Rocket Pharmaceuticals Announces Presentations Highlighting Lentiviral Gene Therapies at the 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT)

October 19, 2021

—Incremental updates in LAD-I and PKD and previously disclosed data in FA to be presented—

—Clinical updates across all programs anticipated later in Q4—

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 19, 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 data presentations at the 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT) taking place virtually October 19-22, 2021. Oral presentations will include clinical data from Rocket's lentiviral vector (LVV)-based gene therapy programs for Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Fanconi Anemia (FA). Elena Almarza, PhD, senior scientist at Rocket, will also give an Invited Talk on the multi-step development path for gene therapy in LAD-I, including vector design and comprehensive pre-clinical evaluation leading to robust clinical proof-of-concept.

These oral presentations will include interim data updates from additional patients in the RP-L201 trial for LAD-I, additional vector copy number data from the RP-L301 trial in PKD and previously disclosed data from RP-L102 in FA. Comprehensive updates for all the company's programs, which include Danon, FA, LAD-I, PKD and IMO, remain on track and are anticipated for later in Q4.

Details for Rocket's oral presentations are as follows:

Title: 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
Session: Primary Immunodeficiencies (Session 2a)
Presenter: Claire Booth, MBBS, PhD, FRCPCH, Infection, Immunity, & Inflammation Department, UCL Great Ormond Street Institute of Child Health, London, United Kingdom
Date: Wednesday, October 20, 2021
Session Time: 9:00 a.m. – 11:00 a.m. CEST / 3:00 a.m. – 5:00 a.m. ET
Presentation Time: 10:30 a.m. CEST / 4:30 a.m. ET
Presentation Number: OR09

Rocket's LAD-I trial will also be featured in an Invited Talk:

Title: Gene Therapy for Leukocyte Adhesion Deficiency Type I (LAD-I): A 12-year Journey
Session: Primary Immunodeficiencies (Session 2a)
Presenter: Elena Almarza, PhD, Senior Scientist, Rocket Pharmaceuticals
Date: Wednesday, October 20, 2021
Session Time: 9:00 a.m. – 11:00 a.m. CEST / 3:00 a.m. – 5:00 a.m. ET
Presentation Time: 9:00 a.m. CEST / 3:00 a.m. ET
Presentation Number: INV14

Title: Gene Therapy for Fanconi Anemia [Group A]: Interim Results of RP-L102 Clinical Trials
Session: Hematopoietic & Bleeding Disorders I (Session 4c)
Presenter: Julián Sevilla, MD, PhD, Hematología y Hemoterapia, Fundación para la Investigación Biomédica HIUNJ, Hospital Infantil Universitario Niño Jesús, Madrid, Spain
Date: Thursday, October 21, 2021
Session Time: 9:00 a.m. – 11:00 a.m. CEST / 3:00 a.m. – 5:00 a.m. ET
Presentation Time: 10:00 a.m. CEST / 4:00 a.m. ET
Presentation Number: OR35

Title: Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Interim Results of a Global Phase 1 Study for Adult and Pediatric Patients
Session: Hematopoietic & Bleeding Disorders II (Session 8a)

Presenter: José Luis López Lorenzo, MD, Hospital Universitario Fundación Jiménez Díaz, Madrid, Spain; Unidad Mixta de Terapias Avanzadas, Instituto de Investigación Sanitaria Fundación Jiménez Díaz, Madrid, Spain
Date: Friday, October 22, 2021
Session Time: 2:00 p.m. – 4:00 p.m. CEST / 8:00 a.m. – 10:00 a.m. ET
Presentation Time: 2:45 p.m. CEST / 8:45 a.m. ET
Presentation Number: OR70

Details for Rocket's poster presentation are as follows:

Title: Preclinical Efficacy and Safety of Lentiviral (LV)-based Gene Therapy in IMO Support Clinical Trial Initiation
Presenter: Ilana Moscatelli, PhD, Department of Molecular Medicine and Gene Therapy, Lund Strategic Center for Stem Cell Biology, Lund, Sweden
Date: Available Tuesday, October 19, 2021 – Friday, October 22, 2021
Poster ID Number: P172

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

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 despite frequent antimicrobial use. 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 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 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 Infantile Malignant Osteopetrosis

Infantile Malignant Osteopetrosis (IMO) is a rare, severe autosomal recessive disorder caused by mutations in the *TCIRG1* gene with the function of osteoclasts, cells which are essential for normal bone remodeling and growth. These mutations lead to skeletal malformations, including fractures and cranial deformities which cause neurologic abnormalities including vision and hearing loss. Patients often have endocrine abnormalities and progressive, frequently fatal bone marrow failure. As a result, death is common within the first decade of life. IMO has an estimated incidence of 1 in 200,000. The only treatment option currently available for IMO is an allogenic bone marrow transplant (HSCT), which allows for the restoration of bone resorption by donor-derived osteoclasts which originate from hematopoietic cells. Long-term survival rates are lower in IMO than those associated with HSCT for many other non-malignant hematologic disorders; severe HSCT-related complications are frequent. There is an urgent need for additional treatment options.

RP-L401 was in-licensed from Lund University and Medizinische Hochschule Hannover. Rocket's IMO research is made possible by a grant from the California Institute for Regenerative Medicine (Grant Number CLIN2-12095). 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 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 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, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold 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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