Rocket Pharmaceuticals Reports Second Quarter 2026 Financial Results and Highlights Recent Progress

August 10, 2026

All three initial patients treated with RP-A501 under the modified pivotal Phase 2 protocol completed the protocol-defined safety observation period; no thrombotic microangiopathy, capillary leak syndrome, or other significant safety concerns observed

Rocket is actively engaging with the FDA to align on the path to rapidly completing the pivotal Phase 2 trial in Danon disease

Multiple clinical and regulatory milestones remain on track for the second half of 2026, including a broader Danon disease program update, a regulatory update for the planned RP-A601 pivotal study in PKP2-ACM, and initial patient dosing with RP-A701 in BAG3-DCM

FDA approval of KRESLADI™ and subsequent monetization of the Priority Review Voucher generated $180 million in non-dilutive capital; focused preparations are underway for commercial availability and patient onboarding beginning in the fourth quarter of 2026

Cash, cash equivalents and investments of approximately $283.7 million; expected cash runway into the second quarter of 2028

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 10, 2026-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and devastating diseases, focused on inherited cardiovascular disorders, today reported financial and recent operational results for the second quarter ended June 30, 2026.

“The second quarter of 2026 materially strengthened Rocket’s position, with the approval of KRESLADI demonstrating our ability to advance a genetic medicine through regulatory approval, the subsequent sale of the Priority Review Voucher generating $180 million in non-dilutive capital, and safety clearance for the first three Danon disease patients in our modified pivotal trial,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. “Together with the more focused operating structure we implemented in late 2025, these achievements provide a stronger financial and executional foundation for advancing our cardiovascular pipeline. Based on the positive safety observations in Danon disease, we are working with the FDA on the path to treating additional patients and completing the pivotal trial as quickly as possible, while progressing toward additional regulatory and clinical milestones across the remainder of our cardiac portfolio, including PKP2-ACM and BAG3-DCM.”

Recent Pipeline and Operational Updates

- **Positive Initial RP-A501 Safety Findings Support Regulatory Engagement on Next Steps in Danon Disease**
  - Rocket previously disclosed that the initial three patients were safely dosed under the modified pivotal Phase 2 protocol for RP-A501 in Danon disease. All three patients have completed the protocol-defined safety observation period, with no thrombotic microangiopathy (TMA), capillary leak syndrome, or other significant safety concerns observed at the recalibrated dose with the refined immunomodulatory regimen.
  - The recalibrated Phase 2 dose of 3.8×10^{13} GC/kg was selected based on the characteristics of the current drug product, the underlying biology of Danon disease and the Phase 1 experience, with the objective of preserving the potential for meaningful clinical benefit while optimizing RP-A501’s benefit-risk profile.
  - Rocket is actively engaging with the FDA to seek alignment on the path to dosing additional patients and completing the pivotal Phase 2 trial and expects to provide an update on the regulatory pathway in the second half of 2026.
  - Rocket is on track to host an investor webinar in the second half of 2026 to provide a comprehensive Danon disease program update.
  - In parallel with the pivotal Phase 2 trial, Rocket’s global natural history study has enrolled more than 50 patients, including both males and females, building an increasingly robust longitudinal dataset to further characterize Danon disease.

- **Continued FDA Engagement and Phase 1 Enrollment for RP-A601 in PKP2 Arrhythmogenic Cardiomyopathy**

(PKP2-ACM)

- Rocket continues to engage with the FDA regarding alignment on the design of a potential pivotal study of RP-A601 in PKP2-ACM with a regulatory update expected in the second half of 2026. Previously reported Phase 1 findings demonstrated increased PKP2 protein expression and improved desmosomal localization, together with directional improvements in arrhythmia measures and right ventricular function.
- The ongoing Phase 1 study remains open and actively enrolling to further characterize biological activity across a broader range of disease severity.
- Details of the Phase 1 study can be found at www.ClinicalTrials.gov under NCT identifier NCT05885412.

