Rocket Pharmaceuticals Presents Positive Clinical Data from Fanconi Anemia, Pyruvate Kinase Deficiency and Severe Leukocyte Adhesion Deficiency-I Programs at the 64th American Society of Hematology (ASH) Annual Meeting

December 12, 2022

Sustained genetic and phenotypic correction and concomitant hematologic stabilization observed in at least six of 10 evaluable FA patients between 12 and 36 months after RP-L102; a seventh patient potentially demonstrates engraftment and stabilization after 36 months

Robust and sustained efficacy in both adult PKD patients at 24 months post-RP-L301 demonstrated by normalized hemoglobin and improved hemolysis parameters

CRANBURY, N.J.--(BUSINESS WIRE)--Dec. 12, 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 positive clinical data from its lentiviral (LV)-based gene therapy programs at the 64th American Society of Hematology (ASH) Annual Meeting, taking place in New Orleans, Louisiana, from December 10-13.

Lentiviral-mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Updated Results from Global RP-L102 Clinical Trials

The poster presentation includes positive updated data (cut-off October 26, 2022) from the ongoing Phase 2 pivotal trial of RP-L102, Rocket's *ex vivo* lentiviral gene therapy candidate for Fanconi Anemia (FA).

- RP-L102 conferred phenotypic correction in at least six of 10 evaluable patients with ≥12 months of follow-up as demonstrated by increased resistance to mitomycin-C (MMC) in bone marrow (BM)-derived colony forming cells, concomitant genetic correction and hematologic stabilization.
    - A seventh patient has displayed evidence of progressively increasing genetic correction as demonstrated by peripheral blood and BM vector copy numbers (VCNs), with recent development of MMC resistance and indicators of hematologic stability after 36 months of follow-up.
    - The primary endpoint has been achieved, based on a trial protocol in which statistical and clinical significance requires a minimum of five patients to attain increased MMC resistance at least 10% above baseline at two or more timepoints and concomitant evidence of genetic correction and clinical stabilization.
- The safety profile of RP-L102 has been highly favorable, and the treatment, administered without any cytotoxic conditioning, has been well tolerated. No signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102 have been observed.
    - As previously disclosed, one patient experienced a Grade 2 transient infusion-related reaction, which resolved; one patient with confirmed engraftment developed a T-cell lymphoblastic lymphoma that was conclusively determined by the investigator, sponsor and the independent data monitoring committee to be related to FA (a cancer predisposition syndrome) and unrelated to RP-L102 gene therapy.
- Based on the positive efficacy and safety data from the Phase 2 pivotal FA trial, Rocket anticipates regulatory filing in the second half of 2023.

Lentiviral-mediated Gene Therapy for Adults and Children with Severe Pyruvate Kinase Deficiency: Results from an Ongoing Global Phase 1 Study

The poster presentation includes positive updated data (cut-off October 26, 2022) from two adult patients with significant anemia who were treated with RP-L301, Rocket's *ex vivo* lentiviral gene therapy candidate for Pyruvate Kinase Deficiency (PKD).

- At 24 months post-infusion, both patients have robust and sustained efficacy demonstrated by normalized hemoglobin

(from baseline levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, independence from red blood cell transfusions and improved quality of life both reported anecdotally and as documented via formal quality of life assessments.
- The safety profile appears highly favorable, with no RP-L301-related serious adverse events through 24 months post-infusion in both adult patients.
- Insertion site analyses in peripheral blood and bone marrow in both adult patients up to 12 months post-RP-L301 demonstrated highly polyclonal patterns and there has been no evidence of insertional mutagenesis.
- Adult and pediatric enrollment is completed in the Phase 1 study. Phase 2 pivotal trial initiation is anticipated in 2023.

Interim Results from an Ongoing Phase 1/2 Study of Lentiviral-mediated *Ex-vivo* Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I

The poster presentation includes previously disclosed top-line data at three to 24 months of follow-up after RP-L201 infusion for all patients and overall survival data for seven patients at 12 months or longer after infusion. RP-L201 is Rocket's *ex vivo* lentiviral gene therapy candidate for severe Leukocyte Adhesion Deficiency-I (LAD-I).

- Observed 100% overall survival at 12 months post-infusion via Kaplan Meier estimate and a statistically significant reduction in all hospitalizations, infection- and inflammatory-related hospitalizations and prolonged hospitalizations for all nine LAD-I patients with three to 24 months of available follow-up. Data also shows evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities.
- The safety profile of RP-L201 has been highly favorable in all patients with no RP-L201-related serious adverse events to date. Adverse events related to other study procedures, including busulfan conditioning, have been previously disclosed and consistent with the safety profiles of those agents and procedures.
- Based on the positive efficacy and safety data from the Phase 2 pivotal LAD-I trial, Rocket has initiated discussions with the FDA and anticipates regulatory filing in the first half of 2023.

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 leads 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.

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 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 United States and the European Union. Children are the most commonly and severely affected subgroup of patients. 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 Leukocyte Adhesion Deficiency-I

Severe Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by mutations in the *ITGB2* gene encoding for the beta-2 integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. As a result, children with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Children who survive infancy experience recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia. Without a successful bone marrow transplant, mortality in patients with severe LAD-I is 60-75% prior to the age of 2 and survival beyond the age of 5 is uncommon. There is a high unmet medical need for patients with severe LAD-I.

Rocket's LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (Grant Number CLIN2-11480). The contents of this press release are solely the responsibility of Rocket and do not necessarily represent the official views of CIRM or any other agency of the State of California.

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 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 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.