# Rocket Pharmaceuticals Announces FDA Alignment on Path to Complete Pivotal Phase 2 Trial of RP-A501 in Danon Disease

September 15, 2026

FDA confirms pivotal efficacy population of 12 patients, including the first three treated under the modified protocol

FDA review of initial safety data supports continued enrollment and dosing under modified protocol

FDA confirms established 12-month co-primary endpoints intended to support potential accelerated approval

Comprehensive Danon disease program webinar planned for October 6, 2026, at 4:30 p.m. ET

CRANBURY, N.J.--(BUSINESS WIRE)--Sep. 15, 2026-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and life-threatening diseases, focused on inherited cardiovascular disorders, today announced alignment with the U.S. Food and Drug Administration (FDA) on continued enrollment and dosing and key elements of the global pivotal Phase 2 trial of RP-A501 for the treatment of Danon disease, including the recalibrated dose, 12-patient pivotal population and established 12-month co-primary endpoints.

The clinical data reviewed by FDA included results from the first three patients treated under the modified protocol. All three completed at least four weeks of follow-up and had been discharged following protocol-specified observation. No clinical or laboratory evidence of thrombotic microangiopathy or capillary leak syndrome had been observed. Following its review of the available safety data, the FDA confirmed continued enrollment and dosing under the modified protocol at the recalibrated dose.

The FDA also confirmed that the pivotal study population will include 12 male patients treated with RP-A501 at the recalibrated dose of $3.8 \times 10^{13}$ genome copies per kilogram (GC/kg) using commercial-grade product. Importantly, the first three patients treated at the recalibrated dose under the modified protocol count toward the 12-patient pivotal population, leaving nine additional patients to be enrolled and treated. Rocket expects to complete dosing of the remaining patients by mid-2027.

The primary assessment will occur at 12 months using the previously established co-primary endpoints of myocardial LAMP2 protein expression and a 10% reduction from baseline in left ventricular mass index. These endpoints are intended to support a potential accelerated approval pathway.

“FDA’s confirmation of the pivotal efficacy framework marks an important milestone for RP-A501 and the Danon disease community,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharmaceuticals. “With the first three patients counting toward the 12-patient pivotal population and the established co-primary endpoints preserved, we now have a clear and actionable path to complete the pivotal study. The initial clinical experience at the recalibrated dose and supportive product-bridging data further reinforce the path forward.”

The modified protocol incorporates a recalibrated RP-A501 dose and an optimized immunomodulatory regimen comprised of rituximab, sirolimus and corticosteroids, together with enhanced eligibility criteria, safety monitoring and risk-mitigation measures. The recalibrated dose was selected based on analytical characterization of the Phase 2 commercial-grade product, including its full-particle content, and is supported by nonclinical bridging data.

Nonclinical bridging data further support the pharmacologic activity of the recalibrated dose. In a Danon disease mouse model, administration of Phase 2 material at $3.8 \times 10^{13}$ GC/kg resulted in cardiac LAMP2B protein expression comparable to that observed at a higher dose and demonstrated comparable vector biodistribution.

Rocket plans to provide a comprehensive update on the Danon disease program during a virtual investor webinar on Tuesday, October 6, 2026, at 4:30 p.m. ET. Additional information, including webcast access details, will be available in the Investors section of the Company’s website.

**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, 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 Rocket's cash runway and financial position, Rocket's planned use of proceeds from the monetization of the KRESLADI™ PRV, Rocket's expectations of our ability to obtain additional funding to conduct our planned research and development efforts, 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, 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 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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