Rocket Pharmaceuticals Announces FDA Clearance of IND for RP-L401 Gene Therapy for Infantile Malignant Osteopetrosis

June 29, 2020

—Rocket's Fifth Gene Therapy Program to Enter the Clinic in the Fourth Quarter of 2020—

NEW YORK--(BUSINESS WIRE)--Jun. 29, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces that it has received clearance from the U.S. Food and Drug Administration (FDA) for the Company's Investigational New Drug (IND) application for RP-L401. RP-L401 is the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Infantile Malignant Osteopetrosis (IMO), a rare, severe monogenic bone resorption disorder characterized by skeletal deformities, neurologic abnormalities and bone marrow failure. RP-L401 was in-licensed from Lund University, under the research leadership of Dr. Johan Richter, M.D., Ph.D. and Dr. Ilana Moscatelli, Ph.D. The vector was in-licensed through a collaboration with Dr. Axel Schambach, M.D., Ph.D. of the Medizinische Hochschule Hannover.

"This FDA acceptance marks the fifth Rocket-Sponsored IND cleared for our gene therapy platform in less than two years and is an important milestone for Rocket," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "IMO represents a significant unmet medical need as patients often die in the first years of life, and the only current treatment option is a bone marrow transplant, which is associated with difficult complications. Promising preclinical data indicate that even a modest level of engraftment of 10-30% corrected hematopoietic progenitors can restore bone resorption and potentially prevent the devastating morbidity and childhood mortality associated with IMO. We are looking forward to opening the clinical trial as we work to provide a potentially curative treatment option for patients with IMO."

Kinnari Patel, Pharm. D., MBA, Chief Operating Officer and Executive Vice President, Development at Rocket, added, "Our IMO program represents the essence of Rocket's vision and mission – to find potentially curative gene therapies for rare, devastating childhood disorders. These patients need more treatment options, and the rapid advancement of this program is a testament to the hard work and expertise of the Rocket team and its academic collaborators."

The non-randomized, open-label Phase 1 clinical trial will enroll two pediatric patients, one month of age or older. The trial is designed to assess safety and tolerability of RP-L401, as well as preliminary efficacy, including potential improvements in bone abnormalities/density, hematologic status and endocrine abnormalities. University of California, Los Angeles will serve as the lead trial site. The trial will be led by principal investigator Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at the University of California, Los Angeles.

About Infantile Malignant Osteopetrosis

Infantile Malignant Osteopetrosis (IMO) is a rare, severe autosomal recessive disorder caused by mutations in the *TCIRG1* gene, which is critical for the process of bone resorption. Mutations in *TCIRG1* interfere with the function of osteoclasts, cells which are essential for normal bone remodeling and growth, leading 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.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") 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 2020 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, 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 when clinical trial sites will resume normal business operations, 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-Q for the quarter ended March 31, 2020, filed May 8, 2020 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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