Rocket Pharmaceuticals Presents Positive Data from LV Hematology Portfolio at the 27th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT)

May 10, 2024

Long-term KRESLADI™ follow-up data demonstrate survival of 100% in the absence of allogeneic hematopoietic stem cell transplantation (HSCT) from 18 to 45 months with a well-tolerated safety profile in all nine patients with severe LAD-I

Previously disclosed results from the global RP-L102 Fanconi Anemia Phase 1/2 trial demonstrate genetic and phenotypic correction combined with hematologic stabilization extending to 42 months with polyclonal integration patterns

Sustained and clinically meaningful hemoglobin improvement and well-tolerated safety profile in PKD patients up to 36 months after RP-L301 treatment

CRANBURY, N.J.--(BUSINESS WIRE)--May 10, 2024-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced longer-term data updates from its lentiviral (LV) vector hematology portfolio presented at the 27th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT). Data updates demonstrate the continued safety and efficacy of the Phase 1/2 pivotal studies of KRESLADI™ (marnetegragene autotemcel) for severe Leukocyte Adhesion Deficiency-I (LAD-I) and RP-L102 for Fanconi Anemia (FA), in addition to the Phase 1 study of RP-L301 for Pyruvate Kinase Deficiency (PKD).

"The positive updates presented at this year's annual meeting demonstrate the sustained safety and efficacy across the totality of our LV hematology portfolio," said Jonathan Schwartz, M.D., Chief Medical & Gene Therapy Officer, Rocket Pharmaceuticals. "Ahead of the upcoming PDUFA date, KRESLADI™ continues to demonstrate 100% HSCT-free survival and significant reductions in infection-related hospitalizations following engraftment in patients with severe LAD-I. In our pivotal studies of RP-L102 for Fanconi Anemia, we continue to see maintained genetic and phenotypic correction combined with hematologic stabilization. Additionally, our Phase 1 study of RP-L301 for PKD shows sustained clinically meaningful hemoglobin improvement in all patients. We are very pleased with the safety profile demonstrated across our LV hematology portfolio, with no drug-related serious adverse events observed to date."

Autologous *Ex-Vivo* Lentiviral Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I Provides Sustained Efficacy with a Well-Tolerated Safety Profile

The oral presentation includes positive, updated data (cut-off July 24, 2023) from the global Phase 1/2 pivotal studies demonstrating sustained efficacy and safety of KRESLADI™ from 18 to 45 months of follow-up for all nine patients with severe LAD-I.

- Observed 100% survival in the absence of allogeneic HSCT at least 18 months post-infusion in all nine patients; all patients enrolled at less than 12 months of age have surpassed 24 months without HSCT. All primary and secondary endpoints were met, including sustained genetic and phenotypic correction.
- When compared with pre-treatment history, data showed substantial decreases in the incidences of significant infections requiring hospitalization or intravenous antimicrobials, combined with evidence of resolution of LAD-I-related skin and periodontal lesions and restoration of wound repair capabilities.
- KRESLADI™ was well-tolerated in all patients with no drug-related serious adverse events reported to date. Adverse events related to other study procedures, including busulfan conditioning, have been previously disclosed and are consistent with the safety profiles of those agents and procedures. No cases of graft failure or autologous graft-versus-host-disease (GvHD) were reported.
- Based on the positive efficacy and safety data from the global pivotal studies of KRESLADI™ for severe LAD-I, the Biologics License Application (BLA) was accepted for review by the U.S. Food and Drug Administration (FDA) who has set the New Prescription Drug User Fee Act (PDUFA) target date of June 30, 2024.

Lentiviral-Mediated Gene Therapy (RP-L102) for Fanconi Anemia [Group A] is Associated with Polyclonal Integration Patterns in the

Absence of Conditioning

The oral presentation includes positive, updated data (cut-off September 11, 2023) from the global Phase 1/2 pivotal studies of RP-L102, Rocket's *ex vivo* LV gene therapy candidate for FA.

- Consistent with results observed from the clinical program, RP-L102 is a potentially curative therapy to prevent FA-related bone marrow failure (BMF), which can be administered without a suitable allogeneic donor or transplant-related toxicities.
- RP-L102 demonstrates sustained and progressively increasing genetic correction in eight of 12 patients with greater than 12 months of follow-up. Observed genetic correction is associated with phenotypic correction and hematologic stability.
- RP-L102 remains well-tolerated with no significant safety signals.
- For the first time, data demonstrate that RP-L102 confers polyclonal insertion patterns indicative of long-term hematopoietic stem cell repopulation of the bone marrow and peripheral blood and clonal diversity in the absence of conditioning.
- Based on the positive efficacy and safety data from the global pivotal studies of RP-L102 for FA, the European Medicines Agency (EMA) accepted the Marketing Authorization Application (MAA) for review. Rocket expects to submit the BLA to the FDA in the first half of 2024.

Gene Therapy for Adult and Pediatric Patients with Severe Pyruvate Kinase Deficiency: Results from a Global Study of RP-L301

The oral presentation includes positive, updated data (cut-off February 5, 2024) from the Phase 1 study from two adult patients with PKD treated with RP-L301 followed up to 36 months and two pediatric patients followed up to 12 months.

