



Rocket Pharmaceuticals Presents Positive Updates on FA and LAD-I Gene Therapy Programs at the 23rd Annual Meeting of the American Society of Gene and Cell Therapy

May 12, 2020

—Follow-up of 12 to 36 Months for FA RP-L102 “Process A” Demonstrates Prolonged Engraftment and Continued Hematologic Correction—

—Sustained Efficacy in LAD-I Provides Initial Proof of Concept for Lentiviral Platform and “Process B” Manufacturing—

—Longer-term “Process B” Data for FA and LAD-I on Track for End of Year—

NEW YORK--(BUSINESS WIRE)--May 12, 2020-- [Rocket Pharmaceuticals, Inc.](#) (NASDAQ: RCKT) (“Rocket”), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders, today presents new clinical data supporting longer-term efficacy and durability of gene therapy for Fanconi Anemia (FA) and Leukocyte Adhesion Deficiency-I (LAD-I) at the 23rd Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) being held virtually May 12-15, 2020. Two oral presentations highlight updates from the company’s Phase 1/2 study of RP-L201 for the treatment of severe LAD-I and the Phase 1/2 study of RP-L102 “Process A” for the treatment of FA.

“The latest additional data from both our LAD-I and FA programs demonstrate sustained engraftment and durable clinical impact,” said Jonathan D. Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. “These results further support the viability of gene therapy in LAD-I and FA, disorders in which bone marrow transplant is the primary curative option and is associated with high rates of toxicity.”

“Patients with severe LAD-I have neutrophil CD18 expression of less than 2% of normal, with extremely high mortality in early childhood,” said Dr. Schwartz. “In this first patient treated with RP-L201 using ‘Process B’, an increase from less than 1% to 47% CD18 expression sustained over six months demonstrates that RP-L201 has the potential to correct the neutrophil deficiency that is the hallmark of LAD-I. We are also pleased with the continued visible improvement of multiple disease-related skin lesions. These results lend further support to the applicability of ‘Process B’ across the lentiviral portfolio. The second patient has also recently been treated, and we look forward to completing the Phase 1 portion of the registrational trial for this program.”

Dr. Schwartz continued, “In our FA program, patients followed for a year or more after treatment with RP-L102 ‘Process A’ continue to demonstrate durable engraftment and hematologic correction, without the use of pre-treatment conditioning regimens. All six patients who received minimally adequate drug product and were followed for more than one year display sustained and progressive engraftment. Notably, hemoglobin levels have normalized to baseline in two patients treated. Today’s update not only gives us confidence as we transition to our improved ‘Process B’ drug product, but also supports the potential of gene therapy in the absence of any conditioning regimen as a definitive hematologic treatment for FA. The ability to treat patients without the side effects associated with allogeneic transplant or the use of genotoxic conditioning, and to restore blood cell counts is a major milestone for the FA scientific community.”

Details on Rocket’s oral presentations at ASGCT:

Title: *A Phase 1/2 Study of Lentiviral-mediated Ex-vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Initial Results from the First Treated Patient*

Session: HSPC Gene Therapies for Blood and Immune Disorders

Presenter: Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA and principal investigator of the Phase 1 trial

Date: Tuesday May 12, 2020

Time: 4:30 p.m. - 4:45 p.m. EDT

Additional results from the first patient treated with RP-L201 for LAD-I continue to demonstrate evidence of safety and potential efficacy. Analyses of peripheral vector copy number (VCN) and CD18-expressing neutrophils were performed six months post treatment with RP-L201 to evaluate engraftment and phenotypic correction. The patient demonstrated peripheral blood VCN levels of 1.3 and CD18-expression of 47%, which is sustained from the 45% expression observed three months post treatment; pretreatment CD18 expression was <1%. The drug product VCN was 3.8. Additionally, the patient continues to display visible improvement of skin lesions. No safety or tolerability issues related to RP-L201 administration have been identified to date.

RP-L201 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 Fundación Jiménez Díaz (IIS-FJD). The lentiviral vector was developed in a collaboration between The University College of London (UCL) and CIEMAT.

Title: *Updated Results of a European Gene Therapy Trial in Fanconi Anemia Patients, Subtype A*

Session: HSPC Gene Therapies for Blood and Immune Disorders

Presenter: Juan A. Bueren, Ph.D., Scientific Director of the FA gene therapy program and Head of the Hematopoietic Innovative Therapies Division at CIEMAT in Spain / CIBERER / IIS-FJD

Date: Tuesday May 12, 2020

Time: 4:45 p.m. - 5:00 p.m. EDT

Nine pediatric patients have been enrolled and treated in the Phase 1/2 clinical trial of RP-L102 "Process A" for the treatment of Fanconi Anemia, seven of whom are evaluable at or beyond the one year mark following treatment. The first four patients (02002, 02004, 02005 and 02006) exhibit robust and durable engraftment, continued hematologic correction and blood count stabilization. Importantly, hemoglobin levels for patients 02002 and 02006 have increased to a healthy, normal range; these patients received more optimal product consistent with the minimal dose criteria established for the "Process B" registrational program. Two additional patients (02008 and 02013) who have been followed for a year or more after treatment display early evidence of engraftment, as measured by increases in peripheral blood VCNs. Patient 02007 received a lower than optimal dose and is beginning to demonstrate preliminary signs of engraftment. Blood counts are not yet available in these patients. Two patients, patients 01003 and 02009, have not been included in this analysis. Patient 02009 is only six months post treatment and will continue to be followed. Patient 01003 received a drug product that did not meet full release criteria due to a technical issue – this was a one-time lab-specific issue that was addressed. To date, no patients in this trial have undergone allogeneic bone marrow transplant.

RP-L102 is being developed in conjunction with CIEMAT, CIBERER and IIS-FJD.

The presentations will be made available on Rocket's website at www.rocketpharma.com/asqct-presentations/ following presentation at the conference.

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 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. Mutation 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 cells¹.

¹Soulier, J., et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. Blood 105: 1329-1336

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 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's pre-clinical pipeline program is for Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 March 31, 2020, filed May 8, 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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