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

May 7, 2026

Pivotal Phase 2 trial of RP-A501 for Danon disease progressing with dosing reinitiated

First patient dosing in Phase 1 study of RP-A701 for BAG3-related dilated cardiomyopathy anticipated in mid-2026

KRESLADI™ granted FDA accelerated approval; Rare Pediatric Disease Priority Review Voucher monetized for $180 million

Pro forma cash, cash equivalents and investments of approximately $322.6 million, including balance as of March 31, 2026, and non-dilutive proceeds from the PRV sale; expected operational runway into the second quarter of 2028

CRANBURY, N.J.--(BUSINESS WIRE)--May 7, 2026-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, commercial-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 ended March 31, 2026.

“During the first quarter, we continued advancing our cardiovascular gene therapy portfolio, including reinitiating dosing in the initial three-patient cohort of our pivotal Phase 2 study of RP-A501 for Danon disease, while also achieving the accelerated approval of KRESLADI for severe LAD-I,” said Gaurav Shah. “The subsequent $180 million non-dilutive monetization of our Rare Pediatric Disease Priority Review Voucher further strengthened our balance sheet and extended our expected cash runway into the second quarter of 2028. We remain focused on disciplined execution across our deep cardiovascular pipeline and anticipate providing an update on the Danon program in the second half of the year.”

Recent Pipeline and Operational Updates

- **Dosing the initial three-patient cohort for the Phase 2 study of RP-A501 in Danon disease is on track.**
  - Rocket previously disclosed that the U.S. Food and Drug Administration’s (FDA) lifted the clinical hold on the Company’s pivotal Phase 2 trial of RP-A501 for the treatment of Danon disease in under three months.
  - Per agreement with FDA, three additional patients are being treated at a recalibrated dose of 3.8×10^{13} GC/kg with a minimum four-week inter-patient dosing interval and a modified immunomodulatory regimen. Following the treatment of these three patients, Rocket will align with FDA regarding the completion of the Phase 2 pivotal study.
  - Following FDA alignment, Rocket anticipates providing a program update in the second half of 2026.
  - Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.

- **Engagement with FDA is ongoing regarding RP-A601 for PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM).**
  - Rocket continues to engage with FDA on alignment for a potential pivotal Phase 2 trial design for RP-A601 in PKP2-ACM, while 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 start-up activities are ongoing for RP-A701 in BAG3-associated dilated cardiomyopathy (BAG3-DCM).**
  - 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. Dosing of the first patient is anticipated in mid-2026.
  - Details of the Phase 1 study can be found at www.ClinicalTrials.gov under NCT identifier NCT07137338.

- **FDA granted accelerated approval for KRESLADI™ (marnetegragene autotemcel) for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).**

- In March 2026, FDA granted KRESLADI™ accelerated approval for the treatment of pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen-matched sibling donor for allogeneic hematopoietic stem cell transplant. This indication is approved under accelerated approval based on increase in neutrophil CD18 and CD11a surface expression.
- On April 28, 2026, Rocket announced a definitive agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV) for $180 million.

First Quarter 2026 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of March 31, 2026, were $144.4 million, excluding PRV monetization.
- **R&D expenses.** Research and development expenses were $31.5 million for the three months ended March 31, 2026, compared to $35.9 million for the three months ended March 31, 2025. The decrease of $4.4 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $5.8 million, stock-based and other compensation and benefit expenses of $2.0 million due to decreased R&D headcount, partially offset by increases in clinical trial expenses of $2.8 million and professional fees of $0.6 million. The reduction reflects disciplined resource allocation following the company's recent organizational realignment.
- **G&A expenses.** General and administrative expenses were $17.1 million for the three months ended March 31, 2026, compared to $28.4 million for the three months ended March 31, 2025. The decrease of $11.3 million in G&A expenses was primarily driven by decreases in legal expenses of $5.7 million due to a legal settlement in 2025, decrease in commercial preparation-related expenses of $4.6 million due to lower headcount and lower spending on commercial launch, and stock-based and other compensation and benefit expenses of $2.8 million due to decreased G&A headcount, partially offset by an increase in milestone related expenses of $2.4 million.
- **Net loss.** Net loss was $47.6 million or $0.42 per share (basic and diluted) for the three months ended March 31, 2026, compared to $61.3 million or $0.56 (basic and diluted) for the three months ended March 31, 2025.
- **Shares outstanding.** 109,123,671 shares of common stock were outstanding as of March 31, 2026.

Financial Guidance

- **Cash position.** As of March 31, 2026, Rocket had cash, cash equivalents and investments of $144.4 million. Rocket expects such resources, together with proceeds from the sale of the Priority Review Voucher, to be sufficient to fund its 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 biotechnology company advancing gene therapies for rare and devastating cardiovascular diseases, with additional programs in hematology and immunology. Rocket's cardiovascular pipeline includes three clinical stage programs that each target one of the major inherited cardiomyopathy subtypes: hypertrophic, arrhythmogenic, and dilated cardiomyopathies. Together these conditions represent more than 100,000 patients in the U.S. and EU. The Company's platform is supported by proprietary AAV manufacturing capabilities, multi-year efficacy and safety data in cardiac gene therapy, and experience treating several cardiac patients across late-stage AAV programs. 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 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 March 31,	
	2026	**2025**
Operating expenses:		
Research and development	$ 31,454	$ 35,942
General and administrative	17,057	28,446
Total operating expenses	48,511	64,388
Loss from operations	(48,511)	(64,388)
Interest expense	(473)	(472)
Interest and other income, net	161	1,336
Accretion of discount on investments, net	1,229	2,190
Net loss	$ (47,594)	$ (61,334)
Net loss per share - basic and diluted	$ (0.42)	$ (0.56)
Weighted-average common shares outstanding - basic and diluted	112,134,059	110,093,461

	March 31, 2026	**December 31, 2025**
Cash, cash equivalents, and investments	$ 144,379	$ 188,929
Total assets	285,407	330,449
Total liabilities	47,368	53,228
Total stockholders' equity	238,039	277,221

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