Rocket Pharmaceuticals Announces Presentations Highlighting Lentiviral Gene Therapies at the 29th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT)

October 12, 2022

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 12, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today announces data presentations at the 29th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT) in Edinburgh, United Kingdom, taking place October 11-14, 2022. Presentations will include clinical data from Rocket's lentiviral vector (LV)-based gene therapy programs for Leukocyte Adhesion Deficiency-I (LAD-I), Fanconi Anemia (FA) and Pyruvate Kinase Deficiency (PKD). Donald B. Kohn, MD, Distinguished Professor of Microbiology, Immunology & Molecular Genetics, Pediatrics, and Molecular & Medical Pharmacology at University of California, Los Angeles (UCLA) and Director of the UCLA Human Gene and Cell Therapy Program, will also give an Invited Talk incorporating previously disclosed data from the RP-L201 trial for LAD-I.

Positive Updated Safety and Efficacy Data from Phase 2 Pivotal Trial for Fanconi Anemia (FA)

The poster and presentation include updated safety and efficacy data from the Phase 2 pivotal trial of RP-L102, Rocket's *ex-vivo* lentiviral gene therapy candidate for the treatment of FA.

- At six months post-infusion, at least one additional patient has demonstrated early signs of engraftment, at levels similar to the five of nine evaluable patients who had increased bone marrow cell resistance to mitomycin-C, ranging from 51% to 94% at 18-24 months, and sustained ≥20% at consecutive timepoints post RP-L102 infusion.
- With regard to the safety update, one of the patients with confirmed engraftment developed a T-cell lymphoblastic lymphoma that was conclusively determined by the investigator, sponsor and the independent data monitoring committee to be related to FA (a cancer predisposition syndrome) and unrelated to RP-L102 (of note, chemotherapy conditioning is not a part of RP-L102 therapy). The information has been shared with the FDA, with no impact to the clinical trial or filings.
    - The patient tolerated induction chemotherapy for lymphoma without significant complications and is currently in complete remission.
    - The presence of RP-L102 mediated gene corrected hematopoietic cells may have facilitated the patient's tolerance of anti-cancer therapy.
    - Intensive evaluation of the tumor indicated negligible vector copy number (VCN) of 0.003 at a juncture two-years post-treatment, whereas VCNs in blood and bone marrow were substantial, 0.26 and 0.42, respectively.
- Updated results from the Phase 2 pivotal trial of RP-L102 for FA remain on track for later this quarter and regulatory filings continue to be anticipated in 2023.

Positive Top-line Clinical Data from Phase 2 Pivotal Trial for Severe Leukocyte Adhesion Deficiency-I (LAD-I)

The oral presentation includes previously disclosed efficacy and safety data at three to 24 months of follow-up after RP-L201 infusion for all patients and overall survival data for seven patients at 12 months or longer after infusion. RP-L201 is Rocket's *ex-vivo* lentiviral gene therapy candidate for the treatment of severe LAD-I.

- Observed 100% overall survival at 12 months post-infusion via Kaplan Meier estimate and a statistically significant reduction in all hospitalizations, infection- and inflammatory-related hospitalizations and prolonged hospitalizations for all nine LAD-I patients with three to 24 months of available follow-up. Data also shows evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities.
- The safety profile of RP-L201 has been highly favorable in all patients with no RP-L201-related serious adverse events to date. Adverse events related to other study procedures, including busulfan conditioning, have been previously disclosed and consistent with the safety profiles of those agents and procedures.

- Based on the positive efficacy and safety data from the Phase 2 pivotal LAD-I trial, Rocket has initiated discussions with the FDA and anticipates regulatory filings in the first half of 2023.

Interim Data from Ongoing Phase 1 Trial for Pyruvate Kinase Deficiency (PKD)

The poster and presentation include previously disclosed safety and efficacy data from the Phase 1 trial of RP-L301, Rocket's *ex-vivo* lentiviral gene therapy candidate for the treatment of PKD.

- At 18 months post-infusion, both adult patients have sustained transgene expression, normalized hemoglobin, improved hemolysis, no red blood cell transfusion requirements post-engraftment and improved quality of life both reported anecdotally and as documented via formal quality of life assessments.
- The safety profile of RP-L301 appears favorable, with no IP-related serious adverse events 18 months post-infusion. Transient transaminase elevation was seen in both patients post-therapy/conditioning, with no clinical stigmata of liver injury and subsequent resolution without clinical sequelae.
- Updated preliminary results from the Phase 1 trial of RP-L301 for PKD remain on track for later this quarter. Pediatric patients are currently being enrolled and treated.

Details for Rocket's Invited Talk and poster presentations are as follows:

Title: Interim Results from an ongoing Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)
Session: Clinical Trials (Plenary 2)
Presenter: Donald B. Kohn, MD - University of California, Los Angeles, Distinguished Professor of Microbiology, Immunology & Molecular Genetics (MIMG), Pediatrics, and Molecular & Medical Pharmacology; Director of the UCLA Human Gene and Cell Therapy Program
Session date and time: Wednesday, 12 October at 11:10-13:15 BST
Location: Edinburgh International Conference Centre (EICC)
Presentation Number: INV20

Title: Lentiviral-Mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Results from Global RP-L102 Clinical Trials
Session: Poster Session 1
Presenter: Julián Sevilla MD, PhD - Fundación para la Investigación Biomédica, Hospital Infantil Universitario Niño Jesús
Session date and time: Wednesday, 12 October at 19:30-21:00 BST
Location: Edinburgh International Conference Centre (EICC)
Poster Number: P139

Title: Preliminary Conclusions of the Phase I/II Gene therapy Trial in Patients with Fanconi Anemia-A
Session: Blood Diseases: Haematopoietic Cell Disorders
Presenter: Juan Bueren, PhD - Unidad de Innovación Biomédica, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT)
Session date and time: Thursday, 13 October at 15:30-17:30 BST
Location: Edinburgh International Conference Centre (EICC)
Presentation Number: INV41

Title: Interim Results from an Ongoing Global Phase 1 Study of Lentiviral-Mediated Gene Therapy for Pyruvate Kinase Deficiency
Session: Poster Session 2
Presenter: José Luis López Lorenzo, MD, Hospital Universitario Fundación Jiménez Díaz
Session date and time: Thursday, 13 October at 17:30-19:15 BST
Location: Edinburgh International Conference Centre (EICC)
Poster Number: P128

Abstracts for the presentations can be found online at: https://www.esgct.eu/.

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. Mutations 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 4,000 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 Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz (IIS-FJD).

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to 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, 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. 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 2022 in light of COVID-19, 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), and Danon Disease, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its Danon Disease program 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, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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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