Rocket Pharmaceuticals Announces Completion of Enrollment in Phase 2 Pivotal Trial of RP-A501 for the Treatment of Danon Disease

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CRANBURY, N.J.--(BUSINESS WIRE)--Sep. 17, 2024-- 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 all patients have been enrolled in the global, pivotal Phase 2 clinical trial evaluating RP-A501 to treat male patients with Danon disease.

After the two-patient safety run-in, followed by harmonized global site activations, the remaining 10 patients were enrolled across the United States (U.S.) and European Union within three months. Given the prevalence of Danon disease across regions, the Company plans to pursue regulatory filings concomitantly in the U.S. and ex-U.S.

“From a clinical perspective, the important thing is that we are moving closer to the goal of having a treatment for patients with Danon disease,” said Barry H. Greenberg, MD, FHFSA, Director of the Advanced Heart Failure Treatment Program and Distinguished Professor of Medicine at UC San Diego Health. “I can attest to the excitement and anticipation within the Danon patient community for this novel, one-time treatment designed to improve cardiac abnormalities associated with Danon disease and help preserve normal cardiac function by delivering functional *LAMP2B* genes to the heart tissue. The rapid recruitment of the Phase 2 trial signifies the positive views of the study clinicians regarding this investigational therapy.”

RP-A501 Phase 2 Pivotal Trial Overview

The global, single-arm, multi-center Phase 2 pivotal trial evaluates the efficacy and safety of RP-A501 in 12 patients with Danon disease, including a pediatric safety run-in (n=2), and a dose level of 6.7×10^{13} GC/kg.

- To support accelerated approval, the study assesses the efficacy of RP-A501 as measured by the biomarker-based co-primary endpoint consisting of improvements in LAMP2 protein expression, and reductions in left ventricular mass.
- Key secondary endpoint is change in troponin. Additional secondary endpoints include natriuretic peptides, Kansas City Cardiomyopathy Questionnaire, New York Heart Association class, event free survival to 24 months and treatment emergent safety events. These endpoints could support full approval with longer-term follow-up.
- A global natural history study is running concurrently with the Phase 2 pivotal trial.
- The pediatric run-in enrolled two patients in a sequential manner with a minimum three-month follow-up prior to subsequent enrollment. In addition, all patients enrolled in the trial are required to have a three-months observational pre-treatment run-in to enable an assessment of troponin (and other biomarker) trajectories to optimally assess this key secondary endpoint.

Details about the Phase 2 study can be found at www.clinicaltrials.gov under NCT identifier NCT06092034.

About RP-A501

RP-A501 is Rocket’s investigational gene therapy product for the treatment of Danon disease and the first gene therapy for a cardiovascular condition to demonstrate safety and efficacy in clinical studies. Danon disease is caused by mutations in the *LAMP2* gene.

RP-A501 consists of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a full-length, wild-type version of the human LAMP2B transgene (AAV9.LAMP2B) which, when inserted into heart cells harboring mutations in the endogenous *LAMP2* gene, has the potential to fully restore cardiac function at its root. RP-A501 represents a single dose treatment and is administered as an intravenous infusion. In preclinical and clinical studies, AAV9.LAMP2B has been shown to target cardiac cells (cardiomyocytes) and deliver the functional LAMP2B gene to heart tissue, which ultimately leads to improved cardiac structure and function in patients.

In 2023, the U.S. Food and Drug Administration granted regenerative medicine advanced therapy designation, and the European Medical Agency granted PRIority MEdicines (PRIME) eligibility to RP-A501.

About Danon Disease

Danon disease is a rare X-linked inherited disorder caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. This 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. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and Europe.

The only available 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.

In 2023, Rocket secured an ICD-10 code from the Centers for Medicare and Medicaid Services (CMS) to document patients with *LAMP2* deficiency in Danon disease.

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 lentiviral 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 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.

Rocket's adeno-associated viral 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.

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 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, 2023, filed February 27, 2024 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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