officer.**
  - With over 20 years of experience in the biopharma industry, Chaudhuri is a seasoned executive with a proven track record of building, transforming, and steering companies towards delivering transformative patient outcomes.
  - Chaudhuri most recently served as the Vice President & Global Head of Hematology at Johnson & Johnson and has also previously held leadership roles at AstraZeneca, Pfizer, and Novartis, where she successfully drove commercial growth and launched innovative therapies with a focus on rare cardiac and hematology diseases.

First Quarter 2025 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of March 31, 2025, were $318.2 million.
- **R&D expenses.** Research and development expenses were $35.9 million for the three months ended March 31, 2025, compared to $45.2 million for the three months ended March 31, 2024. The decrease of $9.3 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $2.5 million, lab supplies and office expense of $2.3 million, compensation and benefits expense of $1.4 million due to decreased R&D headcount, and professional fees of $1.6 million.
- **G&A expenses.** General and administrative expenses were $28.4 million for the three months ended March 31, 2025, compared to $22.1 million for the three months ended March 31, 2024. The increase in G&A expenses was primarily driven by increases in commercial preparation related expenses of $1.5 million, legal expenses of $4.0 million and non-cash stock compensation expense of $0.7 million.
- **Net loss.** Net loss was $61.3 million or $0.56 per share (basic and diluted) for the three months ended March 31, 2025, compared to $62.1 million or $0.66 (basic and diluted) for the three months ended March 31, 2024.
- **Shares outstanding.** 106,753,886 shares of common stock were outstanding as of March 31, 2025.

Financial Guidance

- **Cash position.** As of March 31, 2025, Rocket had cash, cash equivalents and investments of $318.2 million. Rocket expects such resources will be sufficient to fund its operations into the fourth 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 fourth 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 March 31,	
	2025	**2024**
Operating expenses:		
Research and development	$ 35,942	$ 45,227
General and administrative	28,446	22,148
Total operating expenses	64,388	67,375
Loss from operations	(64,388)	(67,375)
Interest expense	(472)	(471)
Interest and other income, net	1,336	3,029
Accretion of discount on investments, net	2,190	2,763
Net loss	$ (61,334)	$ (62,054)
Net loss per share - basic and diluted	$ (0.56)	$ (0.66)
Weighted-average common shares outstanding - basic and diluted	110,093,461	93,549,884

	March 31, 2025	**December 31, 2024**
Cash, cash equivalents, and investments	$ 318,164	$ 372,336
Total assets	471,066	527,700
Total liabilities	58,928	64,466
Total stockholders' equity	412,138	463,234

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