Rocket Pharmaceuticals Announces Positive Clinical Safety Update from Initial Three Patients Treated with RP-A501 Under Modified Phase 2 Protocol for Danon Disease

August 3, 2026

Initial three patients were treated safely; RP-A501 was well-tolerated with no TMA or capillary leak syndrome observed

Rocket is actively engaging with the FDA to align on the path to pivotal trial completion; regulatory pathway update expected in the second half of 2026

Comprehensive Danon disease program update on track for the second half of 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 3, 2026-- Rocket Pharmaceuticals, Inc. (Rocket or the Company) (NASDAQ: RCKT), a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and devastating diseases, focused on inherited cardiovascular disorders, today announced a positive clinical safety update regarding the initial three patients treated under the modified protocol for its global, pivotal Phase 2 trial of RP-A501 in Danon disease. Early safety has been promising with the Company now engaging with the U.S. Food and Drug Administration (FDA) to align on the pathway for treating additional patients and completing the pivotal Phase 2 trial under the modified protocol.

The patients received RP-A501 at the recalibrated dose of 3.8×10^{13} GC/kg together with a refined immunomodulatory regimen comprised of rituximab, sirolimus, and corticosteroids. Treatment proceeded sequentially, with a minimum of four weeks between infusions. To date, no thrombotic microangiopathy (TMA), capillary leak syndrome, or other significant safety concerns have been observed in these patients. The pivotal Phase 2 trial was designed as a 12-patient, single-arm study. The Company is actively engaging with the FDA to align on the path to dosing additional patients and completing the trial and expects to provide an update on the regulatory pathway in the second half of 2026.

“These encouraging early safety observations from the initial three patients treated with RP-A501 reinforce our strong confidence in the program that was built on transformative results from the Phase 1 trial which reflect the potential of gene therapy to address genetic cardiomyopathies,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. “We are working with the FDA to align on the path to completing the pivotal Phase 2 trial as promptly as possible.”

“The recalibrated Phase 2 dose was selected with the expectation that it will deliver potency consistent with the dose at which RP-A501 demonstrated meaningful efficacy in patients in Phase 1,” said Syed Rizvi, M.D., Chief Medical Officer of Rocket Pharmaceuticals. “This recalibration accounts for the higher proportion of full capsids in the current drug product in the setting of Danon disease and was developed in consultation with leading experts and the FDA. We anticipate that the dose will preserve RP-A501’s therapeutic potential while optimizing its benefit-risk profile.”

The Company remains on track to provide a comprehensive Danon disease program update in the second half of 2026.

About RP-A501
RP-A501 is Rocket’s investigational gene therapy for the treatment of Danon disease and the first gene therapy for a cardiovascular condition to demonstrate safety and efficacy in clinical studies. RP-A501 has the potential to restore or stabilize cardiac function in patients with Danon disease. RP-A501 consists of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a functional version of the human *LAMP2B* transgene (AAV9.LAMP2B) which is administered as a single intravenous (IV) infusion. In clinical studies, RP-A501 has been shown to target cardiac cells (cardiomyocytes) and deliver the functional *LAMP2B* gene to heart tissue, which led to improved cardiac structure and function in patients. RP-A501 holds FDA RMAT, Fast Track, Rare Pediatric Disease, and Orphan Drug designations in the U.S. along with ATMP and PRIME designations in the EU.

About Danon Disease
Danon disease is a rare X-linked inherited, multi-organ lysosomal-associated disorder with a devastating clinical course. The causative mutation has been identified in the gene encoding for lysosome-associated membrane protein, otherwise known as *LAMP2*, an important mediator of autophagy and primarily expressed in heart, skeletal muscle and brain tissue. This mutation results in accumulation of autophagosomes and glycogen, particularly in cardiac muscle and other tissues, which ultimately leads to heart failure, and for male patients, frequent death during adolescence or early adulthood. The only available definitive treatment option for Danon disease is cardiac transplantation, which is associated with substantial complications and is not considered curative, representing the high unmet medical need for patients with Danon disease.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and devastating 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 AAV manufacturing capabilities, extensive clinical experience in cardiac gene therapy, and long-term safety and efficacy data supporting advancement across its cardiovascular portfolio.

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 the safety and effectiveness of RP-A501 in its pivotal Phase 2 trial under the modified protocol, the Company's ability to align with the FDA on the path to treating additional Danon disease patients, Rocket's cash runway and financial position, 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 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.

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