Rocket Pharmaceuticals Announces Two Presentations at the European Society for Immunodeficiencies 2020 Meeting

October 1, 2020

--Oral Presentation to Provide Update on Phase 1/2 Clinical Trial Data of RP-L201 for Leukocyte Adhesion Deficiency-I--

--Poster Presentation to Highlight Preclinical Data on RP-L401 for Infantile Malignant Osteopetrosis--

NEW YORK--(BUSINESS WIRE)--Oct. 1, 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 two presentations at the European Society for Immunodeficiencies (ESID) 2020 Meeting to be held virtually October 14-17, 2020. An oral presentation will provide an update on data from the Phase 1/2 clinical trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I). An e-poster will highlight preclinical study data on RP-L401 for Infantile Malignant Osteopetrosis (IMO).

Additional presentation details can be found below:

**Oral Presentation**

**Title:** A Phase 1/2 Study of Lentiviral-Mediated Ex Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Results from Phase 1

**Session Title:** Treatment

**Presenter:** Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at the University of California, Los Angeles

**Session Date:** Friday, October 16, 2020

**Session Time:** 10:45 a.m. – 12:01 p.m. CEST

**Lecture Time:** 11:45 a.m. CEST

**Location:** Hall D

This session will be followed by a Q&A from 12:01 p.m. to 12:30 p.m. CEST

**E-Poster**

**Title:** Preclinical Efficacy and Safety of EFS-HTC1RG1-LV Supports IMO Gene Therapy Clinical Trial Initiation

**Presenter:** Ilana Moscatelli, Ph.D., Associate Researcher, Division of Molecular Medicine and Gene Therapy, Lund University, Sweden

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 (less than 2% normal expression) 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 TC1RG1 gene, which is critical for the process of bone resorption. Mutations in TC1RG1 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](http://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 our 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-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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