



Rocket Pharmaceuticals Announces Positive Clinical Data from RP-L201 Trial for the Treatment of LAD-I at the 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT)

October 20, 2021

—RP-L201 is well tolerated and leads to durable CD18 expression and consistent peripheral vector copy numbers in seven of the initial seven patients with follow-up ranging from 3 to 18 months —

— Positive data updates show initial clinical benefit with no patients requiring hospitalization to date for LAD-I related infections following hematopoietic reconstitution —

CRANBURY, N.J.--(BUSINESS WIRE)--Oct. 20, 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 interim data updates from the RP-L201 Phase 1/2 clinical trial for the treatment of Leukocyte Adhesion Deficiency-I (LAD-I) at the 28th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT). Severe LAD-I is a rare pediatric disease that prevents patients from adequately combating infections. LAD-I leads to recurrent life-threatening bacterial and fungal infections that respond poorly to antimicrobials, require frequent hospitalizations and are ultimately fatal.

"We are excited to share positive data updates from our Phase 1/2 trial for LAD-I and are pleased to report that in each of the initial seven RP-L201 treated patients, CD18 expression has significantly exceeded the 4-10% threshold associated with survival into adulthood, with consistent peripheral blood vector copy number levels," said Jonathan Schwartz, M.D., Chief Medical Officer of Rocket Pharma. "These positive data continue to support the potential of RP-L201 to yield durable clinical benefit in patients with severe LAD-I and advances our potentially registration-enabling dataset in this fatal disorder and across Rocket's lentiviral programs at large. We look forward to reporting additional clinical data later in the fourth quarter."

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 at ESGCT are from the initial seven 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 seven patients, including two patients with at least 12 months of follow-up. All seven 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 vector copy number (VCN) levels have been stable and in the 0.5 – 2.5 copy per genome range. No patients have had LAD-I related infections requiring hospitalization subsequent to hematopoietic reconstitution post RP-L201.

- 18 months post-treatment, Patient 1001 demonstrated sustained CD18 expression in 43.7% of neutrophils, peripheral blood VCN levels of 1.43 copies per genome and resolution of pre-existing skin lesions.
- 9 months post-treatment, Patient 1004 demonstrated sustained CD18 expression in 28.2% of neutrophils and peripheral blood VCN levels of 0.88 copies per genome at 12 months post treatment.
- 9 months post-treatment, Patient 2006 demonstrated sustained CD18 expression in 70.9% of neutrophils and peripheral blood VCN levels of 2.49 copies per genome.
- 6 months post-treatment, Patient 2007 demonstrated 86.6% neutrophil CD18 expression and peripheral blood VCNs of 2.4 at 3 months post treatment.
- 6 months post-treatment, Patient 2008 demonstrated 51.6% neutrophil CD18 expression and peripheral blood VCNs of 1.38 at 3 months post treatment.
- 3 months post-treatment, Patient 2005 demonstrated 51.2% neutrophil CD18 expression and peripheral blood VCNs of 0.79 at 6 months post treatment.
- 3 months post-treatment, Patient 2009 demonstrated 25.6% neutrophil CD18 expression and peripheral blood VCN levels of 0.54.

Information about Rocket's RP-L201 clinical program is available [here](#).

Rocket's LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (CIRM) (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 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 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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Media

Kevin Giordano
Director, Corporate Communications
kgiordano@rocketpharma.com

Investors

Mayur Kasetty, M.D.
Director, Business Development & Operations
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.