Rocket Pharmaceuticals Presents Positive Clinical Data from Company's Lentiviral Gene Therapies for Treatment of Fanconi Anemia, LAD-I and PKD at the 63rd American Society of Hematology (ASH) Annual Meeting

December 13, 2021

—Evidence of engraftment observed in six patients with at least 12-months of follow-up after treatment with RP-L102 in Fanconi Anemia with MMC resistance between 16% and 63% at a minimum of one timepoint—

—All eight initial patients with follow-up from 3 to 24-months treated with RP-L201 demonstrated durable neutrophil CD18 expression that exceeded 4-10% threshold associated with survival into adulthood and consistent with reversal of severe LAD-I phenotype; Ninth and final patient recently treated—

—RP-L301 conferred sustained normal-range hemoglobin through 12-months post-treatment in two adult PKD patients—

—Webcast to be held tomorrow,Tuesday, Dec. 14, at 7:30 a.m. ET—

CRANBURY, N.J.--(BUSINESS WIRE)--Dec. 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 updates from its ongoing Phase 2 registrational trials for Fanconi Anemia (FA) and Leukocyte Adhesion Deficiency-I (LAD-I) and its ongoing Phase 1 trial for Pyruvate Kinase Deficiency (PKD) at the 63rd American Society of Hematology (ASH) Annual Meeting.

"The positive updates presented across our Fanconi Anemia, LAD-I and PKD programs highlight the continued progress and importance of our lentiviral platform in providing potentially curative therapies for rare and devastating bone marrow-derived diseases," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "We have now dosed 11 Fanconi Anemia patients and have at least 12-months of follow-up on eight of these patients in our clinical trial of RP-L102. In six of these eight patients, we see evidence of engraftment and bone marrow mitomycin-C, or MMC, resistance ranging from 16% to 63% measured at least at one timepoint. MMC resistance is a key indicator of the ability of bone marrow stem cells to resist DNA damage, a process that is impaired in Fanconi Anemia. As a reminder, a minimum of five patients with increased MMC resistance greater than or equal to 10% above baseline at two or more timepoints and evidence of clinical stabilization will be required for statistical significance. The increasing MMC resistance in these six patients is encouraging as we move closer toward potential topline readout."

Dr. Shah continued, "We are equally pleased to have completed enrollment of all nine patients in the Phase 1/2 trial of RP-L201 for LAD-I and that evidence of meaningful clinical activity has been observed in the first eight patients for whom there is at least three months of follow-up. Notably, four patients have follow-up of at least 12-months, and none of these patients have had serious infections or required hospitalization following treatment with RP-L201. Finally, the updated data from our global Phase 1 PKD study showed doubling of baseline hemoglobin to normal-range levels, improved hemolysis parameters and red blood cell transfusion independence following engraftment in the two adult patients with severe PKD who were treated with RP-L301. Taken together, I am very pleased about the strong progress of our LVV programs and our team's focus on consistent and reliable execution."

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

The ASH poster presentation included preliminary data from 11 pediatric patients who were treated as of the Nov. 1, 2021, cut-off date with RP-L102, Rocket's *ex-vivo* lentiviral gene therapy candidate for FA. The tolerability profile of RP-L102 appears favorable and all patients were treated without conditioning. As previously reported, one patient experienced a Grade 2 transient infusion-related reaction.

Evidence of engraftment has been observed in six of eight patients with at least 12-months of follow-up. Sustained peripheral blood vector copy number (VCN) levels were seen in six of seven patients with at least 12-months of follow-up. As previously reported, Patient 2, who was further along in bone marrow failure and had complications due to a previously disclosed influenza B infection contracted during the months subsequent to treatment, was withdrawn from the trial at 18-months post-treatment.

Patient Number	Bone Marrow Assessment Performed (Months)	BM CFC MMC Resistance at 10nM MMC (%)

1 (1001)*	24	16**
3 (2004)	21	63
4 (2008)	12	21
5 (2009)	12	29
6 (2010)	12	42
7 (2011)	12	31
8 (2014)	12	0

*Patient 1, 24-months post-treatment, demonstrated a 16% BM progenitor resistance to 10 nM MMC and had modest decline in blood counts with potential stabilization at approximately 21-months and no transfusions required

**Assessment was not performed at study's centralized laboratories

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 ASH oral presentation included preliminary data from eight of nine severe LAD-I patients, as defined by CD18 expression of less than 2%, who received RP-L201 treatment as of the Nov. 8, 2021, data cut-off date. Eight patients had follow-up data of at least three months, and four of the eight patients had been followed for 12-months or longer. One patient recently received RP-L201 infusion after the data cut-off date.

All infusions of RP-L201 were well tolerated and no drug product-related serious adverse events (SAEs) were reported. Evidence of preliminary efficacy was observed in all eight evaluable patients. All eight patients demonstrated durable neutrophil CD18 expression that exceeded the 4-10% threshold associated with survival into adulthood and consistent with reversal of the severe LAD-I phenotype. Peripheral blood VCN levels have been stable and in the 0.54 – 2.94 copies per genome range. No patients had LAD-I related infections requiring hospitalization after hematopoietic reconstitution post-RP-L201.

Patient Number	Bone Marrow Assessment Performed (Months)	CD18 Expression in % of Neutrophils (%)	Peripheral Blood VCN Levels (Copies per Genome)
1001	24	40	1.53
1004	12	36	0.88
2005	12	87	0.80 (Demonstrated at 6-months post-treatment)
2006	12	73	2.49 (Demonstrated at 9-months post-treatment)
2007	6	87	2.94
2008	6	52	1.39 (Demonstrated at 3-months post-treatment)
2009	3	26	0.54
2011	3	56	1.17

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

The ASH poster presentation included preliminary data from two adult patients with significant anemia and transfusion requirements who were treated as of the Nov. 3, 2021, cut-off date with RP-L301, Rocket's *ex-vivo* lentiviral gene therapy candidate for PKD. RP-L301 continues to be well tolerated, with no drug product related SAEs or infusion-related complications observed through 12-months post-treatment.

Preliminary clinical activity was observed in both patients at 12-months post-RP-L301 infusion. Both patients have reported improved quality of life following treatment.

- Patient 1 received a CD34+ cell dose of $3.9x10^6$ cells/kilogram (kg). At 12-months post-treatment the patient had sustained improvement in hemoglobin levels of 13.3 grams (g)/deciliter (dL), compared to an average pre-treatment baseline of ~7.4 g/dL.
- Patient 2 received a CD34+ cell dose of $2.4x10^6$ cells/kg. At approximately 12-months post-treatment the patient had normalized hemoglobin levels of 14.8 g/dL, compared to a pre-treatment baseline of ~7.0 g/dL.

Investor and Analyst Event Details

Rocket will host an in-person Investor and Analyst Event that will simultaneously be webcast tomorrow, Dec. 14, at 7:30 a.m. ET to discuss the FA, LAD-I and PKD data presented at ASH. Investors may register to attend the event in person by emailing investors@rocketpharma.com. A simultaneous webcast of the event and the presentation will be available under "Events" in the Investors section of the Company's website at: https://ir.rocketpharma.com/. The webcast replay will be available on the Rocket website upon completion of the event. This meeting is NOT an official program of the ASH annual meeting.

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 the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease including whether preliminary results of its trials will be representative of later stage or final results, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the potential of and timing for registration filings with the FDA, effectiveness and timing of related preclinical 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 preclinical 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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