Rocket Pharmaceuticals Announces Strategic Corporate Reorganization and Pipeline Prioritization of Cardiovascular Programs

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Restructuring pipeline to focus on AAV cardiovascular platform (Danon disease, PKP2-ACM, BAG3-DCM) and regulatory activities for KRESLADI ™ (severe LAD-I), setting foundation for near- and long-term value creation

Organizational restructuring expected to reduce headcount by approximately 30% and lower 12-month cash burn by nearly 25%

Company expects current cash runway to fund operations into the second quarter of 2027

CRANBURY, N.J.--(BUSINESS WIRE)--Jul. 24, 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 announced a strategic corporate reorganization and pipeline prioritization aimed at maximizing near-term value, extending operational runway into the second quarter of 2027, and positioning the company for sustained long-term growth.

The restructuring and reprioritization initiative focuses Rocket's resources on the adeno-associated virus (AAV) cardiovascular platform, comprised of clinical programs in Danon disease, PKP2-associated arrhythmogenic cardiomyopathy (PKP2-ACM), and BAG3-associated dilated cardiomyopathy (BAG3-DCM), as well as submitting the completed responses to the CRL for KRESLADI™ (formerly RP-L201) for severe leukocyte adhesion deficiency-I (LAD-I). The transition reflects the company's focus on its late-stage AAV pipeline, a strategy previously announced in January 2025.

The company will implement a reduction in workforce of approximately 30%, which, along with other planned cost-saving initiatives, is expected to reduce Rocket's 12-month operating expenses by nearly 25%. Rocket anticipates that its existing cash resources, excluding any potential proceeds from a Priority Review Voucher that may be granted upon FDA approval of KRESLADI™, will fund operations into the second quarter of 2027.

"We are grateful for the remarkable contributions of our entire team and the significant progress we've made in advancing genetic therapies for rare diseases," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. "Our prioritization reflects a commitment to the programs with the most compelling near-term opportunities for patients and to setting a foundation for Rocket's long-term growth and success. Together, our cardiovascular programs have demonstrated strong potential for impact in areas of high unmet need in substantial patient populations."

Rocket is advancing its leadership in cardiovascular gene therapy by strategically leveraging its AAV platform to address monogenic cardiomyopathies with high unmet need. With a portfolio spanning Danon disease, PKP2-ACM, and BAG3-DCM, Rocket is uniquely positioned to develop one-time, potentially curative treatments for distinct molecular subtypes of heart failure. Collectively, these programs target the major genetically defined causes of hypertrophic, arrhythmogenic, and dilated cardiomyopathies which represent a significant portion of inherited heart disease and impact approximately more than 100,000 patients in the U.S. and EU.

Based on business and strategic priorities and as part of its realignment, Rocket anticipates delays associated with the Fanconi Anemia (FA; RP-L102) and Pyruvate Kinase Deficiency (PKD; RP-L301) programs. FDA approval of RP-L102 is no longer anticipated in 2026. These adjustments reflect a disciplined approach to capital allocation and a strategic reprioritization of assets. Additional information is expected to be provided with second quarter earnings updates as the company continues to evaluate strategic options.

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 Rocket's ability to realize the intended benefits of the restructuring plan and reduction in workforce, 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. There can be no assurance that the restructuring plan or the planned reduction in workforce will have the intended effect on the Company's operational results and strategic decisions, that any anticipated charges and any anticipated cost savings associated with the restructuring plan or the reduction in workforce will achieve their intended benefits. 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.

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Investors
Meg Dodge
mdodge@rocketpharma.com

Media
Kevin Giordano
media@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.