Rocket Pharmaceuticals Receives FDA Fast Track Designation for RP-L401 Gene Therapy for Infantile Malignant Osteopetrosis

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—Rocket's Fifth Gene Therapy Program Receives Fast Track Designation—

NEW YORK--(BUSINESS WIRE)--Aug. 27, 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 the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to RP-L401, 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.

"We are proud to announce Rocket's fifth Fast Track designation, an accomplishment that truly embodies our mission and vision of bringing curative gene therapies to children suffering from rare disorders," said Kinnari Patel, Pharm. D., MBA, Chief Operating Officer and Executive Vice President, Development at Rocket. "Advancing five programs into the clinic in five years is a testament that we are on our way towards achieving that mission. IMO is one of the most devastating pediatric disorders, and we are grateful to our team and our collaborators for all of the work that they've done in moving this program forward for children and their families."

The FDA's Fast Track program facilitates the development of products intended to treat serious conditions that have the potential to address unmet medical needs. The designation enables greater access to the FDA for the purpose of expediting the product's development, review, and potential approval. In addition, the Fast Track program allows for eligibility for Accelerated Approval and Priority Review, if relevant criteria are met, and Rolling Review, which means a company can submit completed sections of its Biologic License Application (BLA) for review by FDA, rather than waiting until every section is completed before the entire application can be reviewed.

Rocket's non-randomized, open-label Phase 1 clinical trial of RP-L401 for the treatment of IMO 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 under 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.

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.

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.

RP-L401 was in-licensed from Lund University and Medizinische Hochschule Hannover.

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 June 30, 2020, filed August 5, 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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