Rocket Pharmaceuticals Receives FDA Fast Track and Orphan Drug Designations for RP-A601 Gene Therapy for PKP2 Arrhythmogenic Cardiomyopathy (ACM)

June 8, 2023

CRANBURY, N.J.--(BUSINESS WIRE)--Jun. 8, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and 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 Fast Track and Orphan Drug designations to RP-A601, the Company's gene therapy candidate for the treatment of plakophilin-2 related arrhythmogenic cardiomyopathy (PKP2-ACM).

RP-A601 is Rocket's adeno-associated virus (AAV.rh74)-based gene therapy for PKP2-ACM, a devastating, inherited heart disease associated with life-threatening arrhythmias, cardiac structural abnormalities, and sudden cardiac death. The current standard of care consists of medical therapy, implantable cardioverter defibrillators (ICDs), and ablation procedures, none of which are curative. 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.

Fast Track designation is granted to facilitate the development and expedite the review of medicines that treat serious conditions and fill an unmet medical need. The designation enables increased communication with the FDA throughout the development of RP-A601, offers the potential for accelerated approval and priority review if criteria are met, and permits a Rolling BLA Review. Orphan Drug designation is granted to support the development of medicines for rare disorders that affect fewer than 200,000 patients in the U.S.

Rocket recently announced FDA clearance of the Investigational New Drug (IND) application for RP-A601 based on robust preclinical proof of concept studies that demonstrated decreased arrhythmias and increased survival. RP-A601 is the first gene therapy in development for PKP2-ACM to receive IND clearance and the second of three programs in the Company's AAV-based cardiovascular gene therapy franchise.

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

About FDA Fast Track Designation

Fast Track designation is designed to facilitate the development and expedite the review of drug candidates that are intended to treat serious conditions and for which nonclinical data has demonstrated the potential to address unmet medical need. The program enables increased communication with the FDA for the purpose of expediting the product's development, review, and potential approval. Therapies may qualify for accelerated approval and priority review if criteria are met, in addition to Rolling Review, which affords the opportunity to submit completed sections of the Biologic License Application (BLA) for review by the FDA, rather than waiting until every section is completed before the entire application can be reviewed.

About FDA Orphan Drug Designation

Orphan Drug designation is granted by the FDA for the treatment, prevention or diagnosis of diseases that are life-threatening or chronically debilitating with a prevalence of fewer than 200,000 patients annually. The designation provides certain benefits, including market exclusivity upon regulatory approval, exemption of FDA application fees, and tax credits for qualified clinical trials.

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. 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 this devastating disease.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare childhood disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (LVV)-based gene therapy are for the treatment of 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 rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. Rocket also has received IND clearance for the AAV-based gene therapy program for PKP2-arrhythmogenic cardiomyopathy (ACM) and is advancing a preclinical program for BAG3-associated dilated cardiomyopathy (DCM). For more information about Rocket, please visit www.rocketpharma.com.

Rocket Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, 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), PKP2-ACM 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 Danon Disease program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "aim," "anticipate," "believe," "can," "continue," "design," "estimate," "expect," "intend," "may," "plan," "potential," "will give," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. 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 ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any unexpected expenditures, 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, 2022, filed February 28, 2023 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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Source: Rocket Pharmaceuticals, Inc.