Rocket Pharmaceuticals Presents Positive Clinical Data from Fanconi Anemia, Leukocyte Adhesion Deficiency-I, and Pyruvate Kinase Deficiency Programs at 24th Annual Meeting of the American Society of Gene and Cell Therapy

May 13, 2021

—RP-L102 for Fanconi Anemia Shows Evidence of Preliminary Engraftment in at Least Six of Nine Patients—

— RP-L201 for LAD-I Leads to Durable CD18 Expression, Improved Skin Lesions, and Consistent Peripheral Vector Copy Numbers Up to 18 Months Post-treatment—

—Sustained Hemoglobin Improvements Up to 9 Months Post-treatment with RP-L301 for PKD —

— Positive Benefit/Risk Profile Observed Across LVV Programs as Rocket Advances Towards Regulatory Submissions—

CRANBURY, N.J.--(BUSINESS WIRE)--May 13, 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 clinical data from its Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), and Pyruvate Kinase Deficiency (PKD) gene therapy programs presented at the 24th American Society of Gene and Cell Therapy (ASGCT) Annual Meeting.

"We are very excited to report positive clinical results from three of our lentiviral-based gene therapy programs at this year's ASGCT, which show the great potential of these therapies to successfully treat FA, LAD-I and PKD. In the case of RP-L102 for FA and RP-L201 for LAD-I, the new data advance us closer to regulatory submissions," said Gaurav Shah, M.D., Chief Executive Officer of Rocket. "At least six out of nine patients in our FA Phase 1 and 2 trials now show evidence of engraftment, further supporting the potential of RP-L102 to serve as a hematologic treatment option for FA in the absence of cytotoxic conditioning. Although preliminary, four out of the five patients anticipated necessary for a positive trial outcome have initially met the minimum 10% MMC resistance threshold in the bone marrow on at least one occasion, including two patients at 6-months post-treatment."

Dr. Shah continued, "In our Phase 1/2 trial for LAD-I, all four patients with follow-up ranging from 3 to 18 months had CD18 expression that substantially exceeded the 4-10% threshold associated with survival into adulthood and consistent peripheral blood vector copy number, further demonstrating the potential of RP-L201 to yield durable clinical benefit. All of these patients have been free of serious infections since hospital discharge following RP-L201 therapy. Lastly, data from our Phase 1 trial of RP-L301 for PKD show that both patients' hemoglobin levels have safely normalized, with neither patient requiring red blood cell transfusions after hematopoietic reconstitution while demonstrating improving hemolysis markers. We are proud of the progress we have made across all three programs and look forward to further advancing our investigational gene therapies to offer curative treatments to patients with these devastating diseases."

Gene Therapy for Fanconi Anemia [Group A]: Preliminary Results of Ongoing RP-L102 Clinical Trials

The data described in the presentation are from nine pediatric patients treated with RP-L102, Rocket's *ex vivo* lentiviral gene therapy candidate for FA.

- RP-L102 demonstrated a highly favorable safety profile with all subjects being treated without conditioning and with no sign of dysplasia or other concerning features. One patient experienced a Grade 2 transient infusion-related reaction.
- Increasing evidence of engraftment was observed in two patients with at least 15-months of follow-up and four patients with at least 6-months of follow-up as indicated by peripheral blood VCN. Patient 2, who was further along in bone marrow failure and also had complications due to a previously disclosed influenza B infection contracted during the early stage of treatment, was withdrawn from the trial at 18-months post-treatment..

  - At 24-months post-treatment, Patient 1 demonstrated a 16% BM progenitor resistance to 10 nM MMC and peripheral blood VCN levels of 0.32.
  - At 15-months post-treatment, Patient 3 demonstrated a 29% BM progenitor resistance to 10 nM MMC and peripheral blood VCN levels of 0.12.

- At 6-months post-treatment, Patients 4 and 5 had early evidence of BM progenitor resistance with 25% and 14% respectively to 10 nM MMC, consistent with the BM VCNs observed at this timepoint. Patient 4 and 5 also demonstrated peripheral blood VCN levels of 0.07 and 0.06, respectively.
- Patient 6 demonstrated peripheral blood VCN levels of 0.08 at 4-months post-treatment and Patient 7 demonstrated peripheral blood VCN levels of 0.04 at 6-months post-treatment. BM assessments in Patients 6 and 7 were deferred due to COVID-19 travel concerns.
- Patients 8 and 9 were treated more recently with less than 6-months follow-up, and one demonstrated detectable VCN.

Presentation Details:
Session: Hematologic and Immunologic Diseases
Presenter: Agnieszka Czechowicz, M.D., Ph.D., Assistant Professor of Pediatrics, Division of Stem Cell Transplantation, Stanford University School of Medicine
Date: Tuesday, May 11, 2021
Time: 8:00-10:00 a.m. EDT

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

The data presented in the oral presentation are from four pediatric patients with severe LAD-I, as defined by CD18 expression of less than 2%, who were treated with RP-L201, Rocket's *ex-vivo* lentiviral gene therapy candidate. The safety profile of RP-L201 appears favorable with all infusions well tolerated and no drug product-related serious adverse events (SAEs) .

