Rocket Pharmaceuticals Presents Positive Top-line Data from Severe Leukocyte Adhesion Deficiency-I Program at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT)

May 19, 2022

—RP-L201 well-tolerated with durable CD18 expression for all patients in Phase 2 pivotal trial —

—100% overall survival in patients at one year after RP-L201 infusion based on Kaplan-Meier estimate —

—All patients showed clinical reversal of disease course, including statistically significant reduction in all-cause hospitalizations and incidence of severe infections —

—Initiating discussions with health authorities on filing plans; regulatory filings anticipated in the first half of 2023 —

CRANBURY, N.J.--(BUSINESS WIRE)--May 19, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage, clinical biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today announces positive top-line safety and efficacy data from its Phase 2 pivotal trial for severe Leukocyte Adhesion Deficiency-I (LAD-I) at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT).

"We are excited to present positive top-line safety and efficacy data from our Phase 2 pivotal trial for LAD-I today at ASGCT, representing a significant step forward in the development of RP-L201 for the treatment of LAD-I, one of the most aggressive and highly fatal immunodeficiencies ever characterized. Moreover, they point to the great possibilities lentiviral-based gene therapies can offer for patients with devastating diseases," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "At three to 24 months after RP-L201 infusion, all nine patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events."

Dr. Shah continued, "We are also pleased to report 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."

"While allogeneic transplant options are available, they continue to be associated with considerable toxicity and today's top-line safety and efficacy data point to the potential of RP-L201 to change the treatment paradigm for patients living with severe LAD-I," said Dr. Shah. "These results are a testament to the LAD-I patient community and focus of the Rocket team on steady, reliable execution. Based on these results, we are initiating discussions with health authorities on filing plans and anticipate filings in the first half of 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 (LAD-I)

The oral presentation includes efficacy and safety data (cut-off March 9, 2022) 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 LAD-I.

- All patients, aged five months to nine years, demonstrated sustained CD18 restoration and expression on more than 10% of neutrophils (range: 20%-87%, median: 56%).
- At one year, the overall survival without allogeneic hematopoietic stem cell transplantation across the cohort is 100% based on the Kaplan-Meier estimate.
- All patients demonstrated a statistically significant reduction in the rate of all-cause hospitalizations and severe infections, relative to pre-treatment.
- Evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities has been shown along with sustained phenotypic correction.

- 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.

About RP-L201

RP-L201 is an investigational gene therapy product being developed for severe Leukocyte Adhesion Deficiency-I (LAD-I). The therapy consists of hematopoietic stem cells from the patient that have been genetically modified with a lentiviral vector to contain a functional copy of the *ITGB2* gene. RP-L201 is currently being evaluated in a Phase 1/2 clinical trial. The interim analysis of the trial at three to 24 months of follow-up showed RP-L201 had a favorable safety profile and evidence of efficacy with durable CD18 expression.

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 genetic therapies that 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 that 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. 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, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, and Rocket's plans for the advancement of its LAD-I program based on the data presented at ASGCT and the potential for therapeutic benefit related thereto, 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. 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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