Rocket Pharmaceuticals Announces FDA IND Clearance of RP-A701 for the Treatment of BAG3-associated Dilated Cardiomyopathy

June 30, 2025

RP-A701 is a first-in-class gene therapy for the treatment of BAG3-associated dilated cardiomyopathy

BAG3-associated dilated cardiomyopathy is a rare, inherited heart condition caused by mutations in the BAG3 gene, leading to early-onset, progressive heart failure due to impaired cardiac function, high morbidity, and premature mortality

Rocket plans to conduct a Phase 1 trial in the U.S. and is working towards the first patient treatment

CRANBURY, N.J.--(BUSINESS WIRE)--Jun. 30, 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 it has received clearance from the U.S. Food and Drug Administration (FDA) for the Company's Investigational New Drug (IND) application for RP-A701, an AAVrh.74-based gene therapy candidate for the treatment of BAG3-associated Dilated Cardiomyopathy (BAG3-DCM), a severe form of heart failure characterized by progressive ventricular enlargement and impaired systolic function.

"The FDA clearance of RP-A701, our third clinical-stage gene therapy candidate from our AAV cardiovascular portfolio, is an important milestone for Rocket," said Kinnari Patel, PharmD, MBA, President, Head of R&D and Chief Operating Officer of Rocket Pharma. "With programs in the clinic for each of the major types of genetic cardiomyopathies – hypertrophic, dilated, and arrhythmogenic – we are advancing our mission to bring potentially curative gene therapies to patients with rare and life-threatening cardiovascular diseases. Phase 1 trial start-up activities are currently underway for RP-A701, and we are working towards treating the first patient."

The first-in-human Phase 1 clinical trial will be a multi-center, dose-escalation study designed to evaluate the safety, biological activity, and preliminary efficacy of RP-A701 in adults with BAG3-DCM. Initial study participants will include adults with implantable cardioverter defibrillators (ICDs) and advanced disease at high risk for heart failure progression and cardiac death. Participants will receive a single dose of RP-A701, and the trial will assess BAG3 protein expression, changes in cardiac biomarkers, and clinical predictors of disease progression.

About BAG3-associated Dilated Cardiomyopathy (DCM)
BAG3-DCM is an inherited heart disease caused by mutations in the *BAG3* gene (Bcl2-associated athanogene 3) resulting in early onset, rapidly progressing heart failure, and significant morbidity and mortality. The BAG3 protein contributes to multiple cellular functions, including cardiac contractility, protein quality control (as a co-chaperone), cardiomyocyte structural support, and regulation of autophagy and apoptosis. Loss of BAG3 leads to an accumulation of misfolded and damaged proteins, which can impair the heart's ability to contract, leading to impaired cardiac function, heart failure, and even premature death. We estimate that the prevalence of BAG3-associated DCM in the U.S. is as many as 30,000 individuals. Patients living with BAG3-DCM have an urgent unmet medical need, as current medical and interventional therapies (including implantable cardioverter defibrillator [ICD], cardiac resynchronization devices, and heart transplant) do not consistently prevent disease progression, are associated with significant morbidity including inappropriate ICD shocks and device, procedure, and transplant-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 clinical 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 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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