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

November 12, 2026

FDA alignment establishes path to complete the pivotal Phase 2 trial of RP-A501 in Danon disease; dosing completion expected by mid-2027

October Danon disease investor webinar highlighted updated longer-term RP-A501 Phase 1 findings and encouraging initial safety observations in the first three patients treated under the modified Pivotal Phase 2 protocol

Dosing initiated in the Phase 1 trial of RP-A701 for BAG3-associated dilated cardiomyopathy

KRESLADI™ achieved commercial readiness on October 1, 2026; Qualified Treatment Centers activated and ready to begin commercial patient onboarding

Cash, cash equivalents and investments of approximately $279.4 million as of September 30, 2026, including the initial $35 million Hercules draw, are expected to fund planned operations into Q3 2028. Additional borrowings could extend runway into 2029, subject to borrowing conditions, draw timing and amounts, and operating expenditures.

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 12, 2026-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and life-threatening diseases, with a strategic focus on inherited cardiovascular disorders, today reported financial results for the third quarter ended September 30, 2026, and provided recent business updates.

"With FDA alignment on the pivotal RP-A501 trial in Danon disease, our priority is to complete dosing by mid-2027," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. "We are also advancing our broader cardiovascular portfolio, with dosing initiated in the Phase 1 trial of RP-A701 for BAG3-DCM and continued FDA engagement on a potential pivotal study of RP-A601 in PKP2-ACM. Commercial readiness for KRESLADI and activation of our first Qualified Treatment Centers bring us closer to treating eligible children. Our financial resources and the additional flexibility provided by the Hercules facility support these priorities as we work toward our next clinical and commercial milestones."

Recent Pipeline and Operational Updates

- **FDA Alignment Establishes Path to Complete RP-A501 Pivotal Phase 2 Trial in Danon Disease**
  - On September 15, 2026, Rocket announced FDA alignment on continued enrollment and dosing and key elements of the global pivotal Phase 2 trial of RP-A501 in Danon disease, including the recalibrated dose, 12-patient pivotal population and established 12-month co-primary endpoints. The first three patients treated under the modified protocol count toward the 12-patient pivotal efficacy population. At the 12-month assessment, at least seven of the 12 patients must show both myocardial LAMP2 expression at Grade 1 or higher and a decrease of 10% or more from baseline in left ventricular mass index to meet the pivotal efficacy success criterion. The co-primary endpoints are intended to support a potential accelerated approval pathway.
  - On October 6, Rocket hosted an investor webinar, highlighting key findings and development milestones for RP-A501, including updated longer-term Phase 1 follow-up and encouraging initial safety observations in the first three patients treated under the modified protocol. Rocket reviewed updated Danon disease epidemiology, with independent genotype- and phenotype-based analyses estimating 10,000-11,000 people living with Danon disease in the U.S., including approximately 4,000 males. Rocket has identified more than 900 Danon patients. Prevalence estimates may include undiagnosed or asymptomatic individuals and do not indicate treatment eligibility.
  - Rocket expects to complete pivotal trial dosing by mid-2027, with topline results anticipated in mid-2028.

- **Continued Advancement of RP-A601 in PKP2 Arrhythmogenic Cardiomyopathy (PKP2-ACM)**
  - Rocket continues to advance RP-A601 for PKP2-ACM through its ongoing Phase 1 study and engagement with the FDA regarding the next stage of clinical development. Previously reported Phase 1 findings showed increased

PKP2 protein expression and improved desmosomal localization, together with directional improvements in arrhythmia measures and right ventricular function. The Company will provide further clinical and regulatory updates as the program progresses.
    - 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.

- **Dosing Initiated in Phase 1 Trial of RP-A701 for BAG3-Associated Dilated Cardiomyopathy (BAG3-DCM); EMA Orphan Designation Granted**
    - Rocket has initiated dosing in the Phase 1 trial of RP-A701 for BAG3-DCM, marking an additional clinical milestone for Rocket's cardiovascular gene therapy portfolio.
    - 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.
    - On October 20, 2026, the European Commission granted orphan designation to RP-A701 for the treatment of BAG3-DCM.

- **KRESLADI™ (marnetegragene autotemcel) Achieves Commercial Readiness; Qualified Treatment Centers Activated**
    - Rocket achieved commercial readiness for KRESLADI™ on October 1, 2026. Two Qualified Treatment Centers have been activated and ready to begin commercial patient onboarding, and Rocket's external manufacturing network is in place.

- **Strategic Credit Facility Provides Access to Up to $150 Million to Support Cardiovascular Pipeline**
    - On October 6, 2026, Rocket announced a credit facility agreement with Hercules Capital, Inc. for up to $150 million to support execution of the pivotal Phase 2 trial of RP-A501 in Danon disease and continued advancement of its broader cardiovascular pipeline. Rocket received $35 million at closing and may draw an additional $35 million at its option during specified periods, subject to the agreement's terms and conditions. An additional $30 million may become available upon achievement of a specified Danon clinical milestone, and an additional $50 million is subject to Hercules' investment committee approval.