Preliminary efficacy was evident in all four patients, including two patients with at least 9-months of follow-up. All four patients demonstrated CD18 expression consistent with the reversal of severe LAD-I phenotype.

- 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 Patient 1.
- 6-months post-treatment, Patient 2006 demonstrated CD18 expression of 74%; 3-months post-treatment, peripheral blood VCN levels were 2.3 with kinetics consistent with those of Patient 1.
- 3-months post-treatment, Patient 2005 demonstrated CD18 expression of 51%; 2-months post-treatment, peripheral blood VCN levels were 0.8 with kinetics consistent with those of Patient 1.

Most importantly, each of these patients were able to leave the hospital in the weeks following RP-L201 therapy, and all have been at home without any serious or severe infections following hospital discharge.

Presentation Details:
Session: Genetic Blood and Immune Disorders
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
Date: Tuesday, May 11, 2021
Time: 6:15-6:30 p.m. EDT

Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Updated Results of a Global Phase 1 Study for Adult and Pediatric Patients

The data presented in the oral presentation are from two adult patients with significant anemia and transfusion requirements. The patients were treated with RP-L301, Rocket's *ex vivo* lentiviral gene therapy candidate for PKD. RP-L301 continued to be well tolerated, with no serious safety issues or infusion-related complications observed up to 9-months post-treatment.

Preliminary efficacy was evident in both patients during the initial 9-months and 3-months post-treatment, respectively.

- Patient 1 received a CD34+ cell dose of $3.9x10^6$ cells/kilogram (kg). At 9-months post-treatment the patient demonstrated normalized hemoglobin levels of 13.1 g/dL, compared to an average pre-treatment baseline of ~7.4 grams (g)/deciliter (dL) . Peripheral blood VCN levels were 2.14 at 6-months post-treatment.
- Patient 2 received a CD34+ cell dose of $2.4x10^6$ cells/kg. At 6-months post-treatment the patient demonstrated normalized hemoglobin levels of 14.4g/dL, compared to a pre-treatment baseline of ~7.0 g/dL. Peripheral blood VCN levels were 2.55 at 3 months post-RP-L301 infusion.

Presentation Details:
Session: Gene Therapies for Hemoglobinopathies
Presenter: José Luis López Lorenzo, M.D., Hospital Universitario Fundación Jiménez Díaz, Madrid, Spain
Date: Wednesday, May 12, 2021
Time: 6:45-7:00 p.m. EDT

In addition, the following presentations at this year's conference also detail results from Rocket Pharma clinical studies:

Title: Gene Therapy in Fanconi Anemia: Follow-Up of a Phase I/II Gene Therapy Trial in Patients with Fanconi Anemia, Subtype A

Session: Genetic Blood and Immune Disorders
Presenter: Juan A. Bueren, Ph.D., Head of the Hematopoietic Innovative Therapies Division at the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) in Spain / CIBER-Rare Diseases / IIS-Fundación Jiménez Díaz
Date: Tuesday, May 11, 2021
Time: 5:30-5:45 a.m. EDT

Select results from Dr. Bueren's presentation will also be highlighted by Paula Rio, Ph.D. Details for this Invited Presentation are as follows:

Title: Gene Therapy in Fanconi Anemia: Current Strategies to Enable the Correction of HSCs
Session: International Focus on Stem Cell Gene Therapy
Presenter: Paula Río, Ph.D., Senior Researcher, Hematopoietic Innovative Therapies Division at CIEMAT in Spain / CIBER-Rare Diseases / IIS-Fundación Jiménez Díaz
Date: Thursday May 13, 2021
Time: 10:00-11:45 a.m. EDT

Title: LV-Mediated Gene Therapy of Pyruvate Kinase Deficiency
Session: Cutting Edge Gene and Cell Therapy Research in Europe (Organized by ESGCT)
Presenter: Jose-Carlos Segovia, Head of the Differentiation and Cytometry Unit, Hematopoietic Innovative Therapies Division at CIEMAT in Spain / CIBER-Rare Diseases / IIS-Fundación Jiménez Díaz
Date: Wednesday May 12, 2021
Time: 10:52-11:18 a.m. EDT

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.

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 Pyruvate Kinase Deficiency
Pyruvate kinase deficiency (PKD) is a rare, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red cell destruction and the disorder ranges from mild to life-threatening anemia. PKD has an estimated prevalence of 3,000 to 8,000 patients in the United States and the European Union. Children are the most commonly and severely affected subgroup of patients. Currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload.

RP-L301 was in-licensed from the Centro de Investigaciones Energeticas, Medioambientales y Tecnologicas (CIEMAT), Centro de Investigacion Biomedica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigacion Sanitaria Fundacion Jimenez Diaz (IIS-FJD).

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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