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

August 7, 2025

Prioritizing development of RP-A501 (Danon disease), RP-A601 (PKP2-ACM), and RP-A701 (BAG3-DCM) to advance AAV cardiovascular gene therapy platform for sustained value creation

FDA RMAT designation awarded to RP-A601 for PKP2-ACM; Engaging with FDA on pivotal trial design following encouraging initial Phase 1 data at ASGCT

IND accepted and FDA Fast Track designation received for RP-A701 in BAG3-DCM program; Phase 1 trial start-up activities underway

Organizational restructuring expected to reduce headcount by approximately 30% and lower 12-month cash burn by nearly 25%

Chris Stevens appointed Chief Operating Officer

Cash, cash equivalents and investments of approximately $271.5M; expected operational runway into the second quarter of 2027

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 7, 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 second quarter ending June 30, 2025.

“The second quarter of 2025 marked an important inflection point for Rocket as we refined our strategic focus around our AAV cardiovascular gene therapy platform and took multiple decisive steps to strengthen our financial foundation and thoughtfully adjust to market dynamics. We are fortifying our path to sustained value creation by leaning into programs with the highest value, conserving cash, and driving an efficient, agile organization,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. “With regulatory alignment to resolve the clinical hold for RP-A501 in Danon disease in progress, momentum building for RP-A601 in PKP2-ACM as it advances toward a pivotal Phase 2 trial, and RP-A701 preparing to enter the clinic for the treatment of BAG3-DCM, Rocket is positioned as the leader in the development of gene therapies for inherited cardiomyopathies. While we are pausing additional investments in the FA and PKD programs, we are deeply grateful and committed to the patient communities and are exploring strategic alternatives to advance these programs externally. Finally, our recent reorganization ensures we are appropriately resourced to execute on our near-term milestones with nearly two years of capital.”

Recent Pipeline and Operational Updates

- **Investigation into the Serious Adverse Event (SAE) from the Phase 2 pivotal study of RP-A501 for Danon disease is ongoing.**
  - Rocket previously disclosed an SAE, related to clinical complications from capillary leak syndrome and unfortunately leading to the patient’s death. In response, Rocket paused dosing and initiated a root cause analysis, focusing on the recent addition of a C3 inhibitor to the pre-treatment regimen. On May 23, 2025, FDA placed the trial on clinical hold for further evaluation.
  - Rocket is actively working with FDA, independent safety monitors, and clinical experts to ensure patient safety and resume the trial.
  - While the clinical hold remains in place, the company cannot provide guidance on trial completion timing.

- **Actively engaging with FDA on the advancement of RP-A601 for PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM) in potential pivotal trial following initial positive Phase 1 data at the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT).**
  - In July, Rocket received FDA Regeneration Medicine Advanced Therapy (RMAT) designation for RP-A601 for PKP2-ACM.

- Initial Phase 1 data demonstrating encouraging safety and preliminary efficacy outcomes of RP-A601 for PKP2-ACM were presented at ASGCT in May 2025.
- Rocket is engaging with FDA on potential pivotal trial design to evaluate the efficacy and safety of RP-A601.

- **Phase 1 trial start-up activities are underway for RP-A701 in BAG3-associated dilated cardiomyopathy (BAG3-DCM).**
  - In June, an Investigational New Drug (IND) application for RP-A701, an AAVrh.74-based gene therapy candidate for the treatment of BAG3-DCM, received clearance from FDA.
  - RP-A701 was recently granted FDA Fast Track designation, designed to facilitate the development and expedite the review of therapies for serious or life-threatening conditions that fill an unmet medical need. This enables increased communication with FDA, the potential for accelerated approval, and permits a rolling Biologics License Application (BLA) review.
  - The first-in-human Phase 1 clinical trial will be a multi-center, dose-escalation study designed to evaluate the safety, biological activity, and preliminary efficacy of RP-A701 in adults with BAG3-DCM.
  - BAG3-DCM is a rare, inherited heart condition caused by mutations in the *BAG3* gene, leading to early-onset, progressive heart failure due to impaired cardiac function, high morbidity, and premature mortality. Rocket estimates that the prevalence of BAG3-DCM in the U.S. is as many as 30,000 individuals.

- **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 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 FDA's Center for Biologics Evaluation and Research and submission of complete BLA to resolve Complete Response Letter is anticipated before the end of 2025.

