



Rocket Pharmaceuticals Presents Positive Clinical Data from its Fanconi Anemia and Leukocyte Adhesion Deficiency-I Programs at the 62nd American Society of Hematology Annual Meeting

December 7, 2020

–Two of Three FA “Process B” Patients with at Least 12-Months Follow-Up and Three of Four Additional Patients with Shorter Follow-Up Demonstrate Evidence of Engraftment—

–Follow-Up Data from Phase 1/2 Trial for Leukocyte Adhesion Deficiency-I Demonstrate Durable CD18 Expression—

–Both Trials on Track and Continue to Support Potential Registration Path Using “Process B”–

NEW YORK--(BUSINESS WIRE)--Dec. 7, 2020-- [Rocket Pharmaceuticals, Inc.](https://www.rocketpharma.com) (NASDAQ: RCKT) (“Rocket”), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today presents updated interim data from its Fanconi Anemia (FA) and Leukocyte Adhesion Deficiency-I (LAD-I) programs at the 62nd American Society of Hematology (ASH) Annual Meeting. The data are highlighted in two oral presentations.

“We are highly pleased with the data presented at ASH demonstrating ongoing evidence of efficacy and durability using ‘Process B’ in both FA and LAD-I as we move towards potential registration,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “Follow-up data from the Phase 1 and 2 trials for FA continue to support RP-L102 as a potential hematologic treatment option in the absence of cytotoxic conditioning. In five of the seven patients treated as of October 2020, there was evidence of engraftment. In addition, stabilization of peripheral blood counts in two of the three patients with at least 12-month follow-up, which declined substantially in these patients prior to gene therapy, suggests a halt in bone marrow failure progression. We look forward to reporting longer-term follow-up on these patients in the first half of 2021.”

Dr. Shah continued, “Additionally, we continue to see encouraging evidence of efficacy for RP-L201 for the treatment of LAD-I. Patients have shown sustained CD18 expression of 23% to 40%, far exceeding the 4-10% threshold associated with survival into adulthood. These data, on top of our exciting results from our lentiviral program for PKD, show our steady progress across three of our five gene therapy programs. We are proud of this progress and are committed to advancing our investigational gene therapies through development for patients and families facing these devastating disorders.”

Key findings and details for each presentation are highlighted below. To access the presentations at the conclusion of the oral presentation, please visit: <https://www.rocketpharma.com/ash-presentations/>

Gene Therapy for Fanconi Anemia, Complementation Group A: Updated Results from Ongoing Global Clinical Studies of RP-L102

The data presented in the oral presentation are from seven of the nine patients treated as of the cutoff date of October 2020 in both the U.S. Phase 1 and global Phase 2 studies of RP-L102 for FA. Seven patients had follow-up data of at least 2-months, and three of the seven patients had been followed for 12-months or longer. Key highlights from the presentation include:

- RP-L102 was generally well tolerated with no significant safety issues reported with infusion or post-treatment
- Evidence of preliminary engraftment was observed in five out of seven total patients with bone marrow (BM) vector copy numbers (VCNs) from 0.16 to 0.22 (long-term follow up only) and peripheral VCNs ranging from 0.01 (2-month follow up) to 0.11 (long-term follow up)
- Two of the three patients with greater than 12-months follow up showed evidence of increasing engraftment, mitomycin-C (MMC) resistance and stable blood counts
 - One patient’s course was complicated by *Influenza B* resulting in progressive BM failure. The patient received a successful bone marrow transplant

Presentation Details:

Title: Gene Therapy for Fanconi Anemia, Complementation Group A: Updated Results from Ongoing Global Clinical Studies of RP-L102

Session Title: Gene Editing, Therapy and Transfer I

Presenter: Agnieszka Czechowicz, M.D., Ph.D., Assistant Professor of Pediatrics, Division of Stem Cell Transplantation, Stanford University School of Medicine

Session Date: Monday, December 7, 2020
Session Time: 11:30 a.m. - 1:00 p.m. (Pacific Time)
Presentation Time: 12:15 p.m. (Pacific Time)

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

The data presented in the oral presentation are from three 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. Patient L201-003-1001 was 9-years of age at enrollment and had been followed for 12-months as of a cutoff date of November 2020. Patient L201-003-1004 was 3-years of age at enrollment and had been followed for over 6-months. Patient L201-003-2006 was 7-months of age at enrollment and was recently treated with RP-L201. Key highlights from the presentation include:

- RP-L201 was well tolerated, no safety issues reported with infusion or post-treatment
- All patients achieved hematopoietic reconstitution within 5-weeks
- Peripheral blood VCN and neutrophil CD18-expression were assessed post-treatment to evaluate engraftment and phenotypic correction:
 - 12 months post-treatment, Patient L201-003-1001 demonstrated durable CD18 expression of approximately 40%, peripheral blood VCN levels of 1.2, resolution of skin lesions
 - 6-months post-treatment, Patient L201-003-1004 demonstrated CD18 expression of 23% and peripheral blood VCN kinetics similar to those of the first patient
 - 2-months post-treatment, Patient L201-003-2006 demonstrated CD18 expression of 76% and peripheral blood VCN kinetics similar to those of the first patient

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.

Presentation Details:

Title: 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: Gene Editing, Therapy and Transfer I

Presenter: Donald 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: Monday, December 7, 2020

Session Time: 11:30 a.m. - 1:00 p.m. (Pacific Time)

Presentation Time: 12:30 p.m. (Pacific Time)

Conference Call Details

Rocket management will host a conference call and webcast today December 7, at 6:00 p.m. EST. To access the call and webcast, please click [here](#). The webcast replay will be available on the Rocket website following the completion of the call.

Investors may listen to the call by dialing (866) 866-1333 from locations in the United States or +1 (404) 260-1421 from outside the United States. Please refer to conference ID number 50038102

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 cells.

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) ("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 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 September 30, 2020, filed November 6, 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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Source: Rocket Pharmaceuticals, Inc.