



Rocket Pharmaceuticals Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for RP-A601 Gene Therapy for PKP2-Arrhythmogenic Cardiomyopathy

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CRANBURY, N.J.--(BUSINESS WIRE)--Jul. 17, 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 that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-A601, the Company's investigational adeno-associated virus (AAV)-based gene therapy for the treatment of PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death. RMAT designation was granted based on positive safety and efficacy data from the Phase 1 RP-A601 clinical trial and will provide the benefits of added intensive FDA guidance and expedited review through the program's development.

"The FDA's RMAT designation for RP-A601 represents a meaningful advancement for Rocket and for patients living with PKP2-ACM, a life-threatening genetic heart disease characterized by ventricular arrhythmias and sudden cardiac death," said Kinnari Patel, PharmD, MBA, President, Head of R&D of Rocket Pharmaceuticals. "This marks the fifth RMAT designation in our history and underscores our commitment to developing potentially curative gene therapies for patients with rare and inherited cardiovascular diseases. The early clinical data for RP-A601 are highly encouraging, and we look forward to continued collaboration with the FDA throughout the program's development."

RMAT designation was established under the 21st Century Cures Act to expedite the development and review of promising therapeutic candidates, including gene therapies, that are intended to treat, modify, reverse or cure a serious or life-threatening disease. RMAT designation provides several benefits, such as early interactions with the FDA, including discussions on surrogate or intermediate endpoints that could potentially support accelerated approval and satisfy post-approval requirements, and potential priority review of a product's biologics license application (BLA).

Preliminary results from the ongoing Phase 1 clinical trial of RP-A601 presented at the 2025 Annual Meeting of the American Society of Gene and Cell Therapy demonstrated encouraging early safety and efficacy. All three adult patients treated with a single dose of RP-A601 (8x10¹³ GC/kg) showed increased PKP2 protein expression, including 110% and 398% increases in the two patients with low baseline levels, as well as improved desmosomal integrity with relocalization of key structural proteins. Improvements or stabilization were observed across clinically meaningful endpoints, including right ventricular function, ventricular arrhythmias, and quality of life highlighted by increases of 34–41 points in KCCQ-12 scores and improvements in NYHA classification from Class II to Class I. The safety profile was favorable, with RP-A601 generally well-tolerated, no dose-limiting toxicities, and most adverse events being mild or moderate and self-limited.

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 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 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 2027. 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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