Rocket Pharmaceuticals Provides Update on Anticipated Registration Path for RP-A501 in Danon Disease Following End-of-Phase 1 FDA Meeting

December 22, 2022

Based on ongoing FDA discussion, Rocket to pursue a biomarker-based composite endpoint for a single arm, open-label pivotal trial with a natural history comparator

Initiation of initial component of global study anticipated in 1H'23

CRANBURY, N.J.--(BUSINESS WIRE)--Dec. 22, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today announces updates from the Company's end-of-Phase 1 meeting with the U.S. Food and Drug Administration (FDA) regarding RP-A501, the Company's investigational adeno-associated virus (AAV)-based gene therapy for Danon Disease. During the meeting, Rocket reviewed the positive Phase 1 dataset with the FDA and proposed a study design and endpoints for ongoing clinical development of the investigational gene therapy.

"I am pleased to announce that following discussions with FDA, we anticipate proceeding with a dose of 6.7e13 GC/kg, and we anticipate utilizing a single arm open-label trial design with a robust natural history comparator, pursuant to the FDA's acknowledgment of the challenges associated with executing a randomized controlled trial in Danon Disease," said Gaurav Shah, Chief Executive Officer, Rocket Pharma. "The FDA has also expressed an openness to considering a biomarker-based composite endpoint supported by functional and quality-of-life assessments as measures of patient benefit. We look forward to continued dialogue with the FDA on the design for our proposed pivotal trial, including discussion of appropriate external controls for the study and appropriate endpoints to support accelerated approval."

Dr. Shah continued, "We are now in discussion with the FDA about a trial design that will enable evaluation of two pediatric patients treated with drug product manufactured at our in-house cGMP AAV facility as an initial component of a modestly-sized global pivotal study. We are very encouraged by the highly collaborative ongoing dialogue with the FDA for RP-A501 in Danon Disease and subject to the continued dialogue and agreement with the FDA anticipate initiating the initial component of the global study in the first half of 2023."

Results from the ongoing Phase 1 Danon Disease trial represent one of the most comprehensive investigational gene therapy datasets for any cardiac condition. RP-A501 was generally well tolerated with evidence of durable treatment activity and improvement of Danon Disease for both pediatric patients with up to nine months of follow-up and four adult patients with up to 36 months of follow-up. All adult and pediatric patients who received a closely monitored immunomodulatory regimen showed improvements across tissue, laboratory, and imaging-based biomarkers, as well as in New York Health Association (NYHA) class (from II to I) and Kansas City Cardiomyopathy Questionnaire (KCCQ) scores with follow-up of six to 36 months.

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. There are no specific therapies available for the treatment of Danon 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 a preclinical AAV-based gene therapy program in BAG3-associated dilated cardiomyopathy. 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 its guidance for 2022 and 2023 in light of COVID-19, 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), and Danon Disease, 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 "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. 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, our expectations regarding the delays and impact of COVID-19 on clinical sites, 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, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and 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, 2021, filed February 28, 2022 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.