Rocket Pharmaceuticals Presents Positive LAD-I Clinical Update and Comprehensive IMO Preclinical Review at the European Society for Immunodeficiencies 2020 Meeting

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—Longer-Term Data from the Registration-Enabling Phase 1/2 Trial for Leukocyte Adhesion Deficiency-I Demonstrate Safety and Efficacy of RP-L201—

—Preclinical Data From RP-L401 for Infantile Malignant Osteopetrosis Support Accelerated Clinical Development—

—Results Provide Further Validation for Rocket's "Process B" Lentiviral Platform—

NEW YORK--(BUSINESS WIRE)--Oct. 16, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces clinical data at the European Society for Immunodeficiencies (ESID) 2020 Meeting being held virtually October 14-17, 2020. An oral presentation provides positive longer-term follow-up data from the Phase 1/2 clinical trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I). An e-poster highlights preclinical study data on RP-L401 for Infantile Malignant Osteopetrosis (IMO) supporting clinical development of the trial.

"Today, Rocket presents positive results from our LAD-I gene therapy trial demonstrating further clinical benefit in this severely affected patient population," said Jonathan Schwartz, M.D. Chief Medical Officer and Senior Vice President of Rocket. "Patients with LAD-I have markedly diminished expression of the integrin CD18 and suffer from life-threatening bacterial and fungal infections. Natural history studies indicate that an increase in CD18 expression to 4-10% is associated with survival into adulthood. The two patients enrolled in our Phase 1 trial demonstrated restored CD18 expression substantially exceeding this threshold. In addition, we continue to observe a durable treatment effect in the patient followed through one year, with improvement of multiple disease-related skin lesions after therapy and no further requirements for prophylactic anti-infectives."

Dr. Schwartz continued, "In addition, preclinical results in Infantile Malignant Osteopetrosis represent an early positive signal of *in vivo* efficacy that support evaluation of RP-L401 in a Phase 1 trial. IMO is a devastating bone resorption disorder resulting in skeletal deformities, neurologic abnormalities and bone marrow failure. Rocket has developed RP-L401 as a potential treatment option to prevent the devastating morbidity and childhood mortality associated with IMO. Preclinical data indicate that even a modest level of engraftment can correct the disease phenotype, with increased long-term survival, growth, and normalized bone and tooth development."

Preliminary Data Highlights from Rocket's Phase 1/2 Study of RP-L201 in LAD-I

The data presented in the oral presentation are from two pediatric patients with severe LAD-I, as defined by CD18 expression of less than 2%. Both patients were treated with RP-L201, Rocket's *ex vivo* lentiviral gene therapy candidate. Patient L201-003-1001 was 9-years of age at treatment and has been followed for 12-months and Patient L201-003-1004 was 3-years of age at treatment and has been followed for four months. Key highlights from the presentation include:

- RP-L201 was well tolerated, and no safety issues were reported with infusion or post-treatment
- Both subjects achieved hematopoietic reconstitution in less than 4 weeks
- Peripheral blood vector copy number (VCN) and neutrophil CD18-expression were assessed post-treatment to evaluate engraftment and phenotypic correction:
    - Patient L201-003-1001 demonstrated durable CD18 expression of 40%, peripheral blood VCN levels of 1.0, visible signs of improvement in existing skin lesions and no new infections, as reported 12 months post-treatment
    - Patient L201-003-1004 demonstrated CD18 expression of 28% and early peripheral blood VCN trending similarly to first patient
- As previously reported, the drug product VCN for patient L201-003-1001 was 3.8 with a CD34+ cell dose of 4.2 x 10^6 cells/kilogram (kg)
- Patient L201-003-1004's CD34+ cell dose was 2.8 x 10^6 cells/kg. The drug product VCN was 2.5.

A copy of the oral presentation and e-poster can be accessed by visiting: https://www.rocketpharma.com/ESID/

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 Infantile Malignant Osteopetrosis

Infantile Malignant Osteopetrosis (IMO) is a rare, severe autosomal recessive disorder caused by mutations in the *TCIRG1* gene, which is critical for the process of bone resorption. Mutations in *TCIRG1* interfere with the function of osteoclasts, cells which are essential for normal bone remodeling and growth, leading to skeletal malformations, including fractures and cranial deformities which cause neurologic abnormalities including vision and hearing loss. Patients often have endocrine abnormalities and progressive, frequently fatal bone marrow failure. As a result, death is common within the first decade of life. IMO has an estimated incidence of 1 in 200,000. The only treatment option currently available for IMO is an allogenic bone marrow transplant (HSCT), which allows for the restoration of bone resorption by donor-derived osteoclasts which originate from hematopoietic cells. Long-term survival rates are lower in IMO than those associated with HSCT for many other non-malignant hematologic disorders; severe HSCT-related complications are frequent. There is an urgent need for additional treatment options.

RP-L401 was in-licensed from Lund University and Medizinische Hochschule Hannover.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") 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 2020 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 when clinical trial sites will resume normal business operations, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, filed August 5, 2020 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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