Rocket Pharmaceuticals Reports Positive Long-Term Clinical Data from RP-L201 Trial for the Treatment of Leukocyte Adhesion Deficiency-I at the Clinical Immunology Society 2021 Annual Meeting

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— New Long-Term Follow-Up Data Demonstrate Durable CD18 Expression, Improved Skin Lesions, and Consistent Peripheral Vector Copy Numbers Up to 18-Months Post-Treatment —

— Second Patient Nearing One-Year Primary Endpoint for Phase 2 Portion of Study —

— Two Additional Patients with Shorter Follow-Up Demonstrate Evidence of Engraftment —

— RP-L201 was Well Tolerated in All Patients —

— More Comprehensive Phase 2 Results Expected in Second Half of 2021 —

CRANBURY, N.J.--(BUSINESS WIRE)--Apr. 14, 2021-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces positive interim data from the Company's Phase 1/2 clinical trial studying RP-L201, its lentiviral-based gene therapy for the treatment of severe Leukocyte Adhesion Deficiency-I (LAD-I). Severe LAD-I is a rare pediatric disease that prevents patients from adequately combating infections. LAD-I leads to recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics, require frequent hospitalizations and are ultimately fatal. These results were presented in a virtual poster session at the Clinical Immunology Society (CIS) 2021 Annual Meeting.

"Today's positive updates on our LAD-I program add to the growing body of encouraging evidence that RP-L201 may provide durable clinical benefit for patients with severe LAD-I who face recurrent, life-threatening infections from birth," said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. "We are very pleased to report that a second patient is nearing survival at one-year post-treatment, the primary outcome measure for the Phase 2 portion of the study. In all patients treated, CD18 expression has substantially exceeded the 4-10% threshold associated with survival into adulthood, with consistent peripheral blood vector copy number levels. Improved disease-related skin lesions, absence of new infections post-treatment, and no further requirements for prophylactic anti-infectives were also observed in both Phase 1 patients with prolonged follow-up. Initial evidence of engraftment and phenotypic correction was observed in two additional patients with shorter follow-up. These updates move us one step closer towards BLA/MAA filings in the US and Europe and eventual commercialization of a potentially curative option for the children facing this truly devastating disease. We look forward to providing more comprehensive Phase 2 results in the second half of 2021."

The data reported in the poster presentation are from four pediatric patients with severe LAD-I, as defined by CD18 expression of less than 2%. The patients were treated with RP-L201, Rocket's *ex-vivo* lentiviral gene therapy candidate. Data were reported as of the cutoff date of February 2021. Patient 1001 was 9 years-of-age at enrollment and had been followed for 18-months after RP-L201 therapy. Patient 1004 was 3 years-of-age at enrollment and had been followed for 9-months. Patients 2006 and 2005 were 7 months- and 2 years-of-age at enrollment and had been followed for 3-months. Key highlights from the poster presentation include:

- RP-L201 was well tolerated, no safety issues reported with infusion or treatment
- All patients achieved hematopoietic reconstitution within 5-weeks
- Neutrophil CD18-expression and peripheral blood vector copy numbers (VCN) were assessed post-treatment to evaluate engraftment and phenotypic correction:
    - 18-months post-treatment, Patient 1001 demonstrated durable CD18 expression of ~40% and resolution of skin lesions with no new lesions reported; 12-months post-treatment, peripheral blood VCN levels were 1.2
    - 9-months post-treatment, Patient 1004 demonstrated CD18 expression of ~28%; 6-months post-treatment, peripheral blood VCN levels were 0.75 with kinetics consistent with those of the first patient
    - 3-months post-treatment, Patient 2006 demonstrated CD18 expression of ~70%; 1.5-months post-treatment, peripheral blood VCN kinetics were consistent with those of the first two patients
    - 3-months post-treatment, Patient 2005 demonstrated CD18 expression of ~51%; 1.5-months post-treatment,

peripheral blood VCN kinetics were consistent with those of the first two patients

To access the poster, please visit: www.rocketpharma.com/CIS

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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.

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 which are frequently fatal, Pyruvate Kinase Deficiency (PKD), a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia, and Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 2021 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, 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, 2020, filed March 1, 2021 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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