Rocket Pharmaceuticals Supports Rare Disease Day® and Joins Global Movement to Raise Awareness

February 6, 2020

—Educational Event at Carnegie Hall Featuring Patient Discussions and Opportunities for Patients and Families to Engage with Physicians and Researchers in the NY Healthcare Community—

—Empire State Building to Illuminate in Pink, Blue, Purple and Green in Honor of Rare Disease Day–

NEW YORK--(BUSINESS WIRE)--Feb. 6, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, has joined forces with the 25-30 million Americans living with a rare disease and health care advocates around the world for Rare Disease Day® on February 29. Rare Disease Day is an annual awareness day dedicated to increasing public understanding of rare diseases and the impact on patients' lives.

The theme of Rocket's Rare Disease Day event, hosted in partnership with the RTW Charitable Foundation, is "I am RARE, hear me ROAR," highlighting the Roaring '20s, a decade that saw the discovery of ground-breaking medicines including penicillin and insulin, ultimately saving millions of lives. Throughout the 1920s, advancements in science led to the development of many new technologies, vaccines and drugs improving healthcare for generations to this day. With the recent advent of genomic medicine and research tools, it has been suggested this decade may demonstrate the true potential of gene therapies.

The event will highlight patient artists who raise awareness of their disease through their artwork. At the event, patients will share their personal experiences living with a rare disease, and attendees will have an opportunity to engage in discussions with patients, caregivers, patient advocates, researchers and physicians. The purpose of the event is to provide all who attend an opportunity to learn more about rare disease and the need for new treatment options. In conjunction with the event, for the second year in a row the Empire State Building will be illuminated in pink, blue, purple and green (the colors of the rare disease day movement) in honor of Rare Disease Day and those impacted by rare disease.

"At Rocket, our focus is first and foremost on the patients we are endeavoring to serve, and Rare Disease Day is the perfect time to not only bring that focus to the forefront of everyone's mind, but to give patients the opportunity to tell their stories and give researchers and those working in biotech a better understanding of the incredible individuals within the rare disease community," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development of Rocket. "The theme of Rare Disease Day this year, 'Rare is many, rare is strong and rare is proud!', brings the focus on increased equity for the rare disease community, and we welcome everyone interested to attend and learn more about how they can join this important movement. We believe that as the 1920s saw monumental strides in healthcare and medicine, the 2020s will usher in a new era of finding cures for rare genetic diseases, and we look forward to helping make an impact this decade."

According to the National Institutes of Health (NIH), a disease is rare if it affects fewer than 200,000 people in the United States. There are more than 7,000 rare diseases currently identified and approximately 90% of them are estimated to be still without FDA-approved medical treatments.

Rare Disease Day takes place every year on the last day of February (February 28 or February 29 in a leap year)—the rarest date on the calendar—to underscore the nature of rare diseases and the impact these diseases have on patients' lives. It was established in Europe in 2008 by EURORDIS, the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the U.S. by the National Organization for Rare Disorders (NORD)®, the leading independent, nonprofit organization committed to the identification, treatment, and cure of rare diseases. In 2019, NORD launched the "Show Your Stripes" campaign; the campaign's core message is to wear stripes on Rare Disease Day to raise awareness and show support for those living with rare diseases.

For more information about Rare Disease Day in the U.S. or "Show Your Stripes," go to www.rarediseaseday.us. For information about global activities, go to www.rarediseaseday.org. To search for information about rare diseases, visit NORD's website, www.rarediseases.org.

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 contending 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, and Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. Rocket's pre-clinical pipeline program is for Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of Rocket's product candidates, 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 and its licensors' ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, filed November 8, 2019 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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