Third Quarter 2026 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2026, were $279.4 million.
- **R&D expenses.** Research and development expenses were $31.2 million for the three months ended September 30, 2026, compared to $34.1 million for the three months ended September 30, 2025. The decrease of $2.9 million in R&D expenses was primarily driven by reductions in stock-based and other compensation and benefits expense of $3.5 million due to decreased R&D headcount, decreases in manufacturing and development and direct material costs of $1.9 million and depreciation expenses of $1.2 million due to decreased asset base. These decreases were partially offset by increases in expenses for laboratory-related expenses of $1.4 million, professional fees of $1.4 million, and clinical trial expenses of $0.6 million.
- **G&A expenses.** General and administrative expenses were $19.8 million for the three months ended September 30, 2026, compared to $18.4 million for the three months ended September 30, 2025. The increase of $1.4 million in G&A expenses was primarily driven by increases in post marketing commitment expenses of $3.4 million and commercial preparation related expenses of $0.6 million. These increases were partially offset by decreases in stock-based and other compensation and benefit expenses of $2.2 million due to decreased G&A headcount and legal expenses of $0.6 million.
- **Other Income.** Other Income was $1.8 million for the three months ended September 30, 2026, compared to $1.9 million for the three months ended September 30, 2025. The decrease in other income was primarily driven by a decline in accretion of discount on investments, net, of $0.4 million partially offset by an increase in interest income of $0.3 million.
- **Income Taxes.** Income taxes were $0.1 million for the three months ended September 30, 2026, compared to $0.0 million for the three months ended September 30, 2025, because of a change in estimated income taxes payable during the three months ended September 30, 2026.
- **Net income.** Net loss was $49.2 million or $0.43 per share (basic and diluted) for the three months ended September 30, 2026, compared to $50.3 million or $0.45 (basic and diluted loss per share) for the three months ended September 30, 2025.
- **Shares outstanding.** 111,386,536 shares of common stock were outstanding as of September 30, 2026.

Financial Guidance

- **Cash position.** As of September 30, 2026, Rocket had cash, cash equivalents and investments of $279.4 million. Based on its current operating plan, Rocket expects its cash, cash equivalents and investments as of September 30, 2026, to

fund operations into the third 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 life-threatening 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 adeno-associated virus (AAV) manufacturing capabilities and extensive clinical experience in cardiac gene therapy.

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 actual results to differ materially from those expressed or implied by such forward-looking statements. Rocket makes such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other applicable federal securities laws. All statements other than statements of historical fact contained in this release are forward-looking statements. These statements may be identified by words such as "believe," "expect," "anticipate," "intend," "plan," "estimate," "seek," "will," "may," "potential" and similar expressions, or their negatives. These forward-looking statements include, but are not limited to, statements concerning Rocket's plans to advance its genetic medicines portfolio; the enrollment, dosing, completion and anticipated results of ongoing and planned clinical trials; the timing and outcome of regulatory interactions and potential approval pathways; estimated prevalence of Danon disease, patient identification efforts and the size of addressable patient populations; KRESLADI commercialization and patient access; expected cash runway and the sufficiency of financial resources; the availability of additional borrowings under the Hercules credit facility and satisfaction of applicable borrowing conditions; and Rocket's development strategy and priorities.

Although Rocket believes that the expectations reflected in these forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially as a result of various important factors, including, without limitation: the results, timing and costs of Rocket's ongoing and planned clinical trials; its ability to identify, enroll and dose eligible patients and successfully complete clinical studies; unexpected adverse events or other safety findings; the timing and outcome of regulatory interactions, submissions and review; manufacturing, product comparability, quality and supply considerations; Rocket's dependence on third parties for development, manufacturing, commercialization and other activities; its ability to successfully commercialize its products; its future capital requirements and ability to obtain additional funding; its ability to satisfy conditions for additional borrowings, comply with credit facility covenants and meet debt service obligations; changes in its operating plan, development priorities, expenses or cash requirements; its ability to obtain, maintain and enforce intellectual property protection and defend against third-party infringement claims; and competing therapies and technological developments. Additional risks are described under "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2025, filed with the Securities and Exchange Commission on February 26, 2026, and in its subsequent SEC filings, including its Quarterly Reports on Form 10-Q.

Accordingly, readers 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, except as required by law.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 31,172	$ 34,068	$ 92,123	$ 112,668
General and administrative	19,793	18,351	54,275	71,817
Restructuring	-	(172)	-	3,299
Total operating expenses	50,965	52,247	146,398	187,784
Loss from operations	(50,965)	(52,247)	(146,398)	(187,784)
Gain from sale of PRV	-	-	178,190	-

Interest expense	(473)	(473)	(1,419)	(1,418)
Interest and other income, net	1,014	709	1,522	2,528
Accretion of discount on investments, net	1,244	1,679	3,166	6,089
(Loss) income before Income Taxes	(49,180)	(50,332)	35,061	(180,585)
Provision for Income Taxes	(54)	-	(8,675)	-
Net (loss) income	$ (49,234)	$ (50,332)	$ 26,386	$ (180,585)
Net (loss) income per share - basic	$ (0.43)	$ (0.45)	$ 0.23	$ (1.63)
Net (loss) income per share - diluted	$ (0.43)	$ (0.45)	$ 0.23	$ (1.63)
Weighted-average common shares outstanding - basic	113,647,401	111,571,136	112,880,990	110,900,161
Weighted-average common shares outstanding - diluted	113,647,401	111,571,136	114,706,711	110,900,161

	September 30, 2026	December 31, 2025
Cash, cash equivalents, and investments	$ 279,445	$ 188,929
Total assets	419,504	330,449
Total liabilities	92,780	53,228
Total stockholders' equity	326,724	277,221

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