- **Phase 1 Trial Initiation Anticipated in 2026 for RP-A701 in BAG3-Associated Dilated Cardiomyopathy (BAG3-DCM)**
  - Patient screening and enrollment has begun, with initial patient dosing anticipated in the second half of 2026.
  - The multicenter, dose-escalation study is designed to evaluate the safety, biological activity and preliminary efficacy of RP-A701 in adults with BAG3-DCM. Details of the Phase 1 study can be found at www.ClinicalTrials.gov under NCT identifier NCT07137338.

- **Focused Commercialization Strategy Underway for KRESLADI™ (marnetegragene autotemcel) Following FDA Approval**
  - Following the announcement of the FDA's accelerated approval of KRESLADI™ for severe leukocyte adhesion deficiency-I (LAD-I), Rocket is implementing a focused commercialization strategy calibrated to the ultra-rare patient population and concentrated treatment landscape.
  - The strategy is centered on a limited network of specialized Qualified Treatment Centers and an appropriately scaled commercial infrastructure, supporting patient access while maintaining disciplined investment.
  - KRESLADI represents Rocket's first FDA-approved product and demonstrates the Company's ability to advance a complex genetic medicine through clinical development, CMC, regulatory review, and commercial readiness. Rocket anticipates commercial availability and the start of patient onboarding in the fourth quarter of 2026.
  - On June 12, 2026, Rocket announced the closing of the sale of its Rare Pediatric Disease Priority Review Voucher (PRV) for gross proceeds of $180 million. This substantial non-dilutive capital strengthened Rocket's financial position and supports the continued advancement of its prioritized cardiovascular pipeline.

Second Quarter 2026 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2026, were $283.7 million. The cash position reflects the proceeds from the sale of the Rare Pediatric Disease Priority Review Voucher.
- **R&D expenses.** Research and development expenses were $29.5 million for the three months ended June 30, 2026, compared to $42.7 million for the three months ended June 30, 2025. The decrease of $13.2 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $7.5 million and stock-based and other compensation and benefit expenses of $3.6 million due to decreased R&D headcount, depreciation expenses of $1.0 million due to decreased asset base, and clinical trial expenses of $1.0 million.
- **G&A expenses.** General and administrative expenses were $17.4 million for the three months ended June 30, 2026, compared to $25.0 million for the three months ended June 30, 2025. The decrease of $7.6 million in G&A expenses was primarily driven by decreases in commercial preparation-related expenses of $4.8 million due to lower headcount and lower spending on commercial preparation, stock-based and other compensation and benefit expenses of $1.9 million due to decreased G&A headcount, and legal expenses of $1.4 million.
- **Other Income.** Other Income was $178.8 million for the three months ended June 30, 2026, compared to $2.2 million for the three months ended June 30, 2025. The increase in other income was primarily driven by the sale of the Rare Pediatric Disease Priority Review Voucher for net $178.2 million. The increase was partially offset by a decline in accretion of discount on investments, net, of $1.5 million due to lower investment balance and interest rates year over year.
- **Income Taxes.** Income taxes were $8.6 million for the three months ended June 30, 2026, compared to $0.0 million for the three months ended June 30, 2025, as a result of generating net income in the three months ended June 30, 2026.
- **Net income.** Net income was $123.2 million or $1.09 of basic income per share and $1.08 of diluted income per share for the three months ended June 30, 2026, compared to a net loss of $68.9 million or $0.62 (basic and diluted loss per share) for the three months ended June 30, 2025.
- **Shares outstanding.** 109,539,424 shares of common stock were outstanding as of June 30, 2026.

Financial Guidance

- **Cash position.** As of June 30, 2026, Rocket had cash, cash equivalents and investments of $283.7 million. Based on its current operating plan, Rocket expects its cash, cash equivalents and investments as of June 30, 2026, to fund operations into the second quarter of 2028.

About KRESLADI ™

KRESLADI™ (marnetegragene autotemcel) is an autologous hematopoietic stem cell-based gene therapy designed to address the underlying genetic cause of severe leukocyte adhesion deficiency-I (LAD-I), an ultra-rare, life-threatening pediatric immunodeficiency. The therapy utilizes ex vivo lentiviral vector-mediated gene transfer to introduce a functional copy of the *ITGB2* gene into a patient's hematopoietic stem cells, enabling expression of CD18 and restoration of leukocyte adhesion and migration.

