Rocket Pharmaceuticals Receives Orphan Medicinal Product Designation from the European Commission for RP-A601 for PKP2 Arrhythmogenic Cardiomyopathy

May 29, 2024

CRANBURY, N.J.--(BUSINESS WIRE)--May 29, 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 European Commission (EC), based on a positive opinion issued by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), has granted orphan medicinal product designation for RP-A601, the Company's adeno-associated virus (AAV.rh74)-based gene therapy candidate for the treatment of plakophilin-2 related arrhythmogenic cardiomyopathy (PKP2-ACM).

PKP2-ACM is a devastating, inherited heart disease associated with life-threatening arrhythmias, cardiac structural abnormalities, and sudden cardiac death. Presently, there are no curative treatment options available for PKP2-ACM. The current standard of care consists of medical therapy, implantable cardioverter defibrillators (ICDs), and ablation procedures. Even with treatment, life-threatening arrhythmias and progression of disease can still occur. PKP2-ACM affects approximately 50,000 people in the U.S. and Europe.

Orphan medicinal product designation by the EC is available to novel therapeutics that prevent or treat life-threatening or chronically debilitating conditions that affect fewer than five in 10,000 persons in the European Union (EU). The designation qualifies for financial and regulatory benefits including protocol assistance from the EMA during clinical development, access to centralized marketing authorization, and a 10-year period of marketing exclusivity after product approval.

The Company is enrolling patients in a Phase 1, dose escalation trial evaluating the safety and preliminary efficacy of RP-A601 in at least six adult PKP2-ACM patients with ICDs and who have an overall high risk for life-threatening arrhythmias. The study is assessing the impact of RP-A601 on PKP2 myocardial protein expression, cardiac biomarkers, clinical predictors of life-threatening ventricular arrhythmias, and sudden cardiac death.

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 (AAVrh74) 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 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 "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, Rocket's plans for the advancement of its DD program, including its planned pivotal trial, and the safety, effectiveness and timing of related 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 and Rocket's ability to transition to a commercial stage pharmaceutical company. 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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