



Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2025 Financial Results and Highlights Recent Progress

February 26, 2026

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

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

Cash, cash equivalents and investments of approximately \$188.9M; expected operational runway into the second quarter of 2027

CRANBURY, N.J.--(BUSINESS WIRE)--Feb. 26, 2026-- [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 fourth quarter and year ended December 31, 2025.

"In 2025, we strengthened Rocket's leadership in cardiovascular gene therapy, supported by more than five years of clinical experience in Danon disease and continued advancement across our PKP2-ACM and BAG3-DCM programs," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. "Our focus remains on rigorous clinical execution across three cardiovascular programs, including resuming the Phase 2 pivotal clinical trial in Danon disease, initiating the Phase 1 clinical trial in BAG3-DCM, and advancing alignment with the FDA on the pivotal study design for PKP2-ACM. As we move through 2026, Rocket is well positioned to advance three high-impact cardiovascular programs and thoughtfully expand our cardiac gene therapy pipeline."

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 2025, 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 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 work closely with the FDA to advance alignment on 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. Rocket expects to dose the first BAG3-DCM patient mid-2026.
 - 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™ (marnetegragene autotemcel; marne-cel) for the**

treatment of severe leukocyte adhesion deficiency-I (LAD-I).

- o In October 2025, 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™.

Fourth Quarter and Full Year 2025 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of December 31, 2025, were \$188.9 million.
- **R&D expenses.** Research and development expenses were \$142.0 million for the twelve months ended December 31, 2025, compared to \$171.2 million for the twelve months ended December 31, 2024. The decrease of \$29.2 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of \$10.8 million, professional fees of \$7.0 million, lab supplies and office expenses of \$4.4 million, stock-based and other compensation and benefit expenses of \$3.7 million, and clinical trial expenses of \$2.7 million. The reduction reflects disciplined resource allocation following the company's recent organizational realignment.
- **G&A expenses.** General and administrative expenses were \$86.5 million for the twelve months ended December 31, 2025, compared to \$102.0 million for the twelve months ended December 31, 2024. The decrease of \$15.5 million in G&A expenses was primarily driven by decreases in commercial preparation-related expenses of \$11.5 million from declining payroll and commercial launch services and stock-based and other compensation and benefit expenses of \$5.4 million, partially offset by increase in legal expenses of \$1.4 million.
- **Net loss.** Net loss was \$223.1 million or \$2.01 per share (basic and diluted) for the twelve months ended December 31, 2025, compared to \$258.7 million or \$2.73 (basic and diluted) for the twelve months ended December 31, 2024.
- **Shares outstanding.** 108,319,783 shares of common stock were outstanding as of December 31, 2025.

Restructuring Expenses and Financial Guidance

- **Restructuring expenses.** Approximately \$3.2 million in restructuring and restructuring-related charges were incurred in 2025.
- **Cash position.** As of December 31, 2025, Rocket had cash, cash equivalents and investments of \$188.9 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.

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 "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 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 establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop its direct manufacturing capabilities for its AAV programs, 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, 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'