- **Ongoing strategic corporate restructuring and pipeline prioritization.**
  - As part of its broader strategic reorganization, Rocket implemented a workforce reduction of approximately 30%, across all functions, to align with a pipeline prioritization plan focused exclusively on its AAV cardiovascular gene therapy platform. This restructuring, together with other cost-saving measures, is expected to reduce operating expenses by nearly 25% over the next 12 months. The reorganization enables the company to focus on its late-stage AAV gene therapy programs in Danon disease, PKP2-ACM, and BAG3-DCM, while advancing regulatory activities for KRESLADI™ in severe LAD-I.
  - As part of this realignment, Rocket is pausing additional investments in its Fanconi Anemia (FA; RP-L102) and Pyruvate Kinase Deficiency (PKD; RP-L301) programs.
  - The company continues to evaluate options to advance the FA program with health authorities in alignment with its refined strategic focus and broader corporate priorities.

- **In July, Rocket appointed Chris Stevens as Chief Operating Officer.**
  - Chris Stevens is a highly seasoned executive with 25 years of experience across technical operations, product strategy, and general management, bringing immense commercial technical operations experience to Rocket. Stevens most recently served as the Executive Vice President and Chief Patient Supply Officer at Spark Therapeutics (subsidiary of Roche), where he successfully led teams across manufacturing, supply chain, quality, compliance, engineering, EHS, and facilities management, playing a key role in delivering gene therapies to patients.

Second Quarter 2025 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2025, were $271.5 million. Rocket expects such resources, excluding any potential proceeds from a Priority Review Voucher that may be granted upon FDA approval of KRESLADI™, will be sufficient to fund its operations into the second quarter of 2027.
- **R&D expenses.** Research and development expenses were $42.7 million for the three months ended June 30, 2025, compared to $46.3 million for the three months ended June 30, 2024. The decrease of $3.7 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $2.3 million, professional fees of $2.0 million and building supplies and consumables of $0.8 million, offset by an increase in clinical trial expenses of $1.2 million.
- **G&A expenses.** General and administrative expenses were $25.0 million for the three months ended June 30, 2025, compared to $27.4 million for the three months ended June 30, 2024. The decrease in G&A expenses was primarily driven by decreases in commercial preparation related expenses of $1.4 million and compensation and benefits expense of $0.9

million.

- **Net loss.** Net loss was $68.9 million or $0.62 per share (basic and diluted) for the three months ended June 30, 2025, compared to $69.6 million or $0.74 (basic and diluted) for the three months ended June 30, 2024.
- **Shares outstanding.** 107,884,420 shares of common stock were outstanding as of June 30, 2025.

Restructuring Expenses and Financial Guidance

- **Restructuring expenses.** Approximately $3.5 million in restructuring and restructuring-related charges were incurred in the first half of 2025.

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 clinical program for Danon Disease, a devastating heart failure condition resulting in thickening of the heart, and an early-stage clinical program for PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death. Rocket has also received IND clearance for its AAV-based gene therapy for 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 Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure (BMF) and potentially cancer, 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 ability to realize the intended benefits of the restructuring plan and reduction in workforce, 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 fund its operations into the second quarter of 2027. There can be no assurance that the restructuring plan or the planned reduction in workforce will have the intended effect on the Company's operational results and strategic decisions, that any anticipated charges and any anticipated cost savings associated with the restructuring plan or the reduction in workforce will achieve their intended benefits. 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 June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 42,658	$ 46,345	$ 78,600	$ 91,572
General and administrative	25,020	27,367	53,466	49,515
Restructuring	3,471	-	3,471	-
Total operating expenses	71,149	73,712	135,537	141,087
Loss from operations	(71,149)	(73,712)	(135,537)	(141,087)
Interest expense	(473)	(471)	(945)	(942)

Interest and other income, net	483	2,294	1,819	5,323
Accretion of discount on investments, net	2,220	2,243	4,410	5,006
Net loss	(68,919)	(69,646)	(130,253)	(131,700)
Net loss per share - basic and diluted	$ (0.62)	$ (0.74)	$ (1.18)	$ (1.40)
Weighted-average common shares outstanding - basic and diluted	111,019,647	93,746,243	110,559,113	93,759,894

	June 30, 2025	December 31, 2024
Cash, cash equivalents, and investments	$ 271,494	$ 372,336
Total assets	420,979	527,700
Total liabilities	66,768	64,466
Total stockholders' equity	354,211	463,234

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