KRESLADI is administered as a one-time intravenous infusion following myeloablative conditioning. In clinical studies, treatment with KRESLADI resulted in increased neutrophil CD18 and CD11a surface expression, supporting the biological activity of the therapy and forming the basis for accelerated approval. Continued approval may be contingent upon verification and description of clinical benefit through ongoing clinical follow-up and additional post-marketing data collection.

Severe LAD-I is characterized by recurrent, serious bacterial and fungal infections beginning in early infancy and is associated with high early-childhood mortality without effective treatment.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Serious Infections

Serious infections have occurred with KRESLADI administration. Increased susceptibility to infections may occur due to administration of myeloablative conditioning prior to KRESLADI infusion.

Monitor patients for signs and symptoms of infection before and after KRESLADI infusion and treat appropriately. Administer prophylactic antimicrobials according to institutional guidelines.

Avoid administration of KRESLADI in patients with active bloodstream infections or other serious, untreated infections.

Any blood products required after KRESLADI infusion should be irradiated.

Veno-Occlusive Disease

Veno-occlusive disease has occurred with KRESLADI treatment. Increased susceptibility to veno-occlusive disease may occur due to administration of myeloablative conditioning prior to KRESLADI infusion. Monitor patients for signs and symptoms of veno-occlusive disease including assessment of liver function tests during the first month following KRESLADI infusion.

Neutrophil Engraftment Failure

Neutrophil engraftment failure may occur after treatment with KRESLADI. Neutrophil engraftment failure is defined as failure to achieve three consecutive absolute neutrophil counts (ANC) ≥ 500 cells/microliter obtained on different days by Day 43 after infusion of KRESLADI. Monitor neutrophil counts until engraftment has been achieved. If neutrophil engraftment failure occurs in a patient treated with KRESLADI, provide rescue treatment with the back-up collection of CD34+ cells.

Delayed Platelet Engraftment

Delayed platelet engraftment may occur after treatment with KRESLADI. Monitor platelet counts and bleeding until platelet engraftment and platelet recovery are achieved.

LVV-Mediated Insertional Oncogenesis

Lentiviral vector (LVV)-mediated insertional oncogenesis may occur after treatment with KRESLADI. Hematologic malignancy is a lifelong risk and patients treated with KRESLADI may develop hematologic malignancy at any time following treatment.

Monitor for hematologic malignancies clinically, and with a complete blood count (with differential) at least annually and integration site analysis as warranted for at least 15 years after treatment with KRESLADI and as clinically indicated. If malignancy is detected in any patient who received KRESLADI, contact Rocket Pharmaceuticals, Inc. at 1-800-982-2410 for reporting and to obtain instructions on collection of samples for testing.

Hypersensitivity Reactions

Hypersensitivity reactions including anaphylaxis may occur with the infusion of KRESLADI. The dimethyl sulfoxide (DMSO) in KRESLADI may cause hypersensitivity reactions which may occur in patients with and without prior exposure to DMSO.

Monitor patients for signs and symptoms of hypersensitivity reactions during and after KRESLADI infusion. If a hypersensitivity reaction occurs, pause infusion if ongoing and manage according to clinical practice.

Anti-Retroviral Use

Anti-retroviral medications may interfere with manufacturing of KRESLADI. If a patient requires anti-retrovirals for HIV prophylaxis, mobilization and apheresis of CD34+ cells for KRESLADI manufacturing should be delayed until HIV infection is adequately ruled out. Patients should not take anti-retroviral medications for at least one month prior to mobilization, or for the expected duration required for the elimination of the anti-retroviral medications, and until all cycles of apheresis are completed.

Interference with Serology Testing

Patients who have received KRESLADI are likely to test positive by polymerase chain reaction (PCR) assays for HIV due to LVV provirus insertion resulting in a false-positive test for HIV. Therefore, patients who have received KRESLADI should not be screened for HIV infection using a PCR-based assay.

Blood, Organ, Tissue, and Cell Donation

Patients treated with KRESLADI should not donate blood, organs, tissues, or cells for transplantation at any time in the future.

