



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

November 6, 2025

Pivotal Phase 2 trial of RP-A501 for Danon disease to resume in 1H 2026

KRESLADI™ for severe LAD-I on track for March 28, 2026 PDUFA date

Leadership updates with the appointments of Dr. Syed Rizvi, Chief Medical Officer, Christopher Stevens, Chief Operating Officer, and Sarbani Chaudhuri, Chief Commercial & Medical Affairs Officer

Cash, cash equivalents and investments of approximately \$222.8M; expected operational runway into the second quarter of 2027

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 6, 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 third quarter ending September 30, 2025.

"During the third quarter, we maintained disciplined execution and sharpened our strategic focus on Rocket's AAV cardiovascular gene therapy portfolio," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. "In under three months, we aligned with the FDA to resume the Phase 2 pivotal study of RP-A501 for Danon disease, an important milestone that underscores the FDA's collaborative approach and recognition of the urgent need for innovative therapies in rare cardiovascular disorders. In parallel, we are advancing RP-A601 toward a pivotal Phase 2 study in PKP2-ACM and preparing RP-A701 for first-in-human evaluation in BAG3-DCM. Finally, the upcoming March PDUFA date for KRESLADI™ represents a significant near-term commercial milestone as we work to bring this potentially life-saving therapy to patients with severe LAD-I."

Recent Pipeline and Operational Updates

- **Dosing of additional patients for the Phase 2 study of RP-A501 for Danon disease anticipated in the first half of 2026.**
 - In August, Rocket [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 the FDA, three additional patients will be treated at a recalibrated dose of 3.8×10^{13} GC/kg with a minimum four-week interval between dosing and a modified immunomodulatory regimen. Following the treatment of these three patients, Rocket will align with the FDA regarding the path forward for completion of the Phase 2 pivotal study.
 - Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
- **Engagement with the FDA is ongoing regarding RP-A601 for PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM).**
 - Rocket continues to collaborate with the FDA to align upon potential pivotal Phase 2 trial design to evaluate the efficacy and safety of RP-A601 for PKP2-ACM.
- **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.
 - Details of the Phase 1 study can be found at www.ClinicalTrials.gov under NCT identifier NCT07137338.
- **FDA accepted the resubmission of the BLA for KRESLADI™ (marnetegrage autotemcel; marne-cel) for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).**
 - In October, KRESLADI™ [received](#) a Prescription Drug User Fee Act (PDUFA) target action date for March 28,

2026.

- o Rocket is eligible for a Rare Pediatric Disease Priority Review Voucher (PRV), with the approval of KRESLADI™.

- **Leadership updates with several key appointments to support Rocket's transition toward late-stage development and anticipated commercialization.**

- o In September 2025, Syed Rizvi, M.D., was named Chief Medical Officer, adding over 20 years of clinical strategy, development and commercialization experience and a proven track record in cell and gene therapy, guiding the development and commercialization of multiple cell therapies for cancer treatments, including three approved autologous CAR-T cell therapies. Dr. Rizvi most recently served as Chief Medical Officer at Poseida Therapeutics, where he advanced the allogeneic CAR-T and gene therapy pipeline through the Company's acquisition by Roche in January 2025. Previously, Dr. Rizvi held global clinical leadership roles at Legend Biotech, Celgene, Novartis, Merck, Caribou Biosciences and Genta Inc.
- o Christopher Stevens, appointed Chief Operating Officer in July, adding more than two decades of global operations, product strategy, manufacturing, and supply chain leadership experience in the biopharmaceutical industry. Mr. Stevens previously served as Executive Vice President and Chief Patient Supply Officer at Spark Therapeutics, where he oversaw end-to-end gene therapy manufacturing and supply, and held senior operational roles at GlaxoSmithKline and Bristol Myers Squibb.
- o Sarbani Chaudhuri, joined as Chief Commercial & Medical Affairs Officer in April, bringing more than 20 years of leadership in delivering multiple pioneering therapeutics from development to blockbuster launches that have transformed patient outcomes and been central to enterprise growth. Most recently, she led hematology at Johnson & Johnson, and previously held business leadership roles at AstraZeneca, Pfizer, and Novartis in oncology and rare diseases.
- o Jonathan Schwartz, M.D., appointed Chief Science and Gene Therapy Officer, reflecting Rocket's continued commitment to scientific and technical excellence as the Company advances toward late-stage development and commercialization. A founding member of Rocket's leadership team and founding Chief Medical Officer, Dr. Schwartz has been instrumental in building and progressing Rocket's industry-leading gene therapy pipeline across cardiology and hematology programs. In his expanded role, he will lead the strategic advancement and application of gene therapy technologies to current and future therapeutic areas, driving innovation across Rocket's platform.

Third Quarter 2025 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2025, were \$222.8 million. Rocket expects such resources, excluding any potential future 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 \$34.1 million for the three months ended September 30, 2025, compared to \$42.3 million for the three months ended September 30, 2024. The decrease of \$8.2 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of \$3.6 million, clinical trial expenses of \$2.4 million, and professional fees of \$2.0 million. The reduction reflects disciplined resource allocation following the company's recent organizational realignment.
- **G&A expenses.** General and administrative expenses were \$18.4 million for the three months ended September 30, 2025, compared to \$27.1 million for the three months ended September 30, 2024. The decrease of \$8.7 million in G&A expenses was primarily driven by decreases in commercial preparation-related expenses of \$6.6 million and non-cash stock-based compensation expense of \$1.5 million.
- **Net loss.** Net loss was \$50.3 million or \$0.45 per share (basic and diluted) for the three months ended September 30, 2025, compared to \$66.7 million or \$0.71 (basic and diluted) for the three months ended September 30, 2024.
- **Shares outstanding.** 108,208,643 shares of common stock were outstanding as of September 30, 2025.

Restructuring Expenses and Financial Guidance

- **Restructuring expenses.** Approximately \$3.3 million in restructuring and restructuring-related charges were incurred in 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 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. 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	\$ 34,068	\$ 42,315	\$ 112,668	\$ 133,887
General and administrative	18,351	27,109	71,817	76,624
Restructuring	(172)	-	3,299	-
Total operating expenses	52,247	69,424	187,784	210,511
Loss from operations	(52,247)	(69,424)	(187,784)	(210,511)
Interest expense	(473)	(471)	(1,418)	(1,413)
Interest and other income, net	709	1,327	2,528	6,650
Accretion of discount on investments, net	1,679	1,849	6,089	6,855
Net loss	(50,332)	(66,719)	(180,585)	(198,419)
Net loss per share - basic and diluted	\$ (0.45)	\$ (0.71)	\$ (1.63)	\$ (2.11)
Weighted-average common shares outstanding - basic and diluted	111,571,136	94,158,491	110,900,161	93,893,729
	September 30, 2025	December 31, 2024		
Cash, cash equivalents, and investments	\$ 222,758	\$ 372,336		
Total assets	368,033	527,700		
Total liabilities	54,364	64,466		
Total stockholders' equity	313,669	463,234		

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