Rocket Pharmaceuticals to Present Updated Data from the Phase 1 Clinical Trial of RP-A501 for Danon Disease at the American Heart Association's 2024 Late-Breaking Science Sessions

November 12, 2024

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 12, 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 the company will be presenting new data on the results of the RP-A501 Phase 1 trial to treat patients with Danon disease at the American Heart Association's 2024 Late-Breaking Science sessions being held from November 16-18, 2024 in Chicago, Illinois.

Details for the presentation are as follows:

> **Title**: Danon Disease Phase 1 RP-A501 Results: The First Single-Dose Intravenous Gene Therapy with Recombinant Adeno-Associated Virus (AAV9:LAMP2B) for a Monogenic Cardiomyopathy
> **Session:** Amyloid, Hypertrophic and Danon Cardiomyopathies: Targeted Therapies and Specific Populations
> **Presenter**: Joseph Rossano, M.D., M.S., FAAP, FACC, Co-Director of the Cardiac Center and Chief of the Division of Cardiology at Children's Hospital of Philadelphia
> **Date and Time**: Monday, November 18, 2024, from 9:45-9:57 a.m. CT

Rocket plans to host an investor webinar on November 18, 2024, at 12:00 p.m. ET. To join the investor webinar, please register at https://www.webcaster4.com/Webcast/Page/3046/51498. Webinar details will be posted on the Events and Presentations page of the Rocket website at www.RocketPharma.com.

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 virus serotype 9 (AAV9) capsid containing a full-length, wild-type version of the human *LAMP2B* transgene (AAV9.LAMP2B) which, when inserted into cardiac cells (cardiomyocytes) harboring mutations in the endogenous *LAMP2* gene, has the potential to substantially restore cardiac function by addressing the root cause of Danon disease. RP-A501 is a single dose treatment administered as an intravenous infusion. In preclinical and clinical studies, AAV9.LAMP2B has been generally well tolerated and shown to target cardiomyocytes and deliver the functional *LAMP2B* gene to heart tissue, which ultimately leads to improvement in cardiac structure and overall clinical function in patients.

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.

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 (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.

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.

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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