



Rocket Pharmaceuticals Highlights FDA-Aligned Pivotal Phase 2 Trial and Updated Clinical Findings for RP-A501 Following Danon Disease Investor Webinar

October 7, 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 7, 2026-- [Rocket Pharmaceuticals, Inc.](#) (NASDAQ: RCKT), a fully integrated commercial-stage biotechnology company advancing genetic medicines for rare and life-threatening diseases, highlighted key findings and development milestones presented during its October 6 Danon disease investor webinar. The update outlined a defined path to completing the FDA-aligned pivotal Phase 2 trial, highlighted sustained biological activity in longer-term Phase 1 follow-up and reported encouraging initial safety observations in the first three patients treated under the modified protocol.

Key highlights included:

- **Pivotal Phase 2 trial:** The FDA-aligned pivotal efficacy population includes 12 male patients receiving commercial-grade RP-A501 at the recalibrated dose. The first three patients treated under the modified protocol count toward this population. Trial success requires at least seven patients to meet both response criteria at 12 months: myocardial LAMP2 protein expression of at least Grade 1 and a reduction in left ventricular mass index of at least 10% from baseline. These co-primary endpoints are intended to support a potential accelerated approval pathway.
- **Modified protocol:** The first three patients treated at the recalibrated dose completed the protocol-specified initial observation period without clinical or laboratory evidence of thrombotic microangiopathy or capillary leak syndrome, as reported during the webinar. Follow-up is ongoing, and these initial observations are preliminary.
- **Phase 1 results:** At approximately 12 months, all six patients in the principal Phase 1 analysis demonstrated myocardial LAMP2 protein expression and at least a 10% reduction in left ventricular mass index. At follow-up of approximately three to seven years, left ventricular mass index was below baseline in five of six patients, with reductions of at least 10% in four patients. All six patients also had sustained reduction of cardiac biomarkers and improvements of quality of life.
- **Original-dose Phase 2 results:** Three of four patients with longitudinal efficacy assessments achieved at least a 10% reduction in left ventricular mass index. Safety findings from the original-dose study, including thrombotic microangiopathy and a fatal serious adverse reaction, informed the recalibrated dose, revised immunomodulation regimen and enhanced safety monitoring now in place.
- **Patient population:** Genetic and clinical analyses converged on a modeled U.S. Danon disease population of approximately 10,000–11,000, including 4,000 males. Rocket's initial focus is males with symptomatic hypertrophic cardiomyopathy.
- **Upcoming milestones:** Rocket expects to complete pivotal trial dosing by mid-2027, with topline results anticipated mid-2028.

A replay of the webinar and the accompanying presentation are available in the Investors section of Rocket's website at ir.rocketpharma.com.

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 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 and extensive clinical experience in cardiac gene therapy.

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 actual results to differ materially from those expressed or implied by such forward-looking statements. Rocket makes such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other applicable federal securities laws. All statements other than statements of historical fact contained in this release are forward-looking statements. These statements may be identified by words such as "believe," "expect," "anticipate," "intend," "plan," "estimate," "seek," "will," "may," "potential" and similar expressions, or their negatives.

These forward-looking statements include, but are not limited to, statements concerning Rocket's plans to advance and complete the pivotal Phase 2 trial of RP-A501 for Danon disease; the expected timing of patient enrollment, dosing completion and topline results; the potential for the established co-primary endpoints and resulting clinical evidence to support an accelerated approval pathway; the potential safety, efficacy and therapeutic benefits of RP-A501; the implications of Phase 1 and original-dose Phase 2 findings and initial safety observations under the modified protocol for continued development; modeled estimates of the U.S. Danon disease population, the potential treatment-eligible population and related patient-identification efforts; and Rocket's plans to advance its broader cardiovascular pipeline.

Although Rocket believes that the expectations reflected in these forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially as a result of various important factors, including, without limitation: the results, timing and costs of Rocket's ongoing and planned clinical trials; its ability to identify, enroll and dose eligible patients and successfully complete clinical studies; unexpected adverse events or other safety findings; the possibility that preliminary findings, results from small patient populations or observations from earlier studies will not be replicated in additional patients, at longer follow-up or under the modified protocol; the limitations of studies without randomized controls and differences in patient populations, doses, products and follow-up periods; the timing and outcome of regulatory interactions, submissions and review, including whether the FDA will determine that the available evidence supports accelerated approval or approval through another pathway, and any requirements for additional studies or post-approval confirmatory trials; manufacturing, product comparability, quality and supply considerations; uncertainties in modeled patient-population estimates and assumptions, diagnosis rates, patient identification and potential treatment eligibility; Rocket's dependence on third parties for development, manufacturing and other activities; its future capital requirements and ability to obtain additional funding; changes in its operating plan, development priorities, expenses or cash requirements; its ability to obtain, maintain and enforce intellectual property protection; and competing therapies and technological developments. Additional risks are described under "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2025, filed with the Securities and Exchange Commission on February 26, 2026, and in its subsequent SEC filings, including its Quarterly Reports on Form 10-Q.

Accordingly, readers 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, except as required by law.

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