# Rocket Pharmaceuticals Presents Preliminary Data from Phase 1 Clinical Trial of RP-A601 for PKP2 Arrhythmogenic Cardiomyopathy at 28th Annual Meeting of the American Society of Gene and Cell Therapy

May 15, 2025

*RP-A601 was generally well-tolerated at a dose of 8.0E13 GC/kg with no dose-limiting toxicities in all three patients with up to 12 months follow-up*

*RP-A601 promoted increased protein expression and desmosomal localization of Plakophilin-2 (PKP2), Desmocollin-2, and Cadherin-2 in all three patients*

*Improvement or stabilization observed in arrhythmia burden, heart function, and quality of life in all patients*

*No further dose escalation planned*

*Investor webinar to be held later today at 4:30 p.m. ET*

CRANBURY, N.J.--(BUSINESS WIRE)--May 15, 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 preliminary data from the Phase 1 clinical trial of RP-A601 for the treatment of plakophilin-2 related arrhythmogenic cardiomyopathy (PKP2-ACM). RP-A601 showed a well-tolerated safety profile with no dose-limiting toxicities, increased PKP2 protein expression, and preliminary indications of improvement or stabilization in arrhythmia burden, heart function, and quality of life in all three patients followed for up to 12 months. Results were presented today as a late-breaking oral presentation at the Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) and will be discussed on a company webinar today at 4:30 p.m. ET.

“Preliminary data from the Phase 1 study of RP-A601 for PKP2-ACM are highly encouraging, signaling potential clinical benefit along with a generally well-tolerated safety profile,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. “These initial results represent the second gene therapy from our AAV cardiovascular portfolio to show positive clinical data, propelling us one step closer towards our mission of delivering potentially curative treatments to patients with rare and devastating heart conditions.”

The preliminary safety and efficacy of RP-A601 were evaluated in a single-arm, open-label, multi-center Phase 1 study in three patients with PKP2-ACM who received a dose of $8x10^{13}$ GC/kg. Initial data from the Phase 1 study (safety cut-off May 6, 2025; efficacy cut-off April 2025) showed that RP-A601 was generally well-tolerated with no dose-limiting toxicities observed in all patients followed for up to 12 months. Most treatment emergent adverse events were mild/moderate in severity and self-limited with only one patient experiencing severe adverse events which resolved without clinical sequelae within two months post-treatment, believed to be associated with the immunomodulatory regimen.

Cardiac biopsies showed RP-A601 increased PKP2 protein expression in all three patients. In the patients with low baseline PKP2 expression (N=2), improvements in PKP2 protein expression relative to total cell protein were approximately 110% and 398%, respectively, from baseline to six months follow-up. In addition, RP-A601 increased protein expression and promoted desmosomal localization of PKP2, Desmocollin-2, and Cadherin-2 in all three patients.

Preliminary indications of improvement or stabilization were observed in arrhythmia burden, heart function, and quality of life. Patients in the Phase 1 trial also demonstrated:

- Improvements/stabilization in right ventricular (RV) function.
    - All patients showed normal RV systolic function at most recent follow-up.
- Improvements in quality-of-life as assessed through the Kansas City Cardiomyopathy Questionnaire (KCCQ) and New York Heart Association (NYHA) Class.
    - Clinical improvement in KCCQ-12 score of 34-41 points (≥5 point increases considered clinically meaningful in adults) and improved NYHA class (from Class II at baseline to Class I; NYHA Class I reflects the absence of clinical signs of heart failure) in both patients followed beyond six months.
- Decreased/stabilized ventricular ectopy (premature ventricular contractions [PVC], non-sustained ventricular tachycardia

[NSVT]) on rhythm monitoring.
    - Patients experienced a 9% to 63% reduction in PVCs from baseline evaluated six to 12 months post-treatment.
    - The only patient who had baseline NSVTs saw a decrease from five to zero NSVT episodes per 24-hour period at six months post-treatment.
- Decreased/stabilized T-wave inversions on electrocardiogram (ECG).
    - One patient saw a reduction in ECG leads with T-wave inversions (precordial and inferior ECG) from six to two at six months post-treatment.

**Investor Webcast Information**
Company management will host a webinar today, May 15, 2025, at 4:30 p.m. ET. To join the investor webinar, please register at https://www.webcaster4.com/Webcast/Page/3046/52359. The webcast is available under "Events" in the Investors section of the Company's website at: https://ir.rocketpharma.com/. The webcast replay will be available on the Rocket website upon completion of the event.

**About RP-A601**
RP-A601 is an investigational gene therapy for the treatment of plakophilin-2 related arrhythmogenic cardiomyopathy (PKP2-ACM). RP-A601 consists of a recombinant adeno-associated serotype rh74 capsid containing a functional version of the human *PKP2* transgene (AAVrh74.PKP2) which is administered as a single intravenous (IV) infusion. RP-A601 is being investigated as a one-time, potentially curative gene therapy treatment that may improve survival and quality of life for patients affected by PKP2-ACM. Rocket holds Fast Track designation in the U.S. and Orphan Drug designation in the U.S. and Europe for the program.

**About PKP2-Arrhythmogenic Cardiomyopathy (PKP2-ACM)**
PKP2-ACM is an inherited heart disease caused by mutations in the *PKP2* gene and characterized by life-threatening ventricular arrhythmias, cardiac structural abnormalities, and sudden cardiac death. PKP2-ACM affects approximately 50,000 adults and children in the U.S. and Europe. Patients living with PKP2-ACM have an urgent unmet medical need, as current medical, implantable cardioverter defibrillator (ICD), and ablation therapies do not consistently prevent disease progression or arrhythmia recurrence, are associated with significant morbidity including inappropriate shocks and device and procedure-related complications, and do not address the underlying pathophysiology or genetic mutation.

**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 program for Danon Disease, a devastating heart failure condition resulting in thickening of the heart, an early-stage program in clinical trials for PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death, and a pre-clinical program targeting 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 Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure (BMF) and potentially cancer, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, 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 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 funds its operations into the fourth quarter of 2026. 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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