- Sustained and clinically meaningful hemoglobin improvement observed in all patients including hemoglobin normalization in three of four patients. No patients have required red blood cell transfusion following neutrophil engraftment. Improvements in hemoglobin supported by improved markers of hemolysis and quality of life have been observed.
- RP-L301 remains well-tolerated, with no drug-related serious adverse events.
- Insertion site analyses in the peripheral blood and bone marrow for both adult patients through 36 months post-RP-L301 demonstrated highly polyclonal patterns with no clonal dominance or insertional mutagenesis. Testing is ongoing for pediatric patients who were more recently treated.
- Based on the positive safety and efficacy data from the global Phase 1 study of RP-L301 for PKD, Rocket is working towards initiation of the Phase 2 pivotal study.

About KRESLADI™ (marnetegragene autotemcel)

KRESLADI™ is an investigational gene therapy for severe Leukocyte Adhesion Deficiency-I (LAD-I) that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral (LV) vector to deliver a functional copy of the *ITGB2* gene, which encodes for the beta-2 integrin component CD18, a key protein that facilitates leukocyte adhesion and enables their extravasation from blood vessels to fight infection. Rocket holds FDA Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric, and Fast Track designations in the U.S., PRIME and Advanced Therapy Medicinal Product (ATMP) designations in the EU, and Orphan Drug designations in both regions for the program. KRESLADI™ was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras and Instituto de Investigación Sanitaria Fundación Jiménez Díaz. The LV vector was developed in a collaboration between University College London and CIEMAT.

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, survival beyond childhood is rare. Currently the only potential curative treatment is an allogeneic hematopoietic stem cell transplant (HSCT), which may not be available in time for these children and itself has substantial morbidity and mortality. 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 RP-L102

RP-L102 is an investigational gene therapy for Fanconi Anemia (FA) that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral (LV) vector to contain a functional copy of the *FANCA* gene. Rocket holds FDA Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric Disease, and Fast Track designations in the U.S., PRIME and Advanced Therapy Medicinal Product (ATMP) designations in the EU, and Orphan Drug designation in both regions for the program. RP-L102 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras and Instituto de Investigación Sanitaria Fundación Jiménez Díaz.

About Fanconi Anemia

Fanconi Anemia (FA) is a rare genetic disorder characterized by bone marrow failure (BMF), cancer predisposition, and congenital malformations. In the absence of allogeneic hematopoietic stem cell transplant (HSCT), the primary cause of death among patients with FA is BMF, which typically

occurs during the first decade of life. Allogeneic HSCT, when available, corrects the hematologic component of FA, but requires myeloablative conditioning. Both chemotherapy conditioning and graft-versus-host disease, a known complication of allogeneic HSCT, are 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 lead 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 an 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. There is a high unmet medical need for patients with FA.

About RP-L301

RP-L301 is an investigational gene therapy that contains autologous hematopoietic stem cells that have been genetically modified with a lentiviral (LV) vector to contain a functional copy of the *PKLR* gene, which is responsible for energy production in red blood cells (RBCs). RBCs carry oxygen to the rest of the body. Rocket holds FDA Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations in the U.S., EMA PRIME designation in the EU, and Orphan Drug designation in both regions for the program. 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 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 blood cell destruction and potentially life-threatening anemia with a significant impact to quality of life. PKD has an estimated prevalence of 4,000 to 8,000 patients in the U.S. and Europe. Children are the most commonly and severely affected subgroup of patients. Patients with PKD have a high unmet medical need, as currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload. Mitapivat, an oral enzyme activator, is approved for use in adult patients, however its efficacy is limited in more severely-afflicted patients, most notably in those who are splenectomized, transfusion-dependent, or whose disease results from deleterious mutations.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. Rocket’s innovative multi-platform approach allows us to design the optimal gene therapy for each indication, creating potentially transformative options that enable people living with devastating rare diseases to experience long and full lives.

Rocket’s lentiviral (LV) vector-based hematology portfolio consists of late-stage programs for Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure (BMF) 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 monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

Rocket’s adeno-associated viral (AAV) vector-based cardiovascular portfolio includes a late-stage program for Danon Disease, a devastating heart failure condition resulting in thickening of the heart, an early-stage program in clinical trials for PKP2-arrhythmogenic cardiomyopathy (ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death, and a pre-clinical program targeting BAG3-associated dilated cardiomyopathy (DCM), a heart failure condition that causes enlarged ventricles.

For more information about Rocket, please visit www.rocketpharma.com and follow us on LinkedIn, YouTube, and X.

Rocket Cautionary Statement Regarding Forward-Looking Statements

This press release contains forward-looking statements concerning Rocket’s future expectations, plans and prospects that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this release are forward-looking statements. 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. These forward-looking statements include, but are not limited to, statements concerning Rocket’s expectations regarding 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), Danon Disease (DD) and other diseases, 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 DD program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, Rocket’s ability to establish key collaborations and vendor relationships for its product candidates, Rocket’s ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates, Rocket’s ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies and Rocket’s ability to transition to a commercial stage pharmaceutical company. 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 dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket’s competitors’ activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket’s ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, the integration of new executive team members and the effectiveness of the newly configured corporate leadership team, Rocket’s ability to acquire additional businesses, form strategic alliances or create joint ventures and its ability to realize the benefit of such acquisitions, alliances or joint ventures, Rocket’s ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, 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, 2023, filed February 27, 2024 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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