Rocket Pharmaceuticals Provides Regulatory Update on KRESLADI™ (marnetegragene autotemcel; marne-cel)

June 28, 2024

CRANBURY, N.J.--(BUSINESS WIRE)--Jun. 28, 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 needs, today announced a regulatory update for KRESLADI™ (marnetegragene autotemcel; marne-cel), a lentiviral (LV) vector-based gene therapy to treat severe leukocyte adhesion deficiency-I (LAD-I). The U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) in response to Rocket's Biologics License Application for KRESLADI™ wherein the FDA requested limited additional Chemistry Manufacturing and Controls (CMC) information to complete its review.

Rocket met with FDA senior leaders from Center for Biologics Evaluation and Research (CBER) to align on the limited scope of additional CMC information needed to support the approval of KRESLADI™as quickly as possible. "It is reassuring to have the FDA as a close collaborator who understands the high unmet medical need, clear clinical benefit and importance of timely patient access," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "CBER leadership's direct involvement and commitment to working expeditiously to deliver this therapy to patients gives us great hope on behalf of the primary immunodeficiency community."

Positive top-line data from the global Phase 1/2 study of KRESLADI™ demonstrated 100% overall survival at 12 months post-infusion (and for the entire duration of follow-up) for all nine LAD-I patients with 18 to 42 months of available follow-up. Data also showed large decreases compared with pre-treatment history in the incidences of significant infections, combined with evidence of resolution of LAD-I-related skin lesions and restoration of wound repair capabilities. All primary and secondary endpoints were met, and KRESLADI™ was well tolerated in all patients with no treatment-related serious adverse events.

About KRESLADI™ (marnetegragene autotemcel; marne-cel)
KRESLADI™ is an investigational gene therapy 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 lentiviral 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. LAD-I is estimated to impact an estimated 800 to 1,000 individuals in the U.S. and Europe. Currently the only potential curative treatment is an allogeneic hematopoietic stem cell transplant, 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 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 "could," "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, including the timing and outcome of the FDA's review of the additional CMC information that Rocket will provide in response to the FDA's request, the safety, effectiveness and timing of 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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