ADVERSE REACTIONS

The most common non-laboratory adverse reactions (≥ 30% of patients) include: mucositis, upper respiratory tract infection, viral infection, febrile neutropenia, skin lesion, nausea/vomiting, rash/dermatitis, pyrexia, device related infection, and skin infection.

The most common laboratory adverse reactions (≥ 30% of patients) include: hemoglobin decreased, platelet count decreased, neutrophil count decreased, leukocyte count decreased, aspartate aminotransferase increased, and alanine aminotransferase increased.

For additional safety information, refer to the full Prescribing Information.

DRUG INTERACTIONS

No formal drug interaction studies have been performed. KRESLADI is not expected to interact with the hepatic cytochrome P-450 family of enzymes or drug transporters.

Vaccines

The safety and effectiveness of immunization with live viral vaccines during or following KRESLADI treatment has not been studied. Vaccination is not recommended during the 6 weeks preceding the start of myeloablative conditioning, and until hematological recovery following treatment with KRESLADI. Where feasible, administer childhood vaccinations prior to myeloablative conditioning for KRESLADI.

Anti-retroviral Medications

Patients should not take anti-retroviral medications for at least one month prior to initiating medications for stem cell mobilization and for the expected duration for elimination of the medications, and until all cycles of apheresis are completed. Anti-retroviral medications may interfere with manufacturing of KRESLADI.

REFERENCE TO FULL PRESCRIBING INFORMATION

Please see full Prescribing Information for KRESLADI.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated commercial-stage biotechnology company developing genetic medicines for rare and devastating diseases, with a strategic focus on inherited cardiovascular disorders and additional programs in hematology and immunology. Rocket's cardiovascular portfolio includes three clinical-stage gene therapy programs targeting hypertrophic, arrhythmogenic, and dilated cardiomyopathies, together representing one of the broadest pipelines focused on inherited heart disease. The Company's integrated platform combines proprietary AAV manufacturing capabilities, extensive clinical experience in cardiac gene therapy, and long-term safety and efficacy data supporting advancement across its cardiovascular portfolio.

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 "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 cash runway and financial position, Rocket's planned use of proceeds from the monetization of the KRESLADI™ PRV, Rocket's expectations of our ability to obtain additional funding to conduct our planned research and development efforts, the safety and effectiveness of RP-A501 in its pivotal Phase 2 trial under the modified protocol, the Company's ability to align with the FDA on the path to treating additional Danon disease patients, 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, Rocket's plans for the advancement of its cardiovascular AAV programs and KRESLADI™, including its planned pivotal trials, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates and Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies. 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, the results of Rocket's ongoing and planned clinical trials, 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, 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, our ability to achieve the expected benefits of our portfolio prioritization and strategic restructuring, including extending our cash runway, 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, 2025, filed February 26, 2026 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,

	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 29,497	$ 42,658	$ 60,951	$ 78,600
General and administrative	17,425	25,020	34,482	53,466
Restructuring	-	3,471	-	3,471
Total operating expenses	46,922	71,149	95,433	135,537
Loss from operations	(46,922)	(71,149)	(95,433)	(135,537)
Gain from sale of PRV	178,190	-	178,190	-
Interest expense	(473)	(473)	(946)	(945)
Interest and other income, net	347	483	508	1,819
Accretion of discount on investments, net	693	2,220	1,922	4,410
Earnings (losses) before Income Taxes	131,835	(68,919)	84,241	(130,253)
Provision for Income Taxes	(8,621)	-	(8,621)	-
Net income (loss)	$ 123,214	$ (68,919)	$ 75,620	$ (130,253)
Net income (loss) per share - basic	$ 1.09	$ (0.62)	$ 0.67	$ (1.18)
Net income (loss) per share - diluted	$ 1.08	$ (0.62)	$ 0.66	$ (1.18)
Weighted-average common shares outstanding - basic	112,844,880	111,019,647	112,491,433	110,559,113
Weighted-average common shares outstanding - diluted	114,513,964	111,019,647	114,329,763	110,559,113

	June 30, 2026	December 31, 2025
Cash, cash equivalents, and investments	$ 283,747	$ 188,929
Total assets	423,098	330,449
Total liabilities	54,268	53,228
Total stockholders' equity	368,830	277,221

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