Rocket Pharmaceuticals Announces Presentations Highlighting Lentiviral and AAV Gene Therapies at the 30th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT)

October 19, 2023

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 19, 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 presentations at the 30th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT) in Brussels, Belgium, taking place October 24-27. Updated data will be presented from the Phase 1 clinical trial of RP-L301 for Pyruvate Kinase Deficiency (PKD). Previously disclosed data will be presented from the Phase 2 pivotal trial of RP-L102 for Fanconi Anemia, Phase 1 trial of RP-A501 for Danon Disease and preclinical studies supporting the Phase 1 trial of RP-A601 for PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM). Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer, Rocket Pharma, will also give an Invited Talk about the importance of collaboration with and commitment to patients in gene therapy development.

Details for the Invited Talks and oral presentations are as follows:

Title: Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency
Session: Lentiviral & Integrative Vectors
Presenter: Julián Sevilla M.D., Ph.D., Fundación para la Investigación Biomédica, Hospital Infantil Universitario Niño Jesús
Session date and time: Tuesday, October 24, 5:00 p.m. – 7:00 p.m. CEST
Presentation number: OR05

Title: Embracing Collaboration and Commitment to Patients to Drive Gene Therapy Forward
Session: Accessibility of Gene Therapy
Presenter: Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer, Rocket Pharma
Session date and time: Wednesday, October 25, 2:30 p.m. – 5:00 p.m. CEST
Presentation number: INV31

Title: Molecular, Cellular and Clinical Implications of Lentiviral-Mediated Gene Therapy in Patients with Fanconi Anemia
Session: Hematopoietic Diseases Gene Therapy
Presenter: Paula Río, Ph.D., CIEMAT/CIBERER/IIS. F. Jiménez Díaz
Session date and time: Thursday, October 26, 9:00 a.m. – 10:30 a.m. CEST
Presentation number: OR41

Title: Gene Therapy for Patients with Danon Disease: First in Man Experience
Session: Cardiovascular & Muscular Diseases
Presenter: Jonathan Schwartz, M.D., Chief Gene Therapy Officer, Rocket Pharma
Session date and time: Friday, October 27, 11:00 a.m. – 1:00 p.m. CEST
Presentation number: INV81

Details for the poster presentation are as follows:

Title: Preclinical Efficacy & Safety of AAVrh.74-PKP2a (RP-A601): Gene Therapy for PKP2-associated Arrhythmogenic Cardiomyopathy
Session: AAV & Non Integrative Vectors
Presenter: Bitha Narayanan, Ph.D., Senior Director, Nonclinical R&D, Rocket Pharma
Session dates and times: Wednesday, October 25, 5:00 p.m. – 6:15 p.m. CEST, and Thursday, October 26, 8:30 p.m. – 9:30 p.m. CEST
Poster number: P067

Additional information can be found online at: https://www.esgctcongress.com/.

About Pyruvate Kinase Deficiency

Pyruvate Kinase Deficiency (PKD) is a rare, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red blood cell destruction and the disorder ranges from mild to life-threatening anemia. PKD has an estimated prevalence of 4,000 to 8,000 patients in the U.S. and Europe.

Children are the most commonly and severely affected subgroup of patients. Patients with PKD have a high unmet medical need, as currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload. Recently, mitapivat, an oral enzyme activator, was approved for use in adult patients, however its efficacy is limited in more severely-afflicted patients, most notably in those who are splenectomized, transfusion-dependent, or whose disease results from deleterious mutations.

RP-L301 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz (IIS-FJD).

About Fanconi Anemia

Fanconi Anemia (FA) is a rare pediatric disease characterized by bone marrow failure, malformations, and cancer predisposition. The primary cause of death among patients with FA is bone marrow failure, which typically occurs during the first decade of life. Allogeneic hematopoietic stem cell transplantation (HSCT), when available, corrects the hematologic component of FA, but requires myeloablative conditioning. Graft-versus-host disease, a known complication of allogeneic HSCT, is associated with an increased risk of solid tumors, mainly squamous cell carcinomas of the head and neck region. Approximately 60-70% of patients with FA have a Fanconi Anemia complementation group A (*FANCA*) gene mutation, which encodes for a protein essential for DNA repair. Mutations in the *FANCA* gene lead to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Increased sensitivity to DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is a ‘gold standard’ test for FA diagnosis. Somatic mosaicism occurs when there is a spontaneous correction of the mutated gene that can lead to stabilization or correction of a FA patient’s blood counts in the absence of any administered therapy. Somatic mosaicism, often referred to as ‘natural gene therapy’ provides a strong rationale for the development of FA gene therapy because of the selective growth advantage of gene-corrected hematopoietic stem cells over FA cells. There is a high unmet medical need for patients with FA.

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 is a high unmet medical need for patients with Danon Disease.

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 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 (LV)-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 is developing AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and 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) 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 "